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As filed with the Securities and Exchange Commission on August 17, 2015.

Registration No. 333-

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

FORM S-1
REGISTRATION STATEMENT
Under
THE SECURITIES ACT OF 1933

REGENXBIO INC.
(Exact Name of Registrant as Specified in its Charter)

Delaware	2836	47-1851754
(State or Other Jurisdiction of Incorporation or Organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification Number)

9712 Medical Center Drive, Suite 100
Rockville, MD 20850
(240) 552-8181

(Address, including zip code and telephone number, including area code, of registrant's principal executive offices)

Kenneth T. Mills
Chief Executive Officer
REGENXBIO Inc.
9712 Medical Center Drive, Suite 100
Rockville, MD 20850
(240) 552-8181
(Name, address, including zip code and telephone number, including area code, of agent for service)

Copies to:

Jay K. Hachigian, Esq. **Richard R. Hesp, Esq.** **Keith J. Scherer, Esq.** **Albert W. Vanderlaan, Esq.** **Gunderson Dettmer Stough Villeneuve Franklin & Hachigian, LLP** **One Marina Park Drive, Suite 900** **Boston, MA 02210** **Telephone: (617) 648-9100** **Telecopy: (617) 648-9199**	**Sara Garon Berl, Esq.** **Vice President, General Counsel and Secretary** **REGENXBIO Inc.** **9712 Medical Center Drive, Suite 100** **Rockville, MD 20850** **Telephone: (240) 552-8181**	**Richard D. Truesdell, Jr., Esq.** **Sophia Hudson, Esq.** **Davis Polk & Wardwell LLP** **450 Lexington Avenue** **New York, NY 10017** **Telephone: (212) 450-4000**

Approximate date of commencement of proposed sale to the public: As soon as practicable after the effective date of this Registration Statement.

If any of the securities being registered on this Form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. ☐

If this Form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, please check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

If this Form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. ☐

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

Large accelerated filer	☐	Accelerated filer	☐
Non-accelerated filer	☒ (Do not check if a smaller reporting company)	Smaller reporting company	☐

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities to be Registered	Proposed Maximum Aggregate Offering Price(1)	Amount of Registration Fee
Common Stock, $0.0001 par value	$100,000,000.00	$11,620.00

(1) Estimated pursuant to Rule 457(o) under the Securities Act of 1933, as amended. Includes additional shares that the underwriters have the option to purchase.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to such Section 8(a), may determine.

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The information in this preliminary prospectus is not complete and may be changed. These securities may not be sold until the registration statement filed with the Securities and Exchange Commission is effective. This preliminary prospectus is not an offer to sell nor does it seek an offer to buy these securities in any jurisdiction where the offer or sale is not permitted.

SUBJECT TO COMPLETION
DATED , 2015

Shares

Common Stock

REGENXBIO Inc. is offering shares of common stock. This is our initial public offering, and no public market currently exists for our common stock. We anticipate that the initial public offering price will be between $ and $ per share.

We have applied to list our common stock on the Nasdaq Global Market under the symbol "RGNX."

We are an "emerging growth company" under the federal securities laws and will be subject to reduced public company reporting requirements. Investing in our common stock involves a high degree of risk. See "Risk Factors" beginning on page 10.

	Per Share	***Total***
Initial public offering price	*$*	*$*
Underwriting discount and commissions(1)	*$*	*$*
Proceeds, before expenses, to us	*$*	*$*

(1) We have agreed to reimburse the underwriters for certain FINRA-related expenses. See "Underwriting."

We have granted the underwriters an option for a period of up to 30 days to purchase up to additional shares of common stock.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

The underwriters expect to deliver the shares on or about , 2015.

MORGAN STANLEY *BofA MERRILL LYNCH* *PIPER JAFFRAY*

CHARDAN CAPITAL MARKETS, LLC

The date of this prospectus is , 2015.

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We and the underwriters have not authorized anyone to provide any information or to make any representations other than those contained in this prospectus or in any free writing prospectuses we have prepared. We and the underwriters take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may provide you. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or of any sale of the common stock.

NAV® is our registered trademark and REGENXBIO Inc. and REGENXBIO are our trademarks. Any other trademarks appearing in this prospectus are the property of their respective holders.

PROSPECTUS SUMMARY

This summary highlights selected information contained elsewhere in this prospectus. Because this is only a summary, it does not contain all of the information you should consider before investing in our common stock. You should carefully read the entire prospectus, especially the risks set forth under the heading "Risk Factors" and our financial statements and related notes included elsewhere in this prospectus, before making an investment decision. References in this prospectus to "REGENXBIO," "our company," "we," "us" and "our" and other similar references refer to REGENXBIO Inc. during the periods presented unless the context requires otherwise.

Overview of REGENXBIO

We are a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. Our proprietary AAV gene delivery platform (our NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10 (NAV Vectors). Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing gene therapy products administered directly into the body, or *in vivo*, based on our NAV Technology Platform. We seek to accomplish our mission through a combination of our internal development efforts and the efforts of our third-party licensees (NAV Technology Licensees). Our NAV Technology Platform is currently being applied in the development of 23 product candidates for a variety of diseases, including five internally developed product candidates and 18 partnered product candidates developed by our NAV Technology Licensees.

Our most advanced internally developed candidates include programs for the treatment of two severe, rare genetic diseases: homozygous familial hypercholesterolemia (HoFH) and Mucopolysaccharidosis Type I (MPS I). We expect these programs to enter Phase I/II clinical trials in the first half of 2016. We also have a program for wet age-related macular degeneration (wet AMD) that is in the preclinical stage and for which we expect to file an Investigational New Drug application (IND) in the second half of 2016. We plan to build internal gene therapy franchises in the metabolic, neurodegenerative and retinal therapeutic areas, and develop multiple product candidates in these and other areas.

Our management team includes leaders who are experienced in building and operating innovative healthcare ventures and have expert knowledge in the development of AAV gene therapy. Our company was formed from a successful collaboration that began in February 2009 between FoxKiser LLP, the University of Pennsylvania (Penn) and gene therapy pioneer James M. Wilson, M.D., Ph.D.

Our Proprietary NAV Technology Platform for Gene Delivery

The foundation of our NAV Technology Platform was discovered in an effort to identify next generation AAV vectors that could overcome the limitations of earlier generation AAV vectors (AAV1 through AAV6). In AAV gene therapy, the viral genes are removed from the AAV, a small, non-pathogenic cold virus, creating a biological delivery vehicle called a vector. A therapeutic gene sequence is then inserted, creating a recombinant vector. We believe the key benefits of NAV Vectors over earlier generation AAV vectors include: higher gene expression, longer-term gene expression, broad and novel tissue selectivity, lower immune response and improved manufacturability. We believe that AAV gene therapies that incorporate the proprietary advances from our NAV Technology Platform (NAV Gene Therapy) have significantly enhanced profiles as potential therapeutics.

We believe our NAV Vectors have been broadly adopted, as approximately 70% of all AAV gene therapy clinical trials relating to new treatment INDs posted on the United States' government clinical trials database from 2012 through 2014 used our NAV Vectors. Proof-of-concept of our NAV Technology Platform is supported by three separately reported Phase I/II third-party clinical trials using AAV8 for the treatment of hemophilia B and a Phase I clinical trial using AAV9 for the treatment of spinal muscular atrophy.

We currently have exclusive rights to over 100 patents and patent applications worldwide covering our NAV Vectors, including composition of matter claims for AAV7, AAV8, AAV9 and AAVrh10, as well as methods for their manufacture and therapeutic uses. We believe this patent portfolio forms a strong foundation for our current programs, and with our ongoing research and development, we expect to continue to expand this substantial patent portfolio. Our patents not only seek to protect our key assets - our NAV Technology Platform and our internal product candidates - they also form the basis for licensing and partnering arrangements.

Our NAV Gene Therapy Product Candidates

We believe that the potential efficiency and broad applicability of our NAV Technology Platform will allow us to develop NAV Gene Therapy treatments that are injected or infused into the bloodstream, spinal fluid or directly into the target tissue to treat a wide range of diseases. Our internal product development program pipeline is shown below and our complete NAV Gene Therapy pipeline, including the product development programs being developed at our NAV Technology Licensees, can be found on page 98 of this prospectus.

INTERNALLY DEVELOPED PRODUCT CANDIDATES

Indication	Development Stage: Research	Development Stage: Preclinical	Development Stage: Clinical	Regulatory / Clinical Status
Metabolic Diseases				
Homozygous Familial Hypercholesterolemia (HoFH)	RGX-501			Phase I/II initiation anticipated 1H 2016
Neurodegenerative Diseases				
Mucopolysaccharidosis Type I (MPS I)	RGX-111			Phase I/II initiation anticipated 1H 2016
Mucopolysaccharidosis Type II (MPS II)	RGX-121			
Retinal Diseases				
Wet Age-related Macular Degeneration (wet AMD)	RGX-314			IND anticipated 2H 2016
X-linked Retinitis Pigmentosa (XLRP)	RGX-321			

Our most advanced internal development programs are for the treatment of two severe, rare genetic diseases.

RGX-501 is our product candidate for the treatment of HoFH, which uses the AAV8 vector to deliver the human low-density lipoprotein receptor (LDLR) gene to liver cells. HoFH is a monogenic disorder caused by abnormalities in the function or expression of the LDLR gene. HoFH patients have very low levels or are completely deficient of LDLR, resulting in very high total blood cholesterol levels. This leads to premature and aggressive plaque buildup, life threatening coronary artery disease (CAD) and aortic valve disease. We estimate approximately 35,000 individuals globally are afflicted with HoFH. The current standard of care for HoFH focuses on early initiation of aggressive treatment because of the severe clinical effects of elevated LDL, however, available treatment options are limited or insufficient. With our development partners at Penn, we intend to file an IND in the second half of 2015 to support the initiation of a dose-escalation Phase I/II clinical trial of intravenously administered RGX-501 in the United States in patients with HoFH beginning in the first half of 2016. We have received orphan drug product designation from the United States Food and Drug Administration (the FDA) for RGX-501. The FDA may designate a product as an orphan product if it is intended to treat a rare disease or condition, which generally is defined as having a patient population of fewer

than 200,000 individuals in the United States. If a biologic with orphan designation is the first of that biologic to receive marketing approval for the designated indication, the biologic receives a period of market exclusivity, subject to limited exceptions.

RGX-111 is our product candidate for the treatment of MPS I, which uses the AAV9 vector to deliver the human a-l-iduronidase (IDUA) gene to the central nervous system (CNS). MPS I is a rare genetic disease caused by deficiency of IDUA, an enzyme required for the breakdown of polysaccharides heparan sulfate and dermatan sulfate in the lysosomes, which are intracellular structures that dispose of waste products inside cells. Many patients develop symptoms related to glycosaminoglycan storage in the CNS, which can include excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. Over 1,000 individuals with MPS I are estimated to be born each year worldwide. Current standard of care treatments do not adequately treat the CNS manifestations of MPS I and leave a significant unmet medical need for a method to safely achieve longer-term IDUA reconstitution in the CNS. We intend to file an IND in the first half of 2016 to support the initiation of a dose-escalation Phase I/II clinical trial of RGX-111 based gene delivery via CNS administration in subjects with MPS I beginning in the first half of 2016.

We also have three additional internal programs in development for the treatment of another neurodegenerative disease, Mucopolysaccharidosis Type II (MPS II), and retinal diseases, wet AMD and X-linked retinitis pigmentosa (XLRP). Between 500 and 1,000 individuals are estimated to be born with MPS II each year worldwide. There may be up to 3,000,000 individuals with wet AMD worldwide, and there are an estimated 8,000 individuals with XLRP in the United States.

We believe there are many more potential applications of our NAV Technology Platform than we currently have the resources to develop on our own. Our partnered development pipeline benefits from the disease-specific expertise of our NAV Technology Licensees. Our partnering strategy provides us the flexibility to sublicense the development of treatments, while we remain focused on our core programs and therapeutic areas. Most of our NAV Technology Licensees have licensed specific NAV Vectors for the indications they are pursuing. We maintain rights in our NAV Technology Platform to all unlicensed indications as well as unlicensed NAV Vectors in disease indications for which we have granted licenses. We believe that the broad applicability of our NAV Technology Platform and any clinical successes of the treatments utilizing NAV Vectors will create new internal and partnered pipeline opportunities.

Our Strategy

Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing *in vivo* gene therapy products based on our NAV Technology Platform. We intend to develop, manufacture, commercialize and license product candidates across multiple therapeutic areas while continuing to expand our NAV Technology Platform. To achieve our goal, we plan to:

- apply our proprietary, next generation AAV vector technology to develop *in vivo* gene therapies for patients;
- focus on rapidly advancing our internal lead proprietary development programs in our core therapeutic areas of metabolic, neurodegenerative and retinal diseases;
- expand to additional product candidates in our core therapeutic areas once proof-of-concept is established;
- further grow the pipeline of products based on our NAV Technology Platform through strategic in-licensing and sublicensing of new programs; and
- maintain and grow our extensive intellectual property portfolio.

Risks Related to Our Business

Our business is subject to numerous risks and uncertainties, including those highlighted in the section titled "Risk Factors" immediately following this prospectus summary. These risks represent challenges to the successful implementation of our strategy and to the growth and future success of our business. Some of these risks include the following:

- Our gene therapy product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No gene therapy product has been approved in the United States and only one such product has been approved in the European Union.
- Our business depends substantially on the success of RGX-111, RGX-121, RGX-314, RGX-321 and RGX-501 (collectively, our Lead Product Candidates), which are all still in preclinical development. If we are unable to obtain regulatory approval for, or successfully commercialize, our Lead Product Candidates or other future product candidates, our business will be materially harmed.
- We have incurred substantial net losses since inception, and have only had one quarter since inception with profitability. We expect to incur losses for the foreseeable future and may never again achieve or maintain profitability.
- In addition to our Lead Product Candidates, our business substantially depends on the success of our NAV Technology Licensees. One or more of our NAV Technology Licensees may be unable to demonstrate through clinical trials that their programs are safe and effective, which may lead to a perception, whether accurate or not, that our NAV Technology is of limited value.
- We may not be successful in our efforts to identify or discover additional product candidates.
- We rely primarily on a sponsored research agreement with The Trustees of the University of Pennsylvania for our nonclinical research and development activities and a loss of this relationship or of the principal investigator for those nonclinical research and development activities, James M. Wilson, M.D., Ph.D., would materially harm our business.
- We have in the past, and in the future plan to, enter into licensing agreements with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements are not successful, our business could be harmed.
- Our rights to license our NAV Technology Platform and to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.
- Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.
- If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.
- In preparation for this offering, we identified two material weaknesses in our internal control over financial reporting. If we are unable to remedy our material weaknesses, or if we fail to establish and maintain effective internal controls, we may be unable to produce timely and accurate financial statements, and we may conclude that our internal control over financial reporting is not effective, which could adversely impact our investors' confidence and our stock price.
- Even if this offering is successful, we will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.

For further discussion of these and other risks you should consider before deciding to invest in our common stock, see the section titled “Risk Factors” immediately following this prospectus summary.

Our Corporate Information

We were originally formed on July 16, 2008 as ReGenX, LLC, a Delaware limited liability company, and we were subsequently renamed ReGenX Biosciences, LLC on December 22, 2009. On September 16, 2014, we underwent a corporate reorganization pursuant to which we were converted into a Delaware corporation under the name REGENXBIO Inc. Our principal offices are located at 9712 Medical Center Drive, Suite 100, Rockville, MD 20850, and our telephone number is (240) 552-8181. Our website address is www.regenxbio.com. Our website and the information contained on, or that can be accessed through, the website will not be deemed to be incorporated by reference in, and are not considered part of, this prospectus. You should not rely on any such information in making your decision whether to purchase our common stock.

Implications of Being an Emerging Growth Company

We qualify as an “emerging growth company” as defined in the Jumpstart Our Business Startups Act of 2012 (JOBS Act). An emerging growth company may take advantage of specified reduced reporting and other burdens that are otherwise applicable generally to public companies. These provisions include:

- a requirement to have only two years of audited financial statements and only two years of related management’s discussion and analysis;
- exemption from the auditor attestation requirement on the effectiveness of our internal controls over financial reporting;
- reduced disclosure about our executive compensation arrangements; and
- no non-binding advisory votes on executive compensation or golden parachute arrangements.

We may take advantage of these provisions until December 31, 2020 (the last day of our fiscal year following the fifth anniversary of the date of the first sale of our common equity securities pursuant to this offering) or until such earlier time that we are no longer an emerging growth company. We would cease to be an emerging growth company if we have more than $1.0 billion in annual revenue, have more than $700.0 million in market value of our capital stock held by non-affiliates, or issue more than $1.0 billion of non-convertible debt over a three-year period. We may choose to take advantage of some but not all of these reduced burdens. We have taken advantage of these reduced reporting burdens in this prospectus, although we may choose not to do so in future filings and if we do not, the information that we provide stockholders may be different than you may receive from other public companies in which you hold equity interests. We have irrevocably elected not to avail ourselves of the ability under the JOBS Act to delay adopting new or revised accounting standards until such time as those standards apply to private companies.

THE OFFERING

Common stock offered by us	Shares
Common stock to be outstanding after this offering	Shares
Option to purchase additional common stock offered by us	Shares
Use of proceeds	We estimate that we will receive net proceeds from this offering of approximately $ million, assuming an initial public offering price of $ per share, the midpoint of the initial public offering price range reflected on the cover page of this prospectus, and after deducting the estimated underwriting discount and offering expenses payable by us. If the underwriters' option to purchase additional shares in this offering is exercised in full, we estimate that our net proceeds will be approximately $ million. As of June 30, 2015, we had cash and cash equivalents of $85.2 million. We intend to use net proceeds from this offering, together with existing cash resources, to advance our development of RGX-501, RGX-111, RGX-314, our other internally developed product candidates and for general working capital and administrative purposes. See "Use of Proceeds" in this prospectus for a more complete description of the intended use of proceeds from this offering.
Risk factors	You should read the "Risk Factors" section of this prospectus for a discussion of factors that you should consider carefully before deciding to invest in shares of our common stock.
Proposed NASDAQ trading symbol	"RGNX"

The number of shares of our common stock to be outstanding following this offering is based on 19,050,708 shares of our common stock outstanding as of June 30, 2015, which includes the conversion of 16,298,045 shares of convertible preferred stock outstanding as of June 30, 2015, and excludes:

- 3,063,200 shares of common stock issuable upon the exercise of options outstanding as of June 30, 2015 under our 2014 Stock Plan at a weighted average exercise price of $1.86 per share;
- 927,100 shares of common stock reserved for issuance under our 2014 Stock Plan; and
- shares of common stock reserved for issuance under our 2015 Equity Incentive Plan, which became effective in June 2015 but with respect to which no awards will be granted prior to the effective date of the registration statement of which this prospectus is a part, and shares of common stock reserved for issuance under our 2015 Employee Stock Purchase Plan which becomes effective on the effective date of the registration statement of which this prospectus is a part, subject in each case to automatic annual adjustment in accordance with the terms of the plan.

Unless otherwise indicated, this prospectus reflects and assumes the following:

- the automatic conversion of all outstanding shares of our convertible preferred stock into 16,298,045 shares of common stock, upon the completion of this offering;
- the filing of our restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior to completion of this offering; and
- no exercise by the underwriters of their option to purchase additional shares of common stock.

SUMMARY FINANCIAL DATA

The following summary financial data for the years ended December 31, 2013 and 2014 are derived from our audited financial statements appearing elsewhere in this prospectus. The summary financial data as of June 30, 2015 and for the six months ended June 30, 2014 and 2015 have been derived from our unaudited financial statements included elsewhere in this prospectus. In our opinion, these unaudited financial statements have been prepared on a basis consistent with our audited financial statements and contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair statement of such financial data. You should read this data together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information under the captions "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of our future results, and our operating results for the six-month period ended June 30, 2015 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2015 or any other interim periods or any future year or period.

(in thousands, except per share data)	Year Ended December 31,		Six Months Ended June 30,	
	2013	2014	2014	2015
Statements of operations data:				
Revenues				
License revenue	$ 1,055	$ 4,355	$ 3,705	$ 570
License revenue from related party	2,700	220	—	1,000
Reagent sales	368	326	291	148
Grant revenue	1,964	1,219	490	289
Total revenues	6,087	6,120	4,486	2,007
Expenses				
Costs of revenues				
Licensing costs to related parties	151	885	741	314
Costs of reagent sales (including amounts to related parties)	173	122	102	49
Research and development (including amounts to related parties)	5,051	4,961	1,787	6,803
General and administrative (including amounts to related parties)	5,474	3,851	1,660	5,113
Foreign currency transaction losses (gains)	14	30	(14)	38
Other operating income	—	(47)	(24)	(21)
Total operating expenses	10,863	9,802	4,252	12,296
Income (loss) from operations	(4,776)	(3,682)	234	(10,289)
Other income (expense)				
Investment income	—	—	—	8
Interest expense	(611)	(321)	(111)	(20)
Total other income (expense)	(611)	(321)	(111)	(12)
Net income (loss)	(5,387)	(4,003)	123	(10,301)
Accretion and dividends on convertible preferred stock and preferred units	(422)	(815)	(467)	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders and members	$ (5,809)	$ (4,818)	$ (344)	$ (11,289)
Net loss attributable to common stockholders per share:				
Basic and diluted	$ (2.50)	$ (1.82)	$ (0.13)	$ (4.21)
Basic and diluted, pro forma (unaudited)(1)		$ (0.58)		$ (0.78)
Weighted average common shares outstanding:				
Basic and diluted	2,320	2,643	2,643	2,679
Basic and diluted, pro forma (unaudited)(1)		6,943		13,149

(in thousands)	As of June 30, 2015		
	Actual	Pro forma(2)	Pro forma as adjusted(3)(4)
Balance sheet data:			
Cash and cash equivalents	$ 85,215	$ 85,215	
Working capital	81,051	81,051	
Total assets	88,800	88,800	
Accrued expenses	3,062	3,062	
Other related party payables	1,919	1,919	
Total liabilities	6,172	6,172	
Convertible preferred stock	111,392	—	
Total stockholders' equity (deficit)	(28,764)	82,628	

(1) See Note 2 to our financial statements included elsewhere in this prospectus for a description of the method used to calculate the basic and diluted net loss per share and pro forma basic and diluted net loss per share.

(2) Pro forma reflects the automatic conversion of all outstanding shares of our preferred stock on June 30, 2015 into 16,298,045 shares of our common stock prior to the completion of this offering.

(3) Pro forma as adjusted reflects the sale of shares of our common stock offered in this offering, assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

(4) A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase (decrease) the pro forma as adjusted amount of each of cash and cash equivalents and total stockholders' (deficit) equity by approximately $ million, assuming that the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting underwriting discounts and commissions and estimated offering expenses payable by us. A share increase in the number of shares offered by us together with a concomitant $1.00 increase in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, would increase each of cash and cash equivalents and total stockholders' (deficit) equity by approximately $ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us. Conversely, a share decrease in the number of shares offered by us together with a concomitant $1.00 decrease in the assumed initial public offering price of $ per share, the midpoint of the initial public offering price range set forth on the cover of this prospectus, would decrease each of cash and cash equivalents and total stockholders' (deficit) equity by approximately $ million after deducting underwriting discounts and commissions and any estimated offering expenses payable by us.

RISK FACTORS

Investing in our common stock involves a high degree of risk. You should consider carefully the risks and uncertainties described below, together with all of the other information in this prospectus, including our financial statements and related notes, before deciding whether to purchase shares of our common stock. If any of the following risks is realized, our business, financial condition, results of operations, and prospects could be materially harmed. In that event, the price of our common stock could decline and you could lose part or all of your investment.

Risks Related to our NAV Technology Platform and the Development of Our Product Candidates

Our gene therapy product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No gene therapy product has been approved in the United States and only one such product has been approved in the European Union.

We have concentrated our research and development efforts on our proprietary adeno-associated virus (AAV) gene delivery platform (our NAV Technology Platform), and our future success depends on our and our licensees' successful development and commercialization of viable gene therapy product candidates. There can be no assurance that we or our licensees will not experience problems or delays in developing current or future product candidates or that such problems or delays will not cause unanticipated costs, or that any such development problems can be solved. We also may experience unanticipated problems or delays in expanding our manufacturing capacity, and this may prevent us from completing our clinical trials, meeting the obligations of our collaborations or commercializing our products on a timely or profitable basis, if at all. For example, we, a partner or another group may uncover one or more previously unknown risks associated with AAV or our NAV Technology Platform, and this may prolong the period of observation required for obtaining regulatory approval, necessitate additional clinical testing or invalidate our NAV Technology.

In addition, the clinical trial requirements of the United States Food and Drug Administration (the FDA), the European Medicines Agency (the EMA) and other regulatory authorities and the criteria these regulators use to determine the quality, safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of such product candidates. The regulatory approval process for novel product candidates such as ours can be significantly more expensive and take longer than for other, better known or more extensively studied product candidates. No gene therapy product has been approved in the United States, and only one gene therapy product, uniQure N.V.'s Glybera, has received marketing authorization from the European Commission. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or how long it will take to commercialize our product candidates. Furthermore, approvals by the European Commission may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. The FDA has established the Office of Cellular, Tissue and Gene Therapies within its Center for Biologics Evaluation and Research (CBER), to consolidate the review of gene therapy and related products, and has established the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER in its review. Gene therapy clinical trials conducted at institutions that receive funding for recombinant DNA research from the United States National Institutes of Health (NIH), also are potentially subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee (the RAC). However, NIH announced in 2014 that the RAC will soon only publicly review clinical trials if the trials cannot be evaluated by standard oversight bodies and pose unusual risks. Although the FDA decides whether individual gene therapy protocols may proceed, the RAC public review process, if undertaken, can delay the initiation of a clinical trial, even if the FDA has reviewed the trial design and details and approved its initiation. Conversely, the FDA can put an investigational new drug (IND) on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. If we were to engage an NIH-funded institution to conduct

a clinical trial, that institution's institutional biosafety committee as well as its institutional review board (IRB) would need to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, in the European Union, the EMA's Committee for Advanced Therapies (CAT) is responsible for assessing the quality, safety and efficacy of advanced-therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. In the European Union, the development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines. This includes the Note for guidance on the quality, preclinical and clinical aspects of gene therapy medicinal products. This guidance document (CPMP/BWP/3088/99) is currently under review in the European Union. The EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate product revenue, and our business, financial condition, results of operations and prospects would be materially harmed.

Our business depends substantially on the success of our Lead Product Candidates RGX-111, RGX-121, RGX-314, RGX-321 and RGX-501 (collectively, our Lead Product Candidates), which are all still in preclinical development. If we are unable to obtain regulatory approval for, or successfully commercialize, our Lead Product Candidates or other future product candidates, our business will be materially harmed.

Our Lead Product Candidates are in the early stage of development and will require preclinical studies, substantial clinical development and testing, manufacturing bridging studies and process validation and regulatory approval prior to commercialization. Successful continued development and ultimate regulatory approval of our Lead Product Candidates is critical for our future business success and our ability to generate product revenue. We have invested, and will continue to invest, a significant portion of our financial resources in the development of our Lead Product Candidates. We will need to raise sufficient funds for, and successfully complete, our planned preclinical and future clinical trials of our Lead Product Candidates in appropriate subjects. The future regulatory and commercial success of these product candidates is subject to a number of risks, including the following:

- we may not have sufficient financial and other resources to complete the necessary preclinical studies and clinical trials for our Lead Product Candidates;
- we may not be able to provide evidence of quality, efficacy and safety for our Lead Product Candidates;
- we do not know the degree to which our Lead Product Candidates will be accepted by patients, the medical community and third-party payors as a therapy for the respective diseases to which they relate, even if approved;
- the results of our clinical trials may not meet the level of statistical or clinical significance required by the FDA, EMA or comparable foreign regulatory bodies for marketing approval;
- subjects in our clinical trials, if any, may die or suffer other adverse effects for reasons that may or may not be related to our Lead Product Candidates;

- subjects in clinical trials, if any, undertaken by licensees under a license we grant of certain intellectual property related to our NAV Technology Platform (our NAV Technology Licensees) may die or suffer adverse effects, that may or may not be related to our NAV Technology Platform;
- certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes;
- we may not successfully establish commercial manufacturing capabilities;
- if approved for treatment of MPS I, MPS II, wet age-related macular degeneration (wet AMD), X-linked retinitis pigmentosa (XLRP) and homozygous familial hypercholesterolemia (HoFH), our Lead Product Candidates will likely compete with other treatments then available, including the off-label use of products already approved for marketing and other therapies currently available or which may be developed;
- our products and products developed by our NAV Technology Licensees, if any, may not maintain a continued acceptable safety profile following regulatory approval;
- we may not maintain compliance with post-approval regulation and other requirements; and
- we may not be able to obtain, maintain or enforce our rights under our licensed patents and other intellectual property rights.

Of the large number of biologics and drugs in development in the pharmaceutical industry, only a small percentage result in the submission of a Biologics License Application (BLA) to the FDA or marketing authorization application (MAA) to the EMA and even fewer are approved for commercialization. Furthermore, even if we do receive regulatory approval to market our Lead Product Candidates, any such approval may be subject to limitations on the indicated uses for which we may market the product. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development programs, we cannot assure you that our Lead Product Candidates will be successfully developed or commercialized. If we or any of our future development partners are unable to develop, or obtain regulatory approval for, or, if approved, successfully commercialize, our Lead Product Candidates, we may not be able to generate sufficient revenue to continue our business.

We may not be successful in our efforts to identify or discover additional product candidates.

The success of our business depends primarily upon our ability to identify, develop and commercialize products based on our NAV Technology Platform. Although our Lead Product Candidates are currently in preclinical development, our research programs may fail to identify other potential product candidates for clinical development for a number of reasons. Our research methodology may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

If any of these events occur, we may be forced to abandon our development efforts for a program or programs, which would materially harm our business and could potentially cause us to cease operations. Research programs to identify new product candidates require substantial technical, financial and human resources. We may focus our efforts and resources on potential programs or product candidates that ultimately prove to be unsuccessful.

We have not tested any of our viral vectors, or product candidates internally derived from these viral vectors, in our own clinical trials.

Gene therapy development has inherent risks. None of our internal product candidates have ever been evaluated in clinical studies and our Lead Product Candidates have limited preclinical results, if any, and we may experience unexpected results in the future. We or any of our future development partners will be required to

demonstrate through adequate and well-controlled clinical trials that our product candidates containing our proprietary vectors are safe and effective, with a favorable benefit-risk profile, for use in their target indications before we can seek regulatory approvals for their commercial sale. Drug development is a long, expensive and uncertain process, and delay or failure can occur at any stage of development, including after commencement of any of our clinical trials.

The results of preclinical studies and early clinical trials are not always predictive of future results. Any product candidate we or any of our future development partners advance into clinical trials, including our Lead Product Candidates, may not have favorable results in later clinical trials, if any, or receive regulatory approval. There is a high failure rate for drugs and biologic products proceeding through clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations that may delay, limit or prevent regulatory approval. In addition, we may experience regulatory delays or rejections as a result of many factors, including due to changes in regulatory policy during the period of our product candidate development. Any such delays could materially harm our business, financial condition, results of operations and prospects.

If our NAV vectors are not shown to be safe and effective, we may not realize the value of our investment in our technology. In addition, success in early clinical trials does not mean that later clinical trials will be successful, because product candidates in later-stage clinical trials may fail to demonstrate sufficient safety or efficacy despite having progressed through initial clinical testing. Furthermore, our future trials will need to demonstrate sufficient safety and efficacy for approval by regulatory authorities in larger patient populations. Companies frequently suffer significant setbacks in advanced clinical trials, even after earlier clinical trials have shown promising results. In addition, only a small percentage of drugs under development result in the submission of a BLA to the FDA or MAA to the EMA and even fewer are approved for commercialization.

We cannot be certain that any of our planned clinical trials will be successful, and any safety concerns observed in any one of our planned clinical trials in our targeted indications could limit the prospects for regulatory approval of our product candidates in those and other indications. In addition, failure of one or more of our viral vectors, whether in our internally developed product candidates or those of our licensees, would impact the licensing of our NAV Technology Platform. Any such failure could materially harm our business, financial condition, results of operations and prospects.

Because we are developing product candidates for the treatment of diseases in which there is little clinical experience using new endpoints or methodologies, there is increased risk that the FDA or other comparable foreign regulatory authorities may not consider the endpoints of our clinical trials to provide clinically meaningful results and that these results may be difficult to analyze.

During the FDA review process, we will need to identify success criteria and endpoints such that the FDA will be able to determine the clinical efficacy and safety profile of our product candidates. As we are developing novel treatments for diseases in which there is little clinical experience with new endpoints and methodologies, there is heightened risk that the FDA, the EMA or comparable foreign regulatory bodies may not consider the clinical trial endpoints to provide clinically meaningful results (reflecting a tangible benefit to patients). In addition, the resulting clinical data and results may be difficult to analyze. Even if the FDA does find our success criteria to be sufficiently validated and clinically meaningful, we may not achieve the pre-specified endpoints to a degree of statistical significance. Further, even if we do achieve the pre-specified criteria, we may produce results that are unpredictable or inconsistent with the results of the non-primary endpoints or other relevant data. The FDA also weighs the benefits of a product against its risks, and the FDA may view the efficacy results in the context of safety as not being supportive of regulatory approval. Other regulatory authorities in the European Union and other countries, such as the EMA's CAT, may make similar comments with respect to these endpoints and data. As discussed above, our gene therapy product candidates are based on a novel technology that makes it difficult to predict the time and cost of development and of subsequently obtaining regulatory approval. No gene therapy product has been approved in the United States. Only one gene therapy product has received marketing authorization from the European Commission.

The results from our preclinical or clinical trials for our Lead Product Candidates may not support as broad a marketing approval as we seek, and the FDA and the EMA or other regulatory authorities may require us to conduct additional clinical trials or evaluate subjects for an additional follow-up period.

While we believe our Lead Product Candidates should be applicable for the treatment of patients with MPS I, MPS II, wet AMD, XLRP and HoFH, the results from our preclinical and planned clinical trials may not support as broad of a marketing approval as we seek. Even if we obtain regulatory approval for our Lead Product Candidates, we may be required by the FDA, the EMA or comparable foreign regulatory bodies to conduct additional clinical trials to support approval of our Lead Product Candidates for patients diagnosed with different mutations of MPS I, MPS II, wet AMD, XLRP and HoFH. This could result in our experiencing significant increases in costs and substantial delays in obtaining, or never obtaining, marketing approval for our Lead Product Candidates to treat patients diagnosed with MPS I, MPS II, wet AMD, XLRP and HoFH, respectively. The inability to market our Lead Product Candidates to treat patients with MPS I, MPS II, wet AMD, XLRP and HoFH, would materially harm our business, financial condition, results of operations and prospects.

We may find it difficult to enroll patients in clinical trials, and this could delay or prevent us from proceeding with clinical trials of our product candidates.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. The timing of our planned clinical trials depends on our ability to recruit patients to participate as well as completion of required follow-up periods. If patients are unwilling to participate in our gene therapy studies because of negative publicity from adverse events related to the biotechnology or gene therapy fields, competitive clinical trials for similar patient populations, clinical trials in products employing our vectors or our platform or for other reasons, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed. These delays could result in increased costs, delays in advancing our product candidates, delays in testing the effectiveness of our product candidates or termination of the clinical trials altogether.

We may not be able to identify, recruit and enroll a sufficient number of patients, or those with required or desired characteristics, to complete our planned clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- size of the patient population and process for identifying subjects;
- design of the trial protocol;
- eligibility and exclusion criteria;
- perceived risks and benefits of the product candidate under study;
- perceived risks and benefits of gene therapy-based approaches to treatment of diseases;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor subjects adequately during and after treatment.

Our current product candidates are being developed to treat a variety of conditions, many of which are rare. We plan to seek initial marketing approvals in the United States and the European Union. We may not be able to initiate or continue clinical trials if we cannot enroll a sufficient number of eligible patients to participate in the clinical trials required by the FDA or the EMA or other comparable foreign regulatory authorities. Our ability to successfully initiate, enroll and complete a clinical trial in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with contract research organizations (CROs) and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of gene therapy protocols;
- our inability to locate qualified local consultants, physicians and partners; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatments.

If we have difficulty enrolling a sufficient number of patients to conduct our clinical trials as planned, we may need to delay, limit or terminate then ongoing or planned clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

We may encounter substantial delays in our planned clinical trials, or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities.

Before obtaining marketing approval from regulatory authorities for the sale of our product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A failure of one or more clinical trials can occur at any stage of testing. Events that may prevent successful or timely completion of preclinical and clinical development include:

- delays in reaching a consensus with regulatory authorities on trial design;
- delays in reaching agreement on acceptable terms with prospective CROs and clinical trial sites;
- delays in opening clinical trial sites or obtaining required IRB or independent Ethics Committee approval at each clinical trial site;
- delays in recruiting suitable subjects to participate in our clinical trials;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA good clinical practice (GCP), or applicable regulatory guidelines in the European Union and other countries;
- delays in the testing, validation, manufacturing and delivery of our product candidates to the clinical sites, including delays by third parties with whom we have contracted to perform certain of those functions;
- delays in having subjects complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or subjects dropping out of a trial;

- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

Any inability to successfully complete research studies, preclinical and clinical development could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. In addition, if we make manufacturing or formulation changes to our product candidates, we may need to conduct additional studies to bridge our modified product candidates to earlier versions. Clinical trial delays also could shorten any periods during which we may have the exclusive right to commercialize our product candidates or allow our competitors to bring products to market before we do, which could impair our ability to successfully commercialize our product candidates and may harm our business, financial condition, results of operations and prospects.

Additionally, if the results of our clinical trials are inconclusive or if there are safety concerns or serious adverse events associated with our product candidates, we may:

- be delayed in obtaining marketing approval for our product candidates, if at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or safety warnings;
- be subject to changes in the way the product is administered;
- be required to perform additional clinical trials to support approval or be subject to additional post-marketing testing requirements;
- have regulatory authorities withdraw, or suspend, their approval of the product or impose restrictions on its distribution in the form of a modified risk evaluation and mitigation strategy;
- be subject to the addition of labeling statements, such as warnings or contraindications;
- be sued; or
- experience damage to our reputation.

Our NAV Technology Platform, our Lead Product Candidates and future product candidates, if any, or NAV Technology Licensees' product candidates, and the process for administering such product candidates may cause undesirable side effects or have other properties that could delay or prevent regulatory approval of product candidates, limit the commercial potential or result in significant negative consequences following any potential marketing approval.

There have been several significant adverse side effects in gene therapy treatments in the past, including reported cases of leukemia in trials using lentivirus vectors and death seen in other trials using adenovirus vectors. For example, in 1999, a gene therapy trial of research subjects with ornithine transcarbamylase (OTC) deficiency, a rare disorder in which the liver lacks a functional copy of the OTC gene, resulted in the death of a trial subject due to complications of adenovirus vector administration. James M. Wilson, M.D., Ph.D. was a co-investigator of the 1999 trial while he was Director of the Institute for Human Gene Therapy of the University of Pennsylvania. While new recombinant vectors have been designed to reduce these side effects, gene therapy is

still a relatively new approach to disease treatment and additional adverse side effects could develop. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material. Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction early after administration which could substantially limit the effectiveness of the treatment. In previous clinical trials involving AAV vectors for gene therapy, some subjects experienced the development of a T-cell response, whereby after the vector is within the target cell, the cellular immune response system triggers the removal of transduced cells by activated T-cells. If our vectors demonstrate a similar effect, we may decide or be required to halt or delay preclinical development or clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures also can cause adverse side effects. If any such adverse events occur in our or third party trials, our clinical trials could be suspended or terminated.

As a result of these concerns, the FDA, the European Commission, the EMA or other comparable foreign regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Even if we are able to demonstrate that all future serious adverse events are not product-related, such occurrences could affect patient recruitment or the ability of enrolled patients to complete the trial. Moreover, if we elect, or are required, to delay, suspend or terminate any clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates and may harm our business, financial condition and prospects significantly.

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy (REMS) to ensure that the benefits outweigh its risks. A REMS may include, among other things, a medication guide outlining the risks of the product for distribution to patients; a communication plan to health care practitioners; and elements to assure safe use, which can severely restrict the distribution of a product by, for example, requiring that health care providers receive particular training and obtain special certification prior to prescribing and dispensing the product, limiting the healthcare settings in which the product may be dispensed, and subjecting patients to monitoring and enrollment in a registry. If FDA requires us to adopt a REMS for our products and we are unable to comply with its requirements, FDA may deem our products to be misbranded and we may be subject to civil money penalties. The European Commission and other comparable foreign regulatory authorities may, following grant of marketing authorization in their territory, impose similar obligations.

Furthermore, if we or others later identify undesirable side effects caused by one of our product candidates, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend, vary or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of these events could prevent us from achieving or maintaining market acceptance of our NAV Technology Platform and our product candidates and could materially harm our business, prospects, financial condition and results of operations.

We may be unable to obtain orphan drug designation or exclusivity. If our competitors are able to obtain orphan drug exclusivity for products that constitute the same drug and treat the same indications as our product candidates, we may not be able to have competing products approved by the applicable regulatory authority for a significant period of time.

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug if it is intended to treat a rare disease or condition, which is defined under the FD&C Act as having a patient population of fewer than 200,000 individuals in the United States, or a patient population greater than 200,000 in the United States where there is no reasonable expectation that the cost of developing the drug will be recovered from sales in the United States. In the European Union, following the opinion of the EMA's Committee for Orphan Medicinal Products, the European Commission grants orphan drug designation to promote the development of products that are intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in 10,000 persons in the European Union. Additionally, orphan designation is granted for products intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and when, without incentives, it is unlikely that sales of the drug in the European Union would be sufficient to justify the necessary investment in developing the drug or biologic product.

Generally, if a product candidate with an orphan drug designation receives the first marketing approval for the indication for which it has such designation, the product is entitled to a period of marketing exclusivity, which precludes the FDA or the European Commission from approving another marketing application for a product that constitutes the same drug treating the same indication for that marketing exclusivity period, except in limited circumstances. If another sponsor receives such approval before we do (regardless of our orphan drug designation), we will be precluded from receiving marketing approval for our product for the applicable exclusivity period. The applicable period is seven years in the United States and 10 years in the European Union. The exclusivity period in the United States can be extended by six months if the BLA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The exclusivity period in the European Union can be reduced to six years if a product no longer meets the criteria for orphan drug designation or if the product is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be revoked if any regulatory agency determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the product to meet the needs of patients with the rare disease or condition.

If we request orphan drug designation for any of our product candidates, there can be no assurances that the FDA or the European Commission will grant any of our product candidates such designation. Additionally, the designation of any of our product candidates as an orphan product does not guarantee that any regulatory agency will accelerate regulatory review of, or ultimately approve, that product candidate, nor does it limit the ability of any regulatory agency to grant orphan drug designation to product candidates of other companies that treat the same indications as our product candidates prior to our product candidates receiving exclusive marketing approval.

Even if we obtain orphan drug exclusivity for a product candidate, that exclusivity may not effectively protect the product candidate from competition because different drugs can be approved for the same condition. In the United States, even after an orphan drug is approved, the FDA may subsequently approve another drug for the same condition if the FDA concludes that the latter drug is not the same drug or is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care. In the European Union, marketing authorization may be granted to a similar medicinal product for the same orphan indication if:

- the second applicant can establish in its application that its medicinal product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;

- the holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- the holder of the marketing authorization for the original orphan medicinal product cannot supply sufficient quantities of orphan medicinal product.

Even if we complete the necessary preclinical and clinical trials, we cannot predict when, or if, we will obtain regulatory approval to commercialize a product candidate and the approval may be for a more narrow indication than we seek.

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner or we may not be able to obtain regulatory approval. Additional delays may result if an FDA Advisory Committee or other regulatory authority recommends non-approval or restrictions on approval. In addition, we may experience delays or rejections based on additional government regulation from future legislation or administrative action or based on changes in regulatory authority policy during the period of product development, clinical trials and the review process.

Regulatory authorities also may approve a product candidate for more limited indications than requested (such as approving RGX-111 only for patients with Hurler Syndrome, a severe subset of MPS I patients) or they may impose significant limitations in the form of narrow indications, warnings or a REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects.

Further, the regulatory authorities may require concurrent approval or the CE mark (a mandatory conformity assessment marking for certain products sold within the European Economic Area (EEA)) of a companion diagnostic device, since it may be necessary to use FDA- cleared or FDA-approved, or CE-marked, diagnostic tests or diagnostic tests approved by other comparable foreign regulatory authorities to diagnose patients or to assure the safe and effective use of our product candidates in trial subjects. FDA refers to such tests as *in vitro* companion diagnostic devices. On July 31, 2014, the FDA announced the publication of a final guidance document describing the agency's current thinking about the development and regulation of *in vitro* companion diagnostic devices. The final guidance articulates a policy position that, when safe and effective use of a therapeutic product depends on a diagnostic device, the FDA generally will require approval or clearance of the diagnostic device at the same time that FDA approves the therapeutic product. The final guidance allows for two exceptions to the general rule of concurrent drug/device approval, namely, when the therapeutic product is intended to treat serious and life-threatening conditions for which no alternative exists, and when a serious safety issue arises for an approved therapeutic agent, and no FDA-cleared or FDA-approved companion diagnostic test is yet available. It is unclear how the FDA will apply this policy to our current or future gene therapy product candidates. Although we believe diagnoses based on symptoms in conjunction with existing genetic tests developed and administered by laboratories certified under the Clinical Laboratory Improvement Amendments (CLIA) are sufficient to diagnose patients for our current product candidates, the FDA may disagree. Should the FDA deem genetic tests used for diagnosing patients for our therapies to be *in vitro* companion diagnostics requiring FDA clearance or approval, we may face significant delays or obstacles in obtaining approval of a BLA for our product candidates. In the European Union, the European Commission has proposed substantial revisions to the current Directive governing *in vitro* diagnostic medical devices. If adopted in their current form, these revisions may impose additional obligations on us that may impact the development and authorization of our product candidates in the European Union.

Even if we obtain regulatory approval for a product candidate, our products will remain subject to regulatory oversight.

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase IV clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. The FDA Guidance for Industry on *Gene Therapy Clinical Trials—Observing Subjects for Delayed Adverse Events* advises that patients treated with some types of gene therapy undergo follow-up observations for potential adverse events for as long as 15 years. The holder of an approved BLA also must submit new or supplemental applications and obtain the FDA approval for certain changes to the approved product, product labeling or manufacturing process. Advertising and promotional materials must comply with the FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practice (cGMP) requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or disagrees with the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

If we fail to comply with applicable regulatory requirements following approval of any of our product candidates, a regulatory authority may take a variety of actions, including:

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose administrative, civil or criminal penalties or monetary fines;
- suspend or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve a pending BLA or comparable foreign marketing application (or any supplements thereto) submitted by us or our strategic partners;
- restrict the marketing or manufacturing of the product;
- seize or detain the product or otherwise require the withdrawal of the product from the market;
- refuse to permit the import or export of products; or
- refuse to allow us to enter into supply contracts, including government contracts.

Any government investigation of alleged violations of law could require us to expend significant time and resources to respond and could generate negative publicity. The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and harm our business, financial condition, results of operations and prospects.

In addition, the FDA’s policies, and those of comparable foreign regulatory authorities, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise

from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability, which would materially harm our business, financial condition, results of operations and prospects.

We face significant competition in an environment of rapid technological change and there is a possibility that our competitors may achieve regulatory approval before us or develop therapies that are safer or more advanced or effective than ours, which may harm our financial condition and our ability to successfully market or commercialize our product candidates.

The biotechnology and pharmaceutical industries, including the gene therapy field, are characterized by rapidly changing technologies, significant competition and a strong emphasis on intellectual property. We face substantial competition from many different sources, including large and specialty pharmaceutical and biotechnology companies, academic research institutions, government agencies and public and private research institutions.

We are aware of several companies focused on developing gene therapies in various indications, as well as several companies addressing other methods for modifying genes and regulating gene expression. Any advances in gene therapy technology made by a competitor may be used to develop therapies that could compete against any of our product candidates.

Many of our potential competitors, alone or with their strategic partners, have substantially greater financial, technical and other resources, such as larger research and development, clinical, marketing and manufacturing organizations. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of competitors. Our commercial opportunity could be reduced or eliminated if competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Competitors also may obtain FDA or other regulatory approval for their products more rapidly or earlier than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against those of competitors.

In addition, as a result of the expiration or successful challenge of our patent rights, we could face more litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize.

Even if we obtain and maintain approval for our product candidates from the FDA, we may never obtain approval for our product candidates outside of the United States which would limit our market opportunities and harm our business.

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a

product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates by the European Commission in the European Union. However, obtaining such approval from the European Commission following the opinion of EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

Further, clinical trials conducted in one country may not be accepted by regulatory authorities in other countries. Also, regulatory approval for any of our product candidates may be withdrawn. If we fail to comply with the regulatory requirements, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed and our business, financial condition, results of operations and prospects will be harmed.

Risks Related to Our Financial Position

We have incurred substantial net losses since inception, and have only had one quarter since inception with profitability. We expect to incur losses for the foreseeable future and may never again achieve or maintain profitability.

Since inception, we have incurred substantial net losses. Our net losses for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2015 were $5.4 million, $4.0 million and $10.3 million, respectively. As of June 30, 2015, we had an accumulated deficit of $39.1 million. We historically have financed our operations primarily through private placements of our preferred stock and sublicensing rights to our NAV Technology Platform. We have devoted substantially all of our efforts to licensing our NAV Technology Platform and to research and development, including preclinical and planned clinical development of our product candidates, as well as to building out our team. We currently do not have any clinical programs, and we expect that it could be several years, if ever, before we commercialize an internal product candidate. We license certain intellectual property related to our NAV Technology Platform to third parties. Our NAV Technology Licensees have multiple preclinical studies and clinical trials in progress. However, no NAV Technology Licensee has an approved or commercialized gene therapy product based on such licensing program. We expect to generate only limited revenue, if any, from our current NAV Technology Licensees and any future NAV Technology Licensees in the near term. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- further develop our sublicensing activities and NAV Technology Platform;
- continue our research studies and preclinical development of our internal product candidates, including our Lead Product Candidates;
- initiate additional preclinical studies and clinical trials for our Lead Product Candidates and future product candidates, if any;
- initiate activities relating to manufacturing;
- seek to identify additional product candidates;
- prepare our BLA and MAA for our Lead Product Candidates and seek marketing approvals for any of our other product candidates that successfully complete clinical trials, if any;

- establish a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval, if any;
- operate as a public company;
- maintain, expand and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

For us to become profitable, we and our NAV Technology Licensees must develop and eventually commercialize product candidates with significant market potential. This will require us and our NAV Technology Licensees to be successful in a range of business challenges, including expansion of the licensing of our NAV Technology Platform, completing preclinical testing of our product candidates, commencing and completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we may obtain marketing approval and satisfying any post-marketing requirements. We may never succeed in any or all of these activities and, even if we do, we may never generate revenues that are sufficient to achieve profitability. If we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, maintain our research and development efforts, expand our business or continue our operations. A decline in the value of our company also could cause you to lose all or part of your investment.

Even if this offering is successful, we will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.

We will require substantial future capital in order to seek to broaden licensing of our NAV Technology Platform, complete the remaining research studies, preclinical and clinical development for our Lead Product Candidates and other future product candidates, if any, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical and clinical trials, if any, of our Lead Product Candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. As of June 30, 2015, our cash and cash equivalents were $85.2 million. We estimate that the net proceeds from this offering will be approximately $ million, based on an initial public offering price of $ per share, the midpoint of the range set forth on the cover of this prospectus, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We expect that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to initiate Phase I/II clinical trials for RGX-501 and RGX-111 and file an IND in preparation for a Phase I clinical trial for RGX-314, as well as fund our operating expenses and capital expenditure requirements through 2017. See “Use of Proceeds” for more information.

Our future capital requirements will depend on many factors, including:

- our planned expansion of the licensing of our NAV Technology Platform;
- the results of our preclinical studies for our Lead Product Candidates and any subsequent clinical trials;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials, if any, for our other product candidates;

- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements remaining in effect;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the costs associated with being a public company.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our licensing agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. Neither we nor any of our NAV Technology Licensees have commercialized any products using our NAV Technology Platform. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

To the extent that additional capital is raised through the sale of equity or equity-linked securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline and existing stockholders may not agree with our financing plans or the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

We have generated limited revenue from our NAV Technology Platform sublicensing and may not successfully expand our licensing activities.

Our ability to generate revenue from our NAV Technology Platform sublicensing depends on the acceptance by third parties of our NAV Technology Platform as their primary gene therapy technology and our ability to market and license our technology platform. We do not anticipate generating revenues from product sales for the next several years, if ever, as described elsewhere in these risk factors and anticipate generating only limited revenue from our NAV Technology Platform sublicensing in the near future. To date, a significant portion of our revenues have been generated from the sublicensing of rights to our NAV Technology Platform. Our ability to generate future revenues from our NAV Technology Platform sublicensing depends on many factors, including:

- our NAV Technology Licensees successfully developing gene therapy products using our NAV Technology Platform;

- obtaining and maintaining market acceptance of our NAV Technology Platform as a primary gene therapy technology;
- maintaining our licensing relationships with our licensor partners, including GlaxoSmithKline LLC (GSK) and The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn);
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any licensing or other arrangements into which we may enter and performing our obligations in such agreements;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference, infringement and other intellectual property related claims; and
- attracting, hiring and retaining qualified personnel.

We have never generated revenue from product candidate sales and have only generated limited revenue from reagent sales.

Our ability to generate revenue from product candidate sales depends on our ability, alone or with partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. All of our revenues to date have been from sublicensing our NAV Technology Platform and the sale of licensed reagents to third-parties for use in research and development. We do not consider reagent sales a core aspect of our business model and we do not dedicate significant resources to sales efforts for reagents. Accordingly, future revenue from reagent sales is uncertain and may fluctuate significantly from period to period. We do not anticipate generating revenues from our and our NAV Technology Licensees' product candidate sales for the next several years, if ever. Our ability to generate future revenues from product candidate sales depends heavily on our, or our NAV Technology Licensees', success in:

- completing research studies and preclinical and clinical development of our product candidates and identifying new gene therapy product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which clinical trials are completed;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- qualifying for adequate coverage and reimbursement by government and third-party payors for our product candidates;
- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for our product candidates, if approved;
- obtaining market acceptance of our product candidates as a viable treatment option;
- addressing any competing technological and market developments;

- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference, infringement and other intellectual property related claims; and
- attracting, hiring and retaining qualified personnel, including research and development, clinical development, regulatory and others.

Even if one or more of the product candidates that we develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA or other regulatory authorities to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Our limited operating history may make it difficult for you to evaluate the success of our business to date and to assess our future viability.

We are a preclinical company formed in July 2008. Our operations to date have been limited to organizing and staffing our company, business planning, raising capital, acquiring our technology, administering and expanding our NAV Technology Platform sublicensing, identifying potential product candidates and undertaking research and preclinical studies of our product candidates and establishing licensing arrangements. We have not yet demonstrated the ability to manage broad expansion of our NAV Technology Platform sublicensing efforts, complete and report preclinical or clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. Consequently, any predictions you make about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We will need to transition from a company with a licensing and research focus to a company that is also capable of supporting clinical development and commercial activities. We may not be successful in such a transition.

Our management will not be required to evaluate the effectiveness of our internal control over financial reporting until the end of the fiscal year for which our second annual report is due. If we are unable to maintain effective internal control over financial reporting, investors may lose confidence in the accuracy of our financial reports.

As a public company, we will be required to maintain internal control over financial reporting and to report any material weaknesses in such internal controls. Section 404 of the Sarbanes-Oxley Act requires that we evaluate and determine the effectiveness of our internal control over financial reporting. Beginning with our second annual report following this offering, we will be required to provide a management report on internal control over financial reporting. When we are no longer an emerging growth company, our management report on internal control over financial reporting will need to be attested to by our independent registered public accounting firm. We do not expect to have our independent registered public accounting firm attest to our management report on internal control over financial reporting while we are an emerging growth company.

If we have a material weakness in our internal control over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. In addition, our internal control over

financial reporting will not prevent or detect all errors and fraud. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

If there are material weaknesses or failures in our ability to meet any of the requirements related to the maintenance and reporting of our internal controls, investors may lose confidence in the accuracy and completeness of our financial reports and that could cause the price of our common stock to decline. In addition, we could become subject to investigations by NASDAQ, the SEC or other regulatory authorities, which could require additional management attention and which could adversely affect our business.

As described below we currently have two material weaknesses, which we are in the process of remediating.

In preparation for this offering, we identified two material weaknesses in our internal control over financial reporting. If we are unable to remedy our material weaknesses, or if we fail to establish and maintain effective internal controls, we may be unable to produce timely and accurate financial statements, and we may conclude that our internal control over financial reporting is not effective, which could adversely impact our investors' confidence and our stock price.

In connection with the audit of our financial statements as of and for the years ended December 31, 2014 and 2013, we identified two material weaknesses in our internal control over financial reporting. A material weakness is defined as a deficiency, or a combination of deficiencies, in internal control over financial reporting, such that there is a reasonable possibility that a material misstatement of our financial statements will not be prevented or detected on a timely basis.

First, we determined that we did not have adequate procedures and controls in our contract review process to ensure the completeness of contracts reviewed and to appropriately identify and account for provisions within our contracts. Second, we determined that we did not maintain a sufficient complement of resources to ensure adequate review and segregation of duties within our financial reporting processes.

These control deficiencies resulted in adjustments to our financial statements for 2013 and 2014 to revenues, equity, research and development, general and administrative, other operating income, and interest expense. Each of the control deficiencies could result in a misstatement of aforementioned accounts or disclosures that would result in a material misstatement of our annual or interim consolidated financial statements that would not be prevented or detected. Accordingly, our management has determined that these control deficiencies constitute material weaknesses.

We are in the process of implementing measures designed to improve internal control over financial reporting to remediate the control deficiencies that led to our material weaknesses. We cannot assure you that the measures we have taken to date, together with any measures we may take in the future, will be sufficient to remediate the control deficiencies that led to our material weaknesses in our internal control over financial reporting or to avoid potential future material weaknesses. In addition, neither our management nor an independent registered public accounting firm has ever performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act because no such evaluation has been required. Had we or our independent registered public accounting firm performed an evaluation of our internal control over financial reporting in accordance with the provisions of the Sarbanes-Oxley Act, additional material weaknesses may have been identified. If we are unable to successfully remediate our existing or any future material weaknesses in our internal control over financial reporting, or identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports in addition to applicable stock exchange listing requirements, investors may lose confidence in our financial reporting, and our stock price may decline as a result.

Risks Related to Third Parties

We rely primarily on a sponsored research agreement with The Trustees of the University of Pennsylvania for our nonclinical research and development activities and a loss of this relationship or of the principal investigator for that nonclinical research, James M. Wilson, M.D., Ph.D., would materially harm our business.

In February 2009, we entered into an exclusive worldwide license agreement with Penn for patent and other intellectual property rights relating to a gene therapy technology platform based on certain novel recombinant AAV vectors discovered at Penn in the laboratory of our Chief Scientific Advisor, James M. Wilson, M.D., Ph.D. This license was most recently amended in September 2014. In February 2009, we also entered into a sponsored research agreement (the 2009 SRA) with Penn pursuant to which we fund the nonclinical research of Dr. Wilson relating to AAV gene therapy and obtain an option to acquire an exclusive worldwide license in certain intellectual property created pursuant to such 2009 SRA. In December 2014, we entered into another sponsored research agreement (the 2014 SRA) with Penn funding related nonclinical research of Dr. Wilson.

Under the 2014 SRA, we fund nonclinical research at Penn and pay certain intellectual property legal and filing expenses and receive the rights to the research results. All patentable inventions conceived, created, or conceived and reduced to practice pursuant to the 2014 SRA, together with patent rights represented by or issuing from the United States patents and patent applications (including provisional patent applications) automatically become exclusively licensed to us under our existing licensing agreement with Penn and all research results are automatically licensed to us as know-how in our existing license agreement. The 2014 SRA will expire on December 31, 2016. We expect to amend the 2014 SRA in order to continue to fund work and receive rights to the results of the nonclinical research we fund at Penn. Also, a loss of our relationship with Penn or Dr. Wilson would materially harm our business.

We have in the past, and in the future plan to, enter into licensing agreements with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements are not successful, our business could be harmed.

We have entered into agreements involving the licensing of parts of our NAV Technology Platform and relating to the development and commercialization of certain product candidates and plan to enter into additional licensing agreements or collaborations in the future. We have limited control over the amount and timing of resources that our future collaborators or current and future partners, including our NAV Technology Licensees, dedicate to the development or commercialization of product candidates or of products utilizing licensed components of our NAV Technology Platform. Our ability to generate revenues from these arrangements will depend on our and our partners and collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our partners have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our collaborator is responsible could be harmful to the public perception and prospects of our gene delivery platform.

Any current or future licensing agreements or future collaborations we enter into may pose risks, including the following:

- licensees or collaborators have significant discretion in determining the efforts and resources that they will apply to these licensing agreements or collaborations;
- licensees or collaborators may not perform their obligations as expected;
- the clinical trials conducted as part of these licensing agreements or collaborations may not be successful;
- subjects in clinical trials undertaken by licensees or future collaborators, including our NAV Technology Licensees, may die or suffer adverse effects;

- licensees or collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the licensees' or collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- licensees or collaborators may delay clinical trials, provide insufficient funding for clinical trials, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- licensees or collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the licensees or collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates developed in collaboration with us may be viewed by our licensees or collaborators as competitive with their own product candidates or products, which may cause licensees or collaborators to cease to devote resources to the commercialization of our product candidates;
- a licensee or collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- disagreements with licensees or future collaborators, including disagreements over intellectual property and other proprietary rights, contract interpretation or the preferred course of development of any product candidates, may cause delays or termination of the research, development or commercialization of such product candidates, may lead to additional responsibilities for us with respect to such product candidates or may result in litigation or arbitration, any of which would be time-consuming and expensive;
- licensees or collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;
- disputes may arise with respect to the ownership of our other rights to intellectual property developed pursuant to our licensing agreements or collaborations;
- licensees or collaborators may infringe or otherwise violate the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- licensing agreements or collaborations may be terminated for the convenience of the licensee or collaborator and, if terminated, we could be required to raise additional capital to pursue further development or commercialization of the applicable product candidates.

If our licensing agreements or collaborations do not result in the successful development and commercialization of products, or if one of our licensees or collaborators terminates its agreement with us, we may not receive any future milestone or royalty payments, as applicable, under the collaboration. If we do not receive the payments we expect under these agreements, our development of product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and the perception of us in the business and financial communities could be harmed. All of the risks relating to product development, regulatory approval and commercialization described in this prospectus apply to the activities of our collaborators, including our license partners.

We may in the future decide to partner or collaborate with pharmaceutical and biotechnology companies for the development and potential commercialization of our product candidates. These relationships, or those like them, may require us to incur non-recurring and other charges, increase our near- and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive licensing agreement or collaboration agreement will depend, among other things, upon our assessment of the collaborator's resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator's evaluation of a variety of factors. If we license rights to product candidates, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate the licensed product candidates with our existing operations and company culture.

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing.

We may seek to establish strategic partnerships for developing and/or commercializing certain of our product candidates, due to capital costs required to develop the product candidates or manufacturing constraints. We may not be successful in our efforts to establish such a strategic partnership or other alternative arrangements for our product candidates because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy or market opportunity. In addition, we may be restricted under existing collaboration agreements from entering into future agreements with potential collaborators. We cannot be certain that, following a strategic transaction or license, we will achieve an economic benefit that justifies such transaction.

If we are unable to reach agreements with suitable licensees or collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, reduce or delay its development program, delay its potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to fund development or commercialization activities on our own, we may need to obtain additional expertise and additional capital, which may not be available to us on acceptable terms or at all. If we fail to enter into collaborations and do not have sufficient funds or expertise to undertake the necessary development and commercialization activities, we may not be able to further develop our product candidates and our business, financial condition, results of operations and prospects may be materially harmed.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

Because we will rely on third parties, including contractors, to research, develop and manufacture our product candidates, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, consulting agreements or other similar agreements with our advisors, employees, third-party contractors and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, including our trade secrets. Despite the contractual provisions employed when working with third parties, these provisions may be breached, and the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's independent discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may materially harm our business.

In addition, these agreements typically restrict the ability of our advisors, employees, third-party contractors and consultants to publish data potentially relating to our trade secrets, although our agreements may contain

certain limited publication rights. For example, any academic institution that we may collaborate with in the future will usually expect to be granted rights to publish data arising out of such collaboration, provided that we are notified in advance and given the opportunity to delay publication for a limited time period in order for us to secure patent protection of intellectual property rights arising from the collaboration, in addition to the opportunity to remove confidential or trade secret information from any such publication. In the future we may also conduct joint research and development programs that may require us to share trade secrets under the terms of our research and development or similar agreements. Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of our agreements with third parties, independent development or publication of information by any of our third-party collaborators. A competitor's discovery of our trade secrets would impair our competitive position and harm our business.

Risks Related to Manufacturing

Gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.

We currently have a development, manufacturing and testing agreement and cooperation agreement with WuXi AppTec, Inc. (WuXi) to manufacture supplies of our product candidates in the future. Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we employ multiple steps to control our manufacturing process to assure that the process works and the product candidate is made strictly and consistently in compliance with the process. Problems with the manufacturing process, even minor deviations from the normal process, could result in product defects or manufacturing failures that result in lot failures, product recalls, product liability claims or insufficient inventory. We may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet FDA, European Union or other comparable applicable foreign standards or specifications with consistent and acceptable production yields and costs. To date, no cGMP gene therapy manufacturing facility in the United States has received approval from the FDA for the manufacture of an approved gene therapy product, and, therefore, the timeframe required for us to obtain such approval is uncertain.

In addition, FDA, EMA and other comparable foreign regulatory authorities may require us to submit samples of any lot of any approved product together with the protocols showing the results of applicable tests at any time. Under some circumstances, the FDA, the EMA or other comparable foreign regulatory authorities may require that we not distribute a lot until the agency authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

We also may encounter problems hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to additional attractive development programs. Problems in third-party manufacturing process or facilities also could restrict our ability to meet market demand for our products. Additionally, should our agreement with WuXi be terminated for any reason, there are a limited number of manufacturers who would be suitable replacements and it would take a significant amount of time to transition the manufacturing to a replacement.

Delays in obtaining regulatory approval of our manufacturing process or disruptions in our manufacturing process may delay or disrupt our commercialization efforts. To date, no cGMP gene therapy manufacturing facility in the United States has received approval from the FDA for the manufacture of an approved gene therapy product.

Before we can begin to commercially manufacture our product candidates in third-party or our own facilities, we must obtain regulatory approval from the FDA for the manufacturing process and facility. A manufacturing authorization must also be obtained from the appropriate regulatory authorities in a European Union Member State. In addition, we must pass a pre-approval inspection of our manufacturing facility by the FDA before any of our product candidates can obtain marketing approval, if ever. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

We currently rely and expect to continue to rely on third parties to conduct our product manufacturing, and these third parties may not perform satisfactorily.

We do not currently plan to independently manufacture material for our planned preclinical and clinical programs. We currently rely, and expect to continue to rely, on third parties for the production of our preclinical study and planned clinical trial materials and, therefore, we can control only certain aspects of their activities.

In addition, we rely on additional third parties to manufacture ingredients of our product candidates and to perform quality testing, and reliance on these third parties entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- reduced control for certain aspects of manufacturing activities;
- termination or nonrenewal of manufacturing and service agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers and service providers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or service provider.

Any of these events could lead to clinical trial delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future product candidates. Some of these events could be the basis for FDA or European Union Member State regulatory authority action, including injunction, recall, seizure or total or partial suspension of product manufacture.

Any contamination in our manufacturing process, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our research studies, preclinical, clinical development or marketing schedules.

Given the nature of biologics manufacturing, there is a risk of contamination during manufacturing. Any contamination could materially harm our ability to produce product candidates on schedule and could harm our results of operations and cause reputational damage.

Some of the raw materials required in our manufacturing process are derived from biologic sources. Such raw materials are difficult to procure and may be subject to contamination or recall. A material shortage, contamination, recall or restriction on the use of biologically derived substances in the manufacture of our product candidates could adversely impact or disrupt the commercial manufacturing or the production of clinical material, which could materially harm our development timelines and our business, financial condition, results of operations and prospects.

Risks Related to the Commercialization of Our Product Candidates

If we are unable to establish sales, medical affairs and marketing capabilities or enter into agreements with third parties to market and sell our product candidates, if approved, we may be unable to generate any product revenue.

We currently have no products to sell and therefore no product sales and marketing organization. To successfully commercialize any products that may result from our development programs, we will need to develop these capabilities, either on our own or with others. The establishment and development of our own commercial team or the establishment of a contract sales force to market any products we may develop will be expensive and time-consuming and could delay any product launch. Moreover, we cannot be certain that we will be able to successfully develop this capability. We may enter into collaborations regarding one or more of our product candidates with other entities to utilize their marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current licensees or future licensees or collaborators do not commit sufficient resources to commercialize our products, or we are unable to develop the necessary capabilities on our own, we will be unable to generate sufficient product revenue to sustain our business. We compete with many companies that currently have extensive, experienced and well-funded medical affairs, marketing and sales operations to recruit, hire, train and retain marketing and sales personnel. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates. Without an internal team or the support of a third party to perform marketing and sales functions, we may be unable to compete successfully against these more established companies.

Our efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may never be successful. Such efforts may require more resources than are typically required due to the complexity and uniqueness of our potential products. If any of our product candidates is approved but fails to achieve market acceptance among physicians, patients or third-party payors, we will not be able to generate significant revenues from such product, which could materially harm our business, financial condition, results of operations and prospects.

If we do not achieve our projected development goals in the time frames we announce and expect, the commercialization of our products may be delayed and, as a result, our stock price may decline.

From time to time, we estimate the timing of the accomplishment of various scientific, clinical, regulatory and other product development goals, which we sometimes refer to as milestones. These milestones may include the commencement or completion of scientific studies and clinical trials and the submission of regulatory filings. From time to time, we may publicly announce the expected timing of some of these milestones. All of these milestones are and will be based on numerous assumptions. The actual timing of these milestones can vary dramatically compared to our estimates, in some cases for reasons beyond our control. If we do not meet these milestones as publicly announced, or at all, the commercialization of our products may be delayed or never achieved and, as a result, our stock price may decline.

Even if we obtain marketing approval for our Lead Product Candidates or any future product candidate, they could be subject to restrictions or withdrawal from the market, and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our product candidates, when and if any of them are approved.

Even if U.S. regulatory approval is obtained, the FDA may still impose significant restrictions on a product's indicated uses or marketing or impose ongoing requirements for potentially costly and time consuming post-approval studies, post-market surveillance or clinical trials. Following approval, if at all, of our Lead Product Candidates or any future product candidates, such candidate will also be subject to ongoing FDA requirements governing the labeling, packaging, storage, distribution, safety surveillance, advertising, promotion, recordkeeping and reporting of safety and other post-market information. In addition, manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with cGMP requirements relating to quality control, quality assurance and corresponding maintenance of records and documents. If we or a regulatory agency discovers previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured, a regulatory agency may impose restrictions on that product, the manufacturing facility or us, including requesting recall or withdrawal of the product from the market or suspension of manufacturing.

If regulatory approval is granted by the European Commission, or a comparable foreign regulatory authority, such approval can include restrictions and onerous post-authorization obligations similar to those that the FDA and other United States regulatory authorities have power to impose. These can include detailed pharmacovigilance obligations.

If we or the manufacturing facilities for our Lead Product Candidates or any future product candidate that may receive regulatory approval, if any, fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend, vary or withdraw regulatory approval;
- suspend any ongoing clinical trials;
- refuse to approve pending applications or supplements or applications filed by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of product or request us to initiate a product recall.

The occurrence of any event or penalty described above may inhibit our ability to commercialize our product candidates and generate revenue.

The FDA has the authority to require a REMS plan as part of a BLA or after approval, which may impose further requirements or restrictions on the distribution or use of an approved drug, such as limiting prescribing to certain physicians or medical centers that have undergone specialized training, limiting treatment to patients who meet certain safe-use criteria and requiring treated patients to enroll in a registry. The European Commission and comparable foreign regulatory authorities have powers to impose similar obligations.

In addition, if our Lead Product Candidates or any future product candidates are approved, our product labeling, advertising and promotion will be subject to regulatory requirements and continuing regulatory review. The FDA, the competent regulatory authorities in European Union Member States, and comparable foreign regulatory authorities strictly regulate the promotional claims that may be made about prescription products. In

particular, a product may not be promoted for uses that are not approved by the FDA, the European Commission, the competent regulatory authorities in European Union Member States, or comparable foreign regulatory authorities as reflected in the product's approved labeling. If we receive marketing approval for a product candidate, physicians may nevertheless prescribe it to their patients in a manner that is inconsistent with the approved label. If we are found to have promoted such off-label uses, we may become subject to significant liability. The FDA, other agencies, the competent regulatory authorities in European Union Member States, and comparable foreign regulatory authorities actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant sanctions. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. In the past, the FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Our gene therapy approach utilizes vectors derived from viruses which may be perceived as unsafe or may result in unforeseen adverse events. Negative public opinion and increased regulatory scrutiny of gene therapy may damage public perception of the safety of our product candidates and harm our ability to conduct our business or obtain regulatory approvals for our product candidates.

Gene therapy remains a novel technology, with no gene therapy product approved to date in the United States and only one gene therapy product approved to date in the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would harm our business, financial condition, results of operations and prospects and may delay or impair the development and commercialization of our product candidates or demand for any products we may develop. For example, earlier gene therapy trials led to several well-publicized adverse events, including cases of leukemia and death seen in other trials using other vectors. Serious adverse events in clinical trials we conduct, or other clinical trials involving our NAV Technology Platform by our NAV Technology Licensees or others, other gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

Even if we receive regulatory approval, we still may not be able to successfully commercialize our Lead Product Candidates or any future product candidate, and the revenue that we generate from any approved product's sales, if any, could be limited.

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. From time to time, public sentiment may be more adverse to commercialization of gene therapy as a therapeutic technique. Even with the requisite approvals from the FDA in the United States, the European Commission in the European Union and other comparable regulatory foreign authorities, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically necessary, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of our product candidates will depend on a number of factors, including:

- demonstration of clinical efficacy and safety compared to other more-established products;
- the limitation of our targeted patient population and other limitations or warnings contained in any FDA, European Commission, or other comparable foreign regulatory authority-approved labeling;

- acceptance of a new formulation by health care providers and their patients;
- the prevalence and severity of any adverse effects;
- new procedures or methods of treatment that may be more effective in treating or may reduce the conditions which our products are intended to treat;
- pricing and cost-effectiveness;
- the effectiveness of our or any future collaborators' sales and marketing strategies;
- our ability to obtain and maintain sufficient third-party coverage and reimbursement from government health care programs, including Medicare and Medicaid, private health insurers and other third-party payors;
- unfavorable publicity relating to product candidates or gene therapy generally; and
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage or reimbursement.

If any product candidate is approved but does not achieve an adequate level of acceptance by physicians, hospitals, healthcare payors or patients, we may not generate sufficient revenue from that product candidate and may not become or remain profitable. Our efforts to educate the medical community and third-party payors on the benefits of our Lead Product Candidates or any future product candidates may require significant resources and may never be successful. In addition, our ability to successfully commercialize our product candidates will depend on our ability to manufacture our products, differentiate our products from competing products and defend and enforce our intellectual property rights relating to our products. Additionally, if the market opportunities for our Lead Product Candidates or any future product candidates are smaller than we believe they are, our product revenues may be harmed and our business may suffer.

We focus our research and product development on treatments for severe genetic and orphan diseases. Our understanding of both the number of people who have these diseases, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are based on estimates. These estimates may prove to be incorrect and new studies may reduce the estimated incidence or prevalence of these diseases. The number of patients in the United States, the European Union and elsewhere may turn out to be lower than expected, may not be otherwise amenable to treatment with our products or patients may become increasingly difficult to identify and access, all of which would harm our business, financial condition, results of operations and prospects.

Further, there are several factors that could contribute to making the actual number of patients who receive any products we develop less than the potentially addressable market. These include the lack of widespread availability of, and limited reimbursement for, new therapies in many underdeveloped markets. Further, the severity of the progression of a disease up to the time of treatment, especially in certain degenerative conditions such as MPS I, MPS II, wet AMD and XLRP, will likely diminish the therapeutic benefit conferred by a gene therapy due to irreversible cell death. Lastly, certain patients' immune systems might prohibit the successful delivery of certain gene therapy products to the target tissue, thereby limiting the treatment outcomes.

The insurance coverage and reimbursement status of newly-approved products is uncertain. Failure to obtain or maintain adequate coverage and reimbursement for our products, if approved, could limit our ability to market those products and decrease our ability to generate product revenue.

We expect the cost of a single administration of gene therapy products, such as those we are developing, to be substantial, when and if they achieve regulatory approval. We expect that coverage and reimbursement by government and private payors will be essential for most patients to be able to afford these treatments. Accordingly, sales of our product candidates will depend substantially, both domestically and abroad, on the extent to which the costs of our product candidates will be paid by health maintenance, managed care, pharmacy

benefit and similar healthcare management organizations, or will be reimbursed by government authorities, private health coverage insurers and other third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

- a covered benefit under its health plan;
- safe, effective and medically necessary;
- appropriate for the specific patient;
- cost-effective; and
- neither experimental nor investigational.

Obtaining coverage and reimbursement for a product from third-party payors is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data. There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. We may not be able to provide data sufficient to gain acceptance with respect to coverage and reimbursement. If coverage and reimbursement are not available, or are available only at limited levels, we may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be adequate to realize a sufficient return on our investment.

Additionally, our Lead Product Candidates are designed to provide therapeutic benefit after a single administration and, therefore, the pricing and reimbursement of a single administration of our product candidates, if approved, must be adequate to support our commercial infrastructure. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

If we obtain approval to commercialize our product candidates outside of the United States, in particular in the European Union, a variety of risks associated with international operations could materially harm our business.

We expect that we will be subject to additional risks in commercializing our product candidates outside the United States, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, floods and fires.

Risks Related to our Business Operations

We may not be successful in our efforts to identify or discover additional product candidates and may fail to capitalize on programs or product candidates that may be a greater commercial opportunity or for which there is a greater likelihood of success.

The success of our business depends upon our ability to identify, develop and commercialize product candidates based on our NAV Technology Platform. Research programs to identify new product candidates require substantial technical, financial and human resources. Although certain of our product candidates are currently in research studies or preclinical development, we may fail to identify potential product candidates for clinical development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

Additionally, because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our spending on current and future research and development programs may not yield any commercially viable products. If we do not accurately evaluate the commercial potential for a particular product candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate. Alternatively, we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement.

If any of these events occur, we may be forced to abandon our development efforts with respect to a particular product candidate or fail to develop a potentially successful product candidate, which could materially harm our business, financial condition, results of operations and prospects.

Our future success depends on our ability to retain key employees, consultants and advisors and to attract, retain and motivate qualified personnel.

We are highly dependent on members of our executive team, including, without limitation, Kenneth T. Mills, our Chief Executive Officer; Stephen Yoo, M.D., our Chief Medical Officer; and Vittal Vasista, our Chief Financial Officer; and scientific advisors, including, without limitation, James M. Wilson, M.D., Ph.D., our Chief Scientific Advisor; the loss of any of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements with each of our executive officers, any of them could leave our employment at any time, as all of our employees are “at will” employees. We currently do not have “key person” insurance on any of our employees. The loss of the services of one or more of our current employees, consultants and advisors might impede the achievement of our research, development, licensing and commercialization objectives.

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which we believe is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, including, without limitation, Kenneth T. Mills, our Chief Executive Officer; Stephen Yoo, M.D., our Chief Medical Officer; and Vittal Vasista, our Chief Financial

Officer; key employees, consultants or advisors, including, without limitation, James M. Wilson, M.D., Ph.D., our Chief Scientific Advisor; may impede the progress of our research, development, licensing and commercialization objectives and materially harm our business, financial condition, results of operations and prospects. Additionally, our current management team has only been working together for a relatively short period of time and a number of members of our current management team have been employed by us for less than a year. We will also need to expand our current accounting and finance teams with additional qualified personnel to ensure proper internal control over financial reporting.

If we are unable to manage expected growth in the scale and complexity of our operations, our performance may suffer.

If we are successful in executing our business strategy, we will need to expand our managerial, operational, financial and other systems and resources to manage our operations, continue our research and development and licensing activities and, in the longer term, build a sales and marketing infrastructure to support commercialization of any of our product candidates that are approved for sale. Future growth would impose significant added responsibilities on members of management. It is likely that our management, finance, development personnel, systems and facilities currently in place may not be adequate to support this future growth. Our need to effectively manage our operations, growth and product candidates requires that we continue to develop more robust business processes and improve our systems and procedures in each of these areas and to attract and retain sufficient numbers of talented employees. We may be unable to successfully implement these tasks on a larger scale and, accordingly, may not achieve our research, development and growth goals.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

Healthcare legislative reform measures may materially harm our business and results of operations.

In the United States, there have been, and continue to be, several legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act (PPACA), was passed. PPACA made major changes in how healthcare is delivered and reimbursed, and increased access to health insurance benefits to the uninsured and underinsured population of the United States.

PPACA, among other things, increased the number of individuals with Medicaid and private insurance coverage, implemented reimbursement policies that tie payment to quality, facilitated the creation of accountable care organizations that may use capitation and other alternative payment methodologies, strengthened enforcement of fraud and abuse laws and encouraged the use of information technology. Many of these changes require implementing regulations which have not yet been drafted or have been released only as proposed rules.

Such changes in the regulatory environment may also result in changes to our payor mix that may affect our operations. While PPACA is expected to increase the number of persons with covered health benefits, we cannot accurately estimate the payment rates for any additional persons that are expected to be covered by health benefits. For example, PPACA's expansion of Medicaid coverage could cause patients who otherwise would have selected private healthcare to participate in government sponsored healthcare programs, and Medicaid and other government programs typically reimburse providers at substantially lower rates than private payors. Our revenue may be adversely impacted if states pursue lower rates or cost-containment strategies as a result of any expansion of their existing Medicaid programs to include additional persons, particularly in states experiencing budget deficits. Exchanges created to facilitate coverage for new persons to be covered by health benefits may also place additional pricing pressure on all providers, regardless of payor. The full impact of many of the provisions under PPACA is unknown at this time. For example, PPACA established an Independent Payment Advisory Board that can recommend changes in payment for physicians under certain circumstances, which the Department of Health and Human Services (HHS) generally would be required to implement unless Congress enacts superseding legislation. PPACA also requires HHS to develop a budget-neutral, value-based payment modifier that provides for differential payment under the Medicare Physician Fee Schedule (the MPFS) for physicians or groups of physicians that is linked to quality of care furnished compared to cost. Physicians in groups of 100 or more eligible professionals who submit claims to Medicare under a single tax identification number will be subject to the value modifier beginning this year, based on their performance in previous years. For example, in 2015, this modifier is based on performance during calendar year 2013, and in 2016, it will be based on performance during calendar year 2014. The modifier will apply to all other physicians by 2017. HHS has not yet developed a value-based payment modifier option for hospital-based physicians.

In November 2012, CMS adopted a rule under the PPACA that generally allowed physicians in certain specialties who provide eligible primary care services to be paid at the Medicare rates in effect in calendar years 2013 and 2014 instead of state-established Medicaid reimbursement rates, referred to as the Medicaid-Medicare Parity. Generally, state Medicaid reimbursement rates are lower than federally established Medicare rates. During 2013, state agencies were required to submit their state plan amendments (SPAs) outlining how they will implement the rule, including frequency and timing of payments to CMS for review and approval. In December 2013, CMS indicated that all SPAs had been approved for enhanced Medicaid-Medicare Parity reimbursement through December 2014. Congress did not act before the end of the year to extend the Medicaid-Medicare Parity and the rule expired. Legislation has been proposed to retroactively extend Medicaid-Medicare Parity for calendar year 2015 but has not yet been enacted. Certain states have decided to fully or partially extend the Medicaid-Medicare Parity. It is unclear at this time how these limited state increases or the continued failure to extend the rule at the federal level will impact our business.

Finally, other legislative changes have been proposed and adopted in the United States since the PPACA was enacted. On August 2, 2011, the Budget Control Act of 2011 was enacted, which, among other things, created the Joint Select Committee on Deficit Reduction, or the Joint Committee, to recommend proposals in spending reductions to Congress. The Joint Committee did not achieve a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This included aggregate reductions to Medicare payments to providers of two percent per fiscal year, which went into effect on April 1, 2013 and, due to subsequent legislative amendments to the statute, will remain in effect through 2024 unless additional Congressional action is taken. On January 2, 2013, the American Taxpayer Relief Act was signed into law, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts

that federal and state governments and other third-party payors will pay for healthcare products and services, which could adversely affect our business, financial condition and results of operations.

Additionally, in the United States, the Biologics Price Competition and Innovation Act of 2009 created an abbreviated approval pathway for biologic products that are demonstrated to be "highly similar" or "biosimilar or interchangeable" with an FDA-approved biologic product. This new pathway could allow competitors to reference data from biologic products already approved after 12 years from the time of approval. This could expose us to potential competition by lower-cost biosimilars even if we commercialize a product candidate faster than our competitors.

We may be subject, directly or indirectly, to federal and state healthcare fraud and abuse laws, false claims laws and health information privacy and security laws. If we are unable to comply, or have not fully complied, with such laws, we could face substantial penalties.

If we obtain FDA, European Commission, or other comparable foreign regulatory authorities' approval for any of our product candidates and begin commercializing those products in the United States or outside the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal, state and foreign fraud and abuse laws and regulations, including, without limitation, the federal Health Care Program Anti-Kickback Statute, the federal civil and criminal False Claims Act and Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to patient privacy laws by both the federal government and the states in which we conduct our business. The laws that will affect our operations include, but are not limited to:

- the federal Health Care Program Anti-Kickback Statute, which prohibits, among other things, persons or entities from knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe or rebate), directly or indirectly, overtly or covertly, in cash or in kind, in return for the purchase, recommendation, leasing or furnishing of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand, and prescribers, purchasers and formulary managers on the other. The PPACA amends the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- federal civil and criminal false claims laws and civil monetary penalty laws which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid or other government payors that are false or fraudulent. The PPACA provides and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH), and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act;
- Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;

- federal transparency laws, including the federal Physician Payment Sunshine Act, that require disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and
- state and foreign law equivalents of each of the above federal laws, state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts in certain circumstances, such as specific disease states.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities could be subject to challenge under one or more of such laws. If our operations are found to be in violation of any of the laws described above or any other government regulations that apply to us, we may be subject to penalties, including civil and criminal penalties, damages, fines, exclusion from participation in government health care programs, such as Medicare and Medicaid, imprisonment and the curtailment or restructuring of our operations, any of which could harm our ability to operate our business and our results of operations.

The provision of benefits or advantages to physicians to induce or encourage the prescription, recommendation, endorsement, purchase, supply, order or use of medicinal products is prohibited in the European Union. The provision of benefits or advantages to physicians is also governed by the national anti-bribery laws of European Union Member States, such as the UK Bribery Act 2010. Infringement of these laws could result in substantial fines and imprisonment.

Payments made to physicians in certain European Union Member States must be publically disclosed. Moreover, agreements with physicians often must be the subject of prior notification and approval by the physician's employer, his or her competent professional organization and/or the regulatory authorities of the individual European Union Member States. These requirements are provided in the national laws, industry codes or professional codes of conduct, applicable in the European Union Member States. Failure to comply with these requirements could result in reputational risk, public reprimands, administrative penalties, fines or imprisonment.

The collection and use of personal health data in the European Union is governed by the provisions of the Data Protection Directive. This Directive, and the national implementing legislation of the individual European Union Member States, imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, notification of data processing obligations to the competent national data protection authorities and the security and confidentiality of the personal data. The Data Protection Directive also imposes strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of the Data Protection Directive and the related national data protection laws of the European Union Member States may result in fines and other administrative penalties. The draft Data Protection Regulation currently going through the adoption process is expected to introduce new data protection requirements in the European Union and substantial fines for breaches of the data protection rules. If the draft Data Protection Regulation is adopted in its current form it may increase our responsibility and liability in relation to personal data that we process and we may be required to put in place additional mechanisms ensuring compliance with the new data protection rules. This may be onerous and harm our business, financial condition, results of operations and prospects.

Product liability lawsuits against us could cause us to incur substantial liabilities and could limit licensing of our NAV Technology Platform or commercialization of any product candidates that we may develop.

We face an inherent risk of product liability exposure related to our licensed NAV Technology Platform and the testing of our product candidates in clinical trials and may face an even greater risk if products utilizing our

NAV Technology Platform are commercialized. If we cannot successfully defend ourselves against claims that our technology or product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for our technology, including any product candidates that we may develop;
- loss of revenue;
- substantial monetary awards to trial participants or patients;
- significant time and costs to defend the related litigation;
- withdrawal of clinical trial participants;
- the inability to license our NAV Technology Platform or commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

Although we currently maintain product liability insurance coverage in the amount of $5.0 million per occurrence and $5.0 million in the aggregate, our product liability insurance coverage may not be adequate to cover all liabilities that we may incur. We anticipate that we will need to increase our insurance coverage each time we commence a clinical trial and if we successfully commercialize any product candidates. Insurance coverage is increasingly expensive. We may not be able to maintain insurance coverage at a reasonable cost or in an amount adequate to satisfy any liability that may arise.

If we or our development partners, including our NAV Technology Licensees, fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could materially harm the success of our business.

We and our development partners, including our NAV Technology Licensees, are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the generation, handling, use, storage, treatment, manufacture, transportation and disposal of, and exposure to, hazardous materials and wastes, as well as laws and regulations relating to occupational health and safety. Our operations involve the use of hazardous and flammable materials, including chemicals and biologic and radioactive materials. Our and our development partners', including our NAV Technology Licensees', operations also produce hazardous waste products. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our or our development partners', including our NAV Technology Licensees', use of hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

Although we maintain workers' compensation insurance in the amount of up to $100,000 per occurrence for certain costs and expenses we may incur due to injuries to our employees resulting from work related injuries, this insurance may not provide adequate coverage against potential liabilities. We do not maintain insurance for toxic tort claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair us or our development partners', including our NAV Technology Licensees', research, development or production efforts. Failure to comply with these laws and regulations also may result in substantial fines, penalties or other sanctions or liabilities, which could materially harm our business, financial condition, results of operations and prospects.

We and our development partners, third-party manufacturer and suppliers use biological materials and may use hazardous materials, and any claims relating to improper handling, storage or disposal of these materials could be time consuming or costly.

We and our development partners, including our NAV Technology Licensees, third-party manufacturer and suppliers may use hazardous materials, including chemicals and biological agents and compounds that could be dangerous to human health and safety or the environment. Our operations and the operations of our third-party manufacturers and suppliers also produce hazardous waste products. Federal, state and local laws and regulations, and comparable foreign laws, govern the use, generation, manufacture, storage, handling and disposal of these materials and wastes. Compliance with applicable environmental laws and regulations may be expensive, and current or future environmental laws and regulations may impair our product development efforts. In addition, we cannot entirely eliminate the risk of accidental injury or contamination from these materials or wastes. We do not carry specific biological or hazardous waste insurance coverage, and our property, casualty and general liability insurance policies (under which we currently have $3.0 million in coverage) specifically exclude coverage for damages and fines arising from biological or hazardous waste exposure or contamination. Accordingly, in the event of contamination or injury, we could be held liable for damages or be penalized with fines in an amount exceeding our resources, and our clinical trials or regulatory approvals could be suspended.

Unfavorable global economic conditions could harm our business, financial condition or results of operations.

Our results of operations could be harmed by general conditions in the global economy and in the global financial markets. The most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the most recent global financial crisis, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. This is particularly true in the European Union, which is undergoing a continued severe economic crisis. A weak or declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payors or our future collaborators. Any of the foregoing could harm our business and we cannot anticipate all of the ways in which the current economic climate and financial market conditions could harm our business.

We and third parties on which we rely may be harmed by natural disasters and our business continuity and disaster recovery plans may not adequately protect us from a serious disaster.

Natural disasters could severely disrupt our operations or the operations of our third parties' manufacturing facilities and materially harm our business, financial condition, results of operations and prospects. If a natural disaster, power outage or other event occurred that prevented us from using all or a significant portion of our headquarters, that damaged critical or that otherwise disrupted operations, it may be difficult or, in certain cases, impossible for us to continue our business for a substantial period of time. The disaster recovery and business continuity plans we have in place currently are limited and may not prove adequate in the event of a serious disaster or similar event. Our third party manufacturing facilities, as well as substantially all of our current supply of product candidates, are located in Pennsylvania, and we do not have any existing back-up facilities in place or plans for such back-up facilities. We may incur substantial expenses as a result of the limited nature of our disaster recovery and business continuity plans, which could materially harm our business, financial condition, results of operations and prospects.

Our internal computer systems, or those of our future collaborators or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our licensing and product development programs.

Our internal computer systems and those of our current and any future collaborators and other contractors or consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system

failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our licensing and development programs and our business operations, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability, our competitive position could be harmed and the further licensing of our NAV Technology Platform and development and commercialization of our product candidates could be delayed.

Our customers are concentrated and therefore the loss of a significant customer may harm our business.

We rely on third parties for aspects of our business. Our revenue for the fiscal years ended December 31, 2013 and 2014 consisted of license revenue, grant revenue and the sale of licensed reagents to third-parties for use in research and development. Three customers accounted for approximately 76% of our total revenue for the year ended December 31, 2013. No other customer accounted for more than 10% of revenue in 2013. Two customers accounted for approximately 47% of our total revenue for the year ended December 31, 2014. No other customer accounted for more than 10% of revenue in 2014. Future license revenue is uncertain due to the contingent nature of our licenses granted to third-parties. We expect grant revenue to decrease in the future as we are not currently seeking any further grant awards. We do not consider reagent sales a core aspect of our business model and we do not dedicate significant resources to sales efforts for reagents. Accordingly, future revenue from sales of reagents is uncertain and may fluctuate significantly from period to period.

Risks Related to Our Intellectual Property

Our rights to license our NAV Technology Platform and to develop and commercialize our product candidates are subject, in part, to the terms and conditions of licenses granted to us by others.

We do not currently own any patents or wholly own any pending patent applications, and we are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our gene therapy product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to license our platform or develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories not included in all of our licenses. For example, under our license agreement with GSK, GSK retained certain exclusive and non-exclusive rights under the patent rights that it licensed from Penn. See “Business—License Agreements and Commercial Licenses—GlaxoSmithKline LLC” for more information.

Licenses to additional third-party technology that may be required for our licensing or development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could materially harm our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering technology that we license from third parties. For example, under our license agreement with Penn, Penn is entitled to control the preparation, prosecution and maintenance of the patent rights licensed to us. However, if we determine that we desire a greater degree of control over such patent rights, the Penn license agreement provides that Penn will work in good faith with us to enter into an arrangement for such additional control with reimbursement by us of certain expenses. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be impacted. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the United States government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the United States. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for our products and technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully license our NAV Technology Platform and commercialize our products and technology may be harmed.

Our success depends, in large part, on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary NAV Technology Platform, our product candidates and our manufacturing technology. Our licensors have sought and we intend to seek to protect our proprietary position by filing patent applications in the United States and abroad related to many of our novel technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates have expired or will soon expire. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

We are a party to intellectual property license agreements with Penn and GSK, each of which is important to our business, and we expect to enter into additional license agreements in the future. Our existing license agreements impose, and we expect that future license agreements will impose, various diligence, development and commercialization timelines, milestone payments, royalties and other obligations on us. See “Business—License Agreements and Commercial Licenses.” If we fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we would not be able to market products covered by the license.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has, in recent years, been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection.

We may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after

filing or, in some cases, not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and product candidates. Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. As a result, our intellectual property may not provide us with sufficient rights to exclude others from commercializing products similar or identical to ours.

Our intellectual property licenses with third parties may be subject to disagreements over contract interpretation, which could narrow the scope of our rights to the relevant intellectual property or technology or increase our financial or other obligations to our licensors.

The agreements under which we currently license intellectual property or technology from third parties are complex, and certain provisions in such agreements may be susceptible to multiple interpretations. The resolution of any contract interpretation disagreement that may arise could narrow what we believe to be the scope of our rights to the relevant intellectual property or technology, or increase what we believe to be our financial or other obligations under the relevant agreement, either of which could materially harm our business, financial condition, results of operations and prospects.

If we fail to comply with our obligations in the agreements under which we license intellectual property rights from third parties or otherwise experience disruptions to our business relationships with our licensors, we could lose license rights that are important to our business.

We have entered into license agreements with third parties and may need to obtain additional licenses from others to advance our research, to expand our licensing program or to allow commercialization of our product candidates. It is possible that we may be unable to obtain additional licenses at a reasonable cost or on reasonable terms, if at all. In that event, we may be required to expend significant time and resources to redesign our technology or product candidates or the methods for manufacturing them or to develop or license replacement technology, all of which may not be feasible on a technical or commercial basis. If we are unable to do so, we may be unable to redesign our platform technology or to develop or commercialize the affected product candidates, which could materially harm our business. We cannot provide any assurances that third-party patents do not exist which might be enforced against our current platform technology, manufacturing methods, product candidates or future methods or products, resulting in either an injunction prohibiting our licensing, manufacture or sales, or, with respect to our sales, an obligation on our part to pay royalties and/or other forms of compensation to third parties.

In each of our existing license agreements, and we expect in our future agreements, patent prosecution of our licensed technology is controlled primarily by the licensor, and we are required to reimburse the licensor for certain costs of patent prosecution and maintenance. If our licensors fail to obtain and maintain patent or other protection for the proprietary intellectual property we license from them, we could lose our rights to the

intellectual property or our exclusivity with respect to those rights, and our competitors could market competing products using the intellectual property. Further, in each of our license agreements we could be responsible for bringing actions against any third party for infringing on the patents we have licensed if our licensor elects not to enforce its rights against the infringing third party. Certain of our license agreements in which we are the licensee also require us to meet development milestones to maintain the license, including establishing a set timeline for developing and commercializing products and minimum diligence obligations in developing and commercializing the product. Disputes may arise regarding intellectual property subject to a licensing agreement, including:

- the scope of rights granted under the license agreement and other interpretation-related issues;
- the extent to which our technology and processes infringe on or otherwise violate intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other intellectual property rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We may not be successful in obtaining necessary rights to our product candidates through acquisitions and in-licenses.

We currently have rights to intellectual property, through licenses from third parties, to license our NAV Technology Platform and develop our product candidates. Because our programs may require the use of intellectual property or other proprietary rights held by third parties, the growth of our business may depend, in part, on our ability to acquire, in-license or use such intellectual property and proprietary rights. We may be unable to acquire or in-license any compositions, methods of use, processes (and patents for such technology) or other intellectual property rights from third parties that we identify as necessary for our technology platform and product candidates. The licensing or acquisition of third-party intellectual property rights is a competitive area, and several more established companies may pursue strategies to license or acquire third-party intellectual property rights that we may consider attractive. These established companies may have a competitive advantage over us due to their size, capital resources and greater clinical development and commercialization capabilities. In addition, companies that perceive us to be a competitor may be unwilling to assign or license rights to us. We also may be unable to license or acquire third party intellectual property rights on terms that would allow us to make an appropriate return on our investment.

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Some of these institutions provide us with an option to negotiate a license to any of the institution’s rights in technology resulting from the collaboration, and under our relationship with Penn, any patentable inventions developed under our 2014 SRA automatically accrue to our existing license with Penn. Regardless of such option, we may be unable to negotiate a license within the specified timeframe or under terms that are acceptable to us. If we are unable to do so, the institution may offer the intellectual property rights to other parties, potentially blocking our ability to pursue our program.

If we are unable to successfully obtain rights to required third-party intellectual property rights or maintain the existing intellectual property rights we have, we may have to abandon development of the relevant program or product candidate and our business, financial condition, results of operations and prospects could suffer.

Obtaining and maintaining our patent protection depends on compliance with various procedural, document submission, fee payment and other requirements imposed by government patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.

Periodic maintenance fees, renewal fees, annuity fees and various other government fees on patents and/or applications will be due to be paid to the United States Patent and Trademark Office (USPTO) and various patent agencies outside of the United States over the lifetime of our licensed patents and/or applications and any patent rights we may own or license in the future. We rely on our licensing partners to pay these fees due to non-U.S. patent agencies with respect to our licensed patent rights. The USPTO and various non-U.S. government patent agencies require compliance with several procedural, documentary, fee payment and other similar provisions during the patent application process. We employ reputable law firms and other professionals to help us comply and we are also dependent on our licensors to take the necessary action to comply with these requirements with respect to our licensed intellectual property. In many cases, an inadvertent lapse can be cured by payment of a late fee or by other means in accordance with the applicable rules. There are situations, however, in which non-compliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, potential competitors might be able to enter the market and this circumstance could materially harm our business.

We may not be able to protect our intellectual property rights throughout the world.

Filing, prosecuting and defending patents on our platform technology or product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. Although Penn, GSK and ARIAD Pharmaceuticals, Inc. (ARIAD) license agreements grant us worldwide rights, certain of our in-licensed U.S. patent rights lack corresponding foreign patents or patent applications. For example, under our license agreement with the Regents of the University of Minnesota, the territory is limited to those countries and territories, including the United States, in which a licensed patent has issued and is unexpired or a licensed patent application is pending. See “Business—License Agreements and Commercial Licenses” for more information regarding these license agreements. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

Issued patents covering our NAV Technology Platform or our product candidates could be found invalid or unenforceable if challenged in court. We may not be able to protect our trade secrets in court.

If one of our licensing partners or we initiate legal proceedings against a third party to enforce a patent covering our NAV Technology Platform or one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including subject-matter eligibility, novelty, non-obviousness, written description or enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld information material to patentability from the USPTO, or made a misleading statement, during prosecution. Third parties also may raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, *inter partes* review and equivalent proceedings in foreign jurisdictions. Such proceedings could result in the revocation or cancellation of or amendment to our patents in such a way that they no longer cover our NAV Technology Platform or our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which the patent examiner and we or our licensing partners were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity or unenforceability, we could lose at least part, and perhaps all, of the patent protection on one or more of our product candidates. Such a loss of patent protection could materially harm our business.

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology, product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

Third parties may initiate legal proceedings alleging that we are infringing their intellectual property rights, the outcome of which would be uncertain and could materially harm our business.

Our commercial success depends, in part, upon our ability to license our NAV Technology Platform, and on our NAV Technology Licensees' ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and *inter partes* review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially harm our ability to license our technology platform or

commercialize our Lead Product Candidates or any future product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue licensing, developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease licensing, developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from licensing our technology platform or manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could similarly harm our business, financial condition, results of operations and prospects.

Intellectual property litigation could cause us to spend substantial resources and distract our personnel from their normal responsibilities.

Competitors may infringe our patents or the patents of our licensing partners, or we may be required to defend against claims of infringement or that our intellectual property is invalid or unenforceable. To counter infringement or unauthorized use claims or to defend against claims of infringement or other intellectual property related claims can be expensive and time consuming. Even if resolved in our favor, litigation or other legal proceedings relating to intellectual property claims may cause us to incur significant expenses, and could distract our technical and management personnel from their normal responsibilities. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments and if securities analysts or investors perceive these results to be negative, it could materially harm the price of our common stock. Such litigation or proceedings could substantially increase our operating losses and reduce the resources available for development activities or any future sales, marketing or distribution activities. We may not have sufficient financial or other resources to adequately conduct such litigation or proceedings. Some of our competitors may be able to sustain the costs of such litigation or proceedings more effectively than we can because of their greater financial resources and more mature and developed intellectual property portfolios. Uncertainties resulting from the initiation and continuation of patent and other intellectual property litigation or proceedings could materially harm our ability to compete in the marketplace.

We may be subject to claims asserting that our employees, consultants or advisors have wrongfully used or disclosed alleged trade secrets of their current or former employers or claims asserting ownership of what we regard as our own intellectual property.

Many of our employees, consultants or advisors are currently, or were previously, employed at universities or other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we try to ensure that our employees, consultants and advisors do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these individuals or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such individual's current or former employer. Litigation may be necessary to defend against these claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in defending against such claims, litigation could result in substantial costs and be a distraction to management.

In addition, while it is our policy to require our employees and contractors who may be involved in the conception or development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who, in fact, conceives or develops intellectual property that we regard as our own. The assignment of intellectual property rights may not be self-executing or the assignment agreements may be breached, and we may be forced to bring claims against third parties, or defend claims that they may bring against us, to determine the ownership of what we regard as our intellectual property.

Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act (the Leahy-Smith Act) was signed into law. The Leahy-Smith Act includes several significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted and also may affect patent litigation. These also include provisions that switched the United States from a "first-to-invent" system to a "first-to-file" system, allow third-party submission of prior art to the USPTO during patent prosecution and set forth additional procedures to attack the validity of a patent by the USPTO administered post grant proceedings. Under a first-to-file system, assuming the other requirements for patentability are met, the first inventor to file a patent application generally will be entitled to the patent on an invention regardless of whether another inventor had made the invention earlier. The USPTO recently developed new regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, only became effective on March 16, 2013. Accordingly, it is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could materially harm our business, financial condition, results of operations and prospects.

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court of the United States (Supreme Court). On March 20, 2012, the Supreme Court issued a decision in Mayo Collaborative Services v. Prometheus Laboratories, Inc. (Prometheus) a case involving patent claims directed to a process of measuring a metabolic product in a patient to optimize a drug dosage for the patient. According to the Supreme Court, the addition of well-understood, routine or conventional activity such as "administering" or "determining" steps was not enough to transform an otherwise patent-ineligible natural phenomenon into patent-eligible subject matter. On July 3, 2012, the USPTO issued a guidance memo to patent examiners indicating that process claims directed to a law of nature, a natural phenomenon or a naturally occurring relation or correlation that do not include additional elements or steps that integrate the natural principle into the claimed invention such that the natural principle is practically applied and the claim amounts to significantly more than the natural principle itself should be rejected as directed to not patent-eligible subject matter. On June 13, 2013, the Supreme Court issued its decision in Association for Molecular Pathology v. Myriad Genetics, Inc. (Myriad) a case involving patent claims held by Myriad relating to the breast cancer susceptibility genes BRCA1 and BRCA2. Myriad held that an isolated segment of naturally occurring DNA, such as the DNA constituting the BRCA1 and BRCA2 genes, is not patent eligible subject matter, but that complementary DNA, which is an artificial construct that may be created from RNA transcripts of genes, may be patent eligible.

The USPTO issued a number of Interim Guidance memoranda on patent eligibility under 35 U.S.C. §101 in 2014 and 2015 to instruct USPTO examiners on the ramifications of the Prometheus and Myriad rulings and the application of the Myriad ruling to natural products and principles including all naturally occurring nucleic acids. On March 4, 2014, the USPTO issued a guidance memorandum to patent examiners entitled *2014 Procedure for Subject Matter Eligibility Analysis of Claims Reciting or Involving Laws of Nature/Natural Principles, Natural Phenomena, and/or Natural Products*. In response to public feedback, these Guidelines were superseded by the

Interim Eligibility Guidance in December 2014, and again updated in January 2015. It is expected that the guidance will be further updated in view of developments in the case law and in response to public feedback. Patents for certain of our product candidates contain claims related to specific DNA sequences that are naturally occurring and, therefore, could be the subject of future challenges made by third parties. In addition, the recent USPTO guidance could make it impossible for us to pursue similar patent claims in patent applications we may prosecute in the future.

We cannot assure you that our efforts to seek patent protection for our technology and products will not be negatively impacted by the decisions described above, rulings in other cases or changes in guidance or procedures issued by the USPTO. We cannot fully predict what impact the Supreme Court's decisions in Prometheus and Myriad may have on the ability of life science companies to obtain or enforce patents relating to their products and technologies in the future. These decisions, the guidance issued by the USPTO and rulings in other cases or changes in USPTO guidance or procedures could materially harm our existing patent portfolio and our ability to protect and enforce our intellectual property in the future.

Moreover, although the Supreme Court has held in Myriad that isolated segments of naturally occurring DNA are not patent-eligible subject matter, certain third parties could allege that activities that we may undertake infringe other gene-related patent claims, and we may deem it necessary to defend ourselves against these claims by asserting non-infringement and/or invalidity positions, or paying to obtain a license to these claims. In any of the foregoing or in other situations involving third-party intellectual property rights, if we are unsuccessful in defending against claims of patent infringement, we could be forced to pay damages or be subjected to an injunction that would prevent us from utilizing the patented subject matter. Such outcomes could harm our business, financial condition, results of operations or prospects.

If we do not obtain patent term extension and data exclusivity for our product candidates, our business may be materially harmed.

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Amendments). The Hatch-Waxman Amendments permit a patent extension term of up to five years as compensation for patent term lost during the FDA regulatory review process. A patent term extension cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval, only one patent may be extended and only those claims covering the approved drug, a method for using it or a method for manufacturing it may be extended. However, we may not be granted an extension because of, for example, failing to exercise due diligence during the testing phase or regulatory review process, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

If our trademarks and trade names are not adequately protected, then we may not be able to build name recognition in our markets of interest and our business may be harmed.

We have pending trademark applications with the USPTO for the mark "REGENXBIO" and the REGENXBIO logo, approval of which is not guaranteed. Once registered, our trademarks or trade names may be challenged, infringed, circumvented or declared generic or determined to be infringing on other marks. We may not be able to protect our rights to these trademarks and trade names, which we need to build name recognition among potential partners or customers in our markets of interest. At times, competitors may adopt trade names or trademarks similar to ours, thereby impeding our ability to build brand identity and possibly leading to market confusion. In addition, there could be potential trade name or trademark infringement claims brought by owners

of other registered trademarks or trademarks that incorporate variations of our registered or unregistered trademarks or trade names. Over the long term, if we are unable to establish name recognition based on our trademarks and trade names, then we may not be able to compete effectively and our business may be harmed. Our efforts to enforce or protect our proprietary rights related to trademarks, trade secrets, domain names, copyrights or other intellectual property may be ineffective and could result in substantial costs and diversion of resources and could harm our financial condition or results of operations.

Intellectual property rights do not necessarily address all potential threats.

The degree of future protection afforded by our intellectual property rights is uncertain because intellectual property rights have limitations, and may not adequately protect our business or permit us to maintain our competitive advantage. For example:

- others may be able to make gene therapy products that are similar to our product candidates or utilize similar gene therapy technology but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending licensed patent applications or those that we may own in the future will not lead to issued patents;
- issued patents that we hold rights to may be held invalid or unenforceable, including as a result of legal challenges by our competitors;
- our competitors might conduct research and development activities in countries where we do not have patent rights and then use the information learned from such activities to develop competitive products for sale in our major commercial markets;
- we may not develop additional proprietary technologies that are patentable;
- the patents of others may harm our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

Should any of these events occur, they could materially harm our business, financial condition, results of operations and prospects.

Risks Related to Our Common Stock and this Offering

The trading price of our common stock is likely to be volatile, and you might not be able to sell your shares at or above the initial public offering price.

There has been no public market for our common stock prior to this offering, and the initial public offering price of our common stock was determined by negotiations between us and the underwriters and may not be indicative of the future prices of our common stock. The market price of our common stock could be subject to wide fluctuations in response to various factors, many of which are beyond our control. These factors include those discussed elsewhere in this “Risk Factors” section and others such as:

- the delay or failure in initiating or completing preclinical studies or clinical trials, or unsatisfactory results of these trials;

- announcements about us or about our competitors including clinical trial results, regulatory approvals, or new product candidate introductions;
- developments concerning our current or future development partners, licensors or product candidate manufacturers;
- developments or changing views regarding the use of gene therapy;
- litigation and other developments relating to our patents or other proprietary rights or those of our competitors;
- conditions in the pharmaceutical or biotechnology industries and the economy as a whole;
- governmental regulation and legislation;
- the recruitment or departure of members of our board of directors, management team or other key personnel, including recruitment of a new chief executive officer;
- changes in our operating results;
- any changes in the financial projections we may provide to the public, our failure to meet these projections, or changes in recommendations by any securities analysts that elect to follow our common stock;
- any change in securities analysts' estimates of our performance, or our failure to meet analysts' expectations;
- the expiration of market standoff or contractual lock-up agreements;
- sales or potential sales of substantial amounts of our common stock; and
- price and volume fluctuations in the overall stock market or resulting from inconsistent trading volume levels of our shares.

In recent years, the stock market in general, and the market for pharmaceutical and biotechnology companies in particular, has experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to changes in the operating performance of the companies whose stock is experiencing those price and volume fluctuations. Broad market and industry factors may seriously affect the market price of our common stock, regardless of our actual operating performance. These fluctuations may be even more pronounced in the trading market for our stock shortly following this offering.

As a newly public company, our stock price may be volatile, and securities class action litigation has often been instituted against companies following periods of volatility of their stock price. Any such litigation, if instituted against us, could result in substantial costs and a diversion of our management's attention and resources.

In the past, following periods of volatility in the overall market and the market price of a particular company's securities, securities class action litigation has often been instituted against these companies. This litigation, if instituted against us, could result in substantial costs and a diversion of our management's attention and resources.

No public market for our common stock currently exists, and an active trading market may not develop or be sustained following this offering.

Prior to this offering, there has been no public market for our common stock. An active trading market may not develop following the closing of this offering or, if developed, may not be sustained. The lack of an active market may impair your ability to sell your shares at the time you wish to sell them or at a price that you consider reasonable. The lack of an active market may also reduce the fair market value of your shares. An inactive market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

If securities analysts do not publish research or publish unfavorable research about our business, our stock price and trading volume could decline.

The trading market for our common stock will depend in part on the research and reports that securities and industry analysts publish about us or our business. We do not currently have and may never obtain research coverage by securities analysts. If no or few securities or industry analysts commence coverage of our company, the trading price for our stock could suffer. In the event we obtain securities or industry analyst coverage, if one or more of the analysts who covers us downgrades our stock or publishes unfavorable research about our business, or if our clinical trials or operating results fail to meet the analysts' expectations, our stock price would likely decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which could cause our stock price and trading volume to decline.

As an investor participating in this offering, you will experience immediate substantial dilution as a result of this offering and future equity issuances.

The initial public offering price per share is substantially higher than the pro forma net tangible book value per share of our common stock outstanding prior to this offering. As a result, investors purchasing common stock in this offering will experience immediate substantial dilution of $ per share, based on the initial public offering price of $ per share, which is the midpoint of the initial public offering price range reflected on the cover page of this prospectus. In addition, to the extent currently outstanding options or warrants are exercised, there will be further dilution to investors in this offering. In addition, we may raise additional capital through public or private equity or debt offerings, subject to market conditions. To the extent that additional capital is raised through the sale of equity or convertible debt securities, the issuance could result in further dilution to our stockholders.

Raising additional funds by issuing securities or through licensing or lending arrangements may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.

We will need to raise additional funding. To the extent that we raise additional capital by issuing equity securities, the share ownership of existing stockholders will be diluted. Any future debt financing may involve covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, redeem our stock, make certain investments, and engage in certain merger, consolidation, or asset sale transactions. In addition, if we seek funds through arrangements with collaborative partners, these arrangements may require us to relinquish rights to some of our technologies or products or otherwise agree to terms unfavorable to us.

Our management will have broad discretion over the actual amounts and timing of the expenditures of the proceeds we receive in this offering and might not apply the proceeds in ways that enhance our operating results or increase the value of your investment.

We expect to use the net proceeds from this offering primarily to initiate clinical trials, as well as for working capital and general corporate purposes. Our management will have broad discretion as to the actual amounts and timing of the expenditures of the net proceeds from this offering, and you will be relying on the judgment of our management regarding the application of these proceeds. Our management might not apply the net proceeds of this offering in ways that enhance our operating results or increase the value of your investment. Additionally, until the net proceeds we receive are used, they may be placed in investments that do not produce income or that lose value. See "Use of Proceeds" located elsewhere in this prospectus.

We have never paid and do not intend to pay cash dividends and, consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.

We have never paid cash dividends on any of our capital stock, and we currently intend to retain future earnings, if any, to fund the development and growth of our business. Therefore, you are not likely to receive any

dividends on our common stock for the foreseeable future or at all. Since we do not intend to pay dividends, your ability to receive a return on your investment will depend on any future appreciation in the market value of our common stock. There is no guarantee that our common stock will appreciate or even maintain the price at which you have purchased it.

Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.

As of June 30, 2015, our executive officers, directors, holders of more than five percent of our capital stock and their respective affiliates beneficially owned 63.7% of our outstanding capital stock and, upon the closing of this offering, that same group will beneficially own % of our outstanding capital stock (assuming no exercise of the underwriters' option to purchase additional shares). Therefore, these stockholders will have the ability to influence us through their ownership position after this offering. These stockholders may be able to determine all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

Substantial future sales of shares by existing stockholders, or the perception that such sales may occur, could cause our stock price to decline.

If our existing stockholders, particularly our directors and executive officers and the venture capital funds affiliated with our current and former directors, sell substantial amounts of our common stock in the public market, or are perceived by the public market as intending to sell substantial amounts of our common stock, the trading price of our common stock could decline below the initial public offering price. Based on 19,050,708 shares outstanding as of June 30, 2015, upon completion of this offering, we will have outstanding shares of common stock. Of these shares, only the shares of common stock sold in this offering and registered shares issued pursuant to our equity plans will be freely tradable in the public market, subject to any applicable lock-up agreements or Rule 144 transfer restrictions applicable to affiliates. Our officers, directors and holders of substantially all of our equity securities have entered into contractual lock-up agreements with the underwriters pursuant to which they have agreed, subject to certain exceptions, not to sell or otherwise transfer any of their common stock or securities convertible into or exchangeable for shares of common stock for a period of 180 days after the date of the final prospectus for this offering. However, we and the lead underwriters in this offering may permit these holders to sell shares prior to the expiration of the lock-up agreements with the underwriters.

Based on shares outstanding as of June 30, 2015 and the shares of common stock issuable upon conversion of all outstanding preferred stock, after the contractual lock-up agreements pertaining to this offering expire 180 days from the date of this prospectus, up to an additional 19,050,708 shares will be eligible for sale in the public market, 12,144,487 of which are held by directors, executive officers and other affiliates and will be subject to volume and other limitations under Rule 144 under the Securities Act.

The 3,063,200 shares that were subject to outstanding options as of June 30, 2015 will become eligible for sale in the public market to the extent permitted by the provisions of various vesting agreements, the contractual lock-up agreements, and Rules 144 and 701 under the Securities Act.

Some of our existing security holders have demand and piggyback rights to require us to register with the SEC up to 16,298,045 shares of our common stock, subject to expiration of the contractual lock-up agreements. If we register these shares of common stock, the stockholders would be able to sell those shares freely in the public market, subject to Rule 144 transfer restrictions applicable to affiliates.

We plan to register an additional shares of our common stock that we may issue under our equity plans. Once we issue these shares, they can be freely sold in the public market upon issuance, subject to any vesting restriction, contractual lock-up agreements, or Rule 144 transfer restrictions applicable to affiliates.

If any of these additional shares described are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline. For additional information, see “Shares Eligible for Future Sale.”

We will incur increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the SEC and NASDAQ have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance. Overall, we estimate that our incremental cost resulting from operating as a public company will be between $1.0 million and $3.0 million per year although it is possible that our actual incremental costs will be higher than we currently estimate.

Pursuant to Section 404 we will be required to furnish a report by our management on our internal control over financial reporting, including an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will incur costs associated with the remediation of material weakness identified in our internal control over financial reporting. We estimate these costs will be between $0.5 million and $1.0 million per year. Additionally, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude within the prescribed timeframe that our internal control over financial reporting is effective as required by Section 404. This could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Provisions in our restated certificate of incorporation and amended and restated bylaws and Delaware law might discourage, delay or prevent a change in control of our company or changes in our management and, therefore, depress the market price of our common stock.

Our restated certificate of incorporation and amended and restated bylaws contain provisions that could depress the market price of our common stock by acting to discourage, delay or prevent a change in control of our company or changes in our management that the stockholders of our company may deem advantageous. These provisions among other things:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit the board of directors to establish the number of directors;
- provide that directors may only be removed “for cause”;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and amended and restated bylaws;
- authorize the issuance of “blank check” preferred stock that our board of directors could use to implement a stockholder rights plan;

- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;
- provide that the board of directors is expressly authorized to make, alter or repeal our bylaws; and
- establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

In addition, Section 203 of the Delaware General Corporation Law may discourage, delay or prevent a change in control of our company. Section 203 imposes certain restrictions on merger, business combinations and other transactions between us and holders of 15% or more of our common stock.

For information regarding these and other provisions, see "Description of Capital Stock."

Our restated certificate of incorporation will designate the Court of Chancery of the State of Delaware as the exclusive forum for certain litigation that may be initiated by our stockholders, which could limit our stockholders' ability to obtain a favorable judicial forum for disputes with us.

Pursuant to our restated certificate of incorporation, as will be in effect upon the completion of this offering, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or other employees to us or our stockholders, (3) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our restated certificate of incorporation or our amended and restated bylaws or (4) any action asserting a claim governed by the internal affairs doctrine. The forum selection clause in our restated certificate of incorporation may limit our stockholders' ability to obtain a favorable judicial forum for disputes with us, our directors, officers or other employees.

We are an emerging growth company and the reduced disclosure and governance requirements applicable to emerging growth companies could make our common stock less attractive to investors.

We are an emerging growth company. Under the Jumpstart Our Business Startups Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

For as long as we continue to be an emerging growth company, we intend to take advantage of certain other exemptions from various reporting requirements that are applicable to other public companies, including reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, exemptions from the requirements of holding a nonbinding advisory stockholder vote on executive compensation and any golden parachute payments not previously approved, exemption from the requirement of auditor attestation on our internal control over financial reporting and exemption from any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements (auditor discussion and analysis). If we do, the information that we provide stockholders may be different than what is available with respect to other public companies.

Investors could find our common stock less attractive because we will rely on these exemptions, which may make it more difficult for investors to compare our business with other companies in our industry. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. In addition, it may be difficult for us to raise additional capital as and when we need it. If we are unable to do so, our financial condition and results of operations could be materially harmed.

We will remain an emerging growth company until the earliest of (1) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds $700.0 million as of the end of the second fiscal quarter, (2) the end of the fiscal year in which we have total annual gross revenue of $1.0 billion or more during such fiscal year, (3) the date on which we issue more than $1.0 billion in non-convertible debt in a three-year period or (4) December 31, 2020, the end of the fiscal year following the fifth anniversary of the completion of this offering.

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus contains forward-looking statements that involve risks and uncertainties. The forward-looking statements are contained principally in "Prospectus Summary," "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and "Business." In some cases, you can identify forward-looking statements by terms such as "anticipate," "believe," "contemplates," "continue," "could," "design," "estimate," "expect," "intend," "likely," "may," "ongoing," "plan," "potential," "predict," "project," "will," "would," "seek," "should," "target," or the negative of these terms, and similar expressions intended to identify forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Forward-looking statements include, but are not limited to, statements about:

- the timing and success of preclinical studies and clinical trials conducted by us and our development partners;
- the ability to obtain and maintain regulatory approval of our product candidates, and the labeling for any approved products;
- the scope, progress, expansion, and costs of developing and commercializing our product candidates;
- our ability to obtain and maintain intellectual property protection for our product candidates;
- our anticipated growth strategies;
- our expectations regarding competition;
- the anticipated trends and challenges in our business and the market in which we operate;
- our ability to attract or retain key personnel;
- the size and growth of the potential markets for our product candidates and the ability to serve those markets;
- the rate and degree of market acceptance of any of our product candidates;
- our ability to establish and maintain development partnerships;
- our expectations regarding federal, state and foreign regulatory requirements;
- regulatory developments in the United States and foreign countries; and
- our use and sufficiency of our existing cash resources and proceeds from this offering.

Forward-looking statements involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances or achievements expressed or implied by the forward-looking statements.

Any forward-looking statement made by us in this prospectus speaks only as of the date on which it is made. Except as required by law, we assume no obligation to update these statements publicly, or to update the reasons actual results could differ materially from those anticipated in these statements, even if new information becomes available in the future.

We discuss many of these risks in this prospectus in greater detail under the heading "Risk Factors."

Unless required by United States federal securities laws, we do not intend to update any of these forward-looking statements to reflect circumstances or events that occur after the statement is made.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of our forward-looking statements by these cautionary statements.

USE OF PROCEEDS

We estimate that our net proceeds from the sale of the common stock that we are offering will be approximately $, assuming an initial public offering price of $ per share, the midpoint of the initial public offering price range reflected on the cover page of this prospectus, and after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters' option to purchase additional shares in this offering is exercised in full, we estimate that our net proceeds will be approximately $. A $1.00 increase (decrease) in the assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, would increase (decrease) the estimated net proceeds to us by $ million, assuming that the number of shares offered by us as set forth on the cover page of this prospectus remains the same and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase (decrease) of 1.0 million in the number of shares offered by us would increase (decrease) the net proceeds to us by $ million, assuming an initial public offering price of $ per share, the midpoint of the price range set forth on the cover page of this prospectus, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

As of June 30, 2015, we had cash and cash equivalents of $85.2 million. We intend to use net proceeds from this offering, together with existing cash resources, as follows:

- approximately $ million to fund external research and development expenses to advance our lead product candidate RGX-501 for the treatment of HoFH through Phase I/II clinical trials;
- approximately $ million to fund external research and development expenses to advance our product candidate RGX-111 for the treatment of MPS I through Phase I/II clinical trials;
- approximately $ million to fund external research and development expenses to advance our product candidate RGX-314 for the treatment of wet AMD through filing of an IND in preparation for a Phase I clinical trial;
- approximately $ million to fund research and development expenses of our other internally developed product candidates and to identify and advance new programs or product candidates into preclinical studies; and
- the remainder for working capital, general and administrative expenses, internal research and development expenses, manufacturing and other general corporate purposes, including in-licenses and potential acquisitions.

We believe opportunities may exist from time to time to expand our current business through acquisitions or in-licenses of complementary products or technologies or acquisitions of companies with complementary products or technologies. We have no current agreements, commitments or understandings for any specific acquisitions or in-licenses at this time; however, we may use a portion of the net proceeds for these purposes.

The expected use of net proceeds of this offering represents our current intentions based upon our present plans and business conditions. The amounts we actually expend in these areas may vary significantly from our current intentions and will depend upon a number of factors, including future sales growth, success of our product development and commercialization efforts, cash generated from future operations, if any, and actual expenses to operate our business. As of the date of this prospectus, we cannot specify with certainty all of the particular uses for the net proceeds to be received upon the closing of this offering. Accordingly, our management will have broad discretion in the application of the net proceeds, and investors will be relying on the judgment of our management regarding the application of the net proceeds of this offering.

Pending use of proceeds from this offering, we intend to invest the proceeds in a variety of capital preservation investments, including short-term, investment-grade, interest-bearing instruments.

DIVIDEND POLICY

We have never declared or paid cash dividends on our common or preferred stock. We currently intend to retain all available funds and any future earnings for use in the operation of our business and do not anticipate paying any cash dividends in the foreseeable future. Any future determination to declare cash dividends will be made at the discretion of our board of directors and will depend on our financial condition, results of operations, capital requirements, general business conditions, and other factors that our board of directors may deem relevant.

CAPITALIZATION

The following table sets forth our cash and cash equivalents and our capitalization as of June 30, 2015:

- on an actual basis;
- on a pro forma basis to reflect the automatic conversion of all outstanding shares of preferred stock into 16,298,045 shares of our common stock prior to the completion of this offering; and
- on a pro forma as adjusted basis to additionally reflect the issuance and sale by us of shares of our common stock in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus.

You should read this information together with our financial statements and related notes appearing elsewhere in this prospectus and the information set forth under the heading "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

(in thousands, except share and per share data)	As of June 30, 2015		
	Actual	Pro Forma	Pro Forma As Adjusted
Cash and cash equivalents	$ 85,215	$ 85,215	
Convertible preferred stock:			
Series A convertible preferred stock; $0.0001 par value; 2,393,127 shares authorized, issued and outstanding at June 30, 2015, and no shares issued and outstanding pro forma and pro forma as adjusted	3,000	—	—
Series B convertible preferred stock; $0.0001 par value; 1,906,295 shares authorized, issued and outstanding at June 30, 2015, and no shares issued and outstanding pro forma and pro forma as adjusted	7,892	—	—
Series C convertible preferred stock; $0.0001 par value; 4,631,774 shares authorized, issued and outstanding at June 30, 2015, and no shares issued and outstanding pro forma and pro forma as adjusted	30,000	—	—
Series D convertible preferred stock; $0.0001 par value; 7,366,849 shares authorized, issued and outstanding at June 30, 2015, and no shares issued and outstanding pro forma and pro forma as adjusted	70,500	—	
Stockholders' equity (deficit):			
Common stock; $0.0001 par value; 23,100,000 shares authorized, actual and pro forma; 2,752,663 shares issued and outstanding at June 30, 2015; 19,050,708 shares issued and outstanding pro forma; shares authorized and shares issued and outstanding pro forma as adjusted	—	2	—
Preferred stock; $0.0001 par value; no shares authorized, issued and outstanding, actual, 10,000,000 shares authorized, and no shares issued and outstanding pro forma and pro forma as adjusted	—	—	—
Additional paid-in-capital	10,346	121,736	—
Accumulated deficit	(39,110)	(39,110)	—
Total stockholders' (deficit) equity	(28,764)	82,628	
Total capitalization	$ 82,628	$ 82,628	

The actual, pro forma and pro forma as adjusted outstanding shares information in the table above excludes the following:

- 3,063,200 shares of common stock issuable upon the exercise of options outstanding as of June 30, 2015 under the 2014 Stock Plan at a weighted average exercise price of $1.86 per share;
- 927,100 shares of common stock reserved for issuance under our 2014 Stock Plan; and
- shares of common stock reserved for issuance under our 2015 Equity Incentive Plan, which became effective in June 2015 but with respect to which no awards will be granted prior to the effective date of the registration statement of which this prospectus is a part, and shares of common stock reserved for issuance under our 2015 Employee Stock Purchase Plan which becomes effective on the effective date of the registration statement of which this prospectus is a part, subject in each case to automatic annual adjustment in accordance with the terms of the plan.

DILUTION

If you invest in our common stock in this offering, your interest will be diluted to the extent of the difference between the initial public offering price per share of our common stock in this offering and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

As of June 30, 2015, we had pro forma net tangible book value of $81.6 million, or $4.28 per share of common stock, after taking into account the expected conversion of our outstanding convertible preferred stock into common stock. Without giving effect to the conversion of our outstanding preferred stock into common stock, we had a historical net tangible book value (deficit) of $(29.8) million, or $(10.83) per share of common stock, as of June 30, 2015. Historical net tangible book value (deficit) per share is equal to our total tangible assets, less total liabilities and convertible preferred stock, divided by the number of outstanding shares of our common stock. After giving effect to (1) the conversion of all of our outstanding convertible preferred stock into 16,298,045 shares of common stock prior to the completion of this offering, and (2) the sale of shares of common stock in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us, at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, our pro forma as adjusted net tangible book value as of June 30, 2015 would have been approximately $ million, or approximately $ per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of $ per share to our existing stockholders and an immediate dilution of $ per share to investors participating in this offering. The following table illustrates this per share dilution:

Assumed initial public offering price per share		$
Historical net tangible book value (deficit) per share as of June 30, 2015	$(10.83)	
Increase per share attributable to assumed conversion of convertible preferred stock	15.11	
Pro forma net tangible book value per share as of June 30, 2015	4.28	
Increase in pro forma net tangible book value per share attributable to investors participating in this offering		
Pro forma as adjusted net tangible book value per share after this offering		
Pro forma as adjusted dilution per share to purchasers of common stock in this offering		$

Each $1.00 increase (decrease) in the assumed initial public offering price of $ per share would increase (decrease) our pro forma as adjusted net tangible book value by approximately $ million, the pro forma as adjusted net tangible book value per share by approximately $ per share and the dilution to investors purchasing shares in this offering by approximately $ per share, assuming the number of shares offered by us, as set forth on the cover of this prospectus, remains the same and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We may also increase or decrease the number of shares we are offering. An increase of 1.0 million shares in the number of shares offered by us would increase our pro forma net tangible book value (deficit) by approximately $, or $ per share, and the pro forma dilution per share to purchasers of common stock in this offering would be $ per share, assuming that the assumed initial public offering price remains the same, and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us. Similarly, a decrease of 1.0 million shares in the number of shares offered by us would decrease our pro forma net tangible book value by approximately $, or $ per share, and the pro forma dilution per share to purchasers of common stock in this offering would be $ per share, assuming that the assumed initial public offering price remains the same, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. The pro forma information discussed above is illustrative only and will adjust based on the actual initial public offering price and other terms of this offering determined at pricing.

If the underwriters exercise their option to purchase additional shares of our common stock in full in this offering, the pro forma net tangible book value per share after this offering would be $ per share, the

increase in pro forma net tangible book value per share to existing stockholders would be $ per share and the dilution to purchasers of common stock in this offering would be $ per share.

The following table summarizes, on a pro forma as adjusted basis as of June 30, 2015, the differences between the number of shares of common stock purchased from us, the total consideration and the average price per share paid by existing stockholders (giving effect to the conversion of all of our convertible preferred stock into 16,298,045 shares of common stock outstanding on June 30, 2015, prior to the completion of this offering) and by investors participating in this offering, after deducting estimated underwriting discounts and commissions and estimated offering expenses, at an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus.

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders					
Purchasers of common stock in this offering					
Totals		100.0%		100.0%	

The foregoing tables exclude:

- 3,063,200 shares of common stock issuable upon the exercise of options outstanding as of June 30, 2015 under the 2014 Stock Plan at a weighted average exercise price of $1.86 per share;
- 927,100 shares of common stock reserved for issuance under our 2014 Stock Plan; and
- shares of common stock reserved for issuance under our 2015 Equity Incentive Plan, which became effective in June 2015 but with respect to which no awards will be granted prior to the effective date of the registration statement of which this prospectus is a part, and shares of common stock reserved for issuance under our 2015 Employee Stock Purchase Plan which becomes effective on the effective date of the registration statement of which this prospectus is a part, subject in each case to automatic annual adjustment in accordance with the terms of the plan.

Furthermore, we may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. New investors will experience further dilution if any of our outstanding options are exercised, new options are issued and exercised under our equity incentive plans or we issue additional shares of common stock, other equity securities or convertible debt securities in the future.

SELECTED FINANCIAL DATA

The selected statements of operation data for the years ended December 31, 2013 and 2014 are derived from our audited financial statements appearing elsewhere in this prospectus. The selected financial data as of June 30, 2015 and for the six months ended June 30, 2014 and 2015 have been derived from our unaudited financial statements included elsewhere in this prospectus. In our opinion, these unaudited financial statements have been prepared on a basis consistent with our audited financial statements and contain all adjustments, consisting only of normal and recurring adjustments, necessary for a fair statement of such financial data. You should read this data together with our audited financial statements and related notes appearing elsewhere in this prospectus and the information under the caption "Management's Discussion and Analysis of Financial Condition and Results of Operations." Our historical results are not necessarily indicative of our future results, and our operating results for the six-month period ended June 30, 2015 are not necessarily indicative of the results that may be expected for the fiscal year ending December 31, 2015 or any other interim periods or any future year or period.

(in thousands, except per share data)	Year Ended December 31,		Six Months Ended June 30,	
	2013	2014	2014	2015
Statements of operations data:				
Revenues				
License revenue	$ 1,055	$ 4,355	$3,705	$ 570
License revenue from related party	2,700	220	—	1,000
Reagent sales	368	326	291	148
Grant revenue	1,964	1,219	490	289
Total revenues	6,087	6,120	4,486	2,007
Expenses				
Costs of revenues				
Licensing costs to related parties	151	885	741	314
Costs of reagent sales (including amounts to related parties)	173	122	102	49
Research and development (including amounts to related parties)	5,051	4,961	1,787	6,803
General and administrative (including amounts to related parties)	5,474	3,851	1,660	5,113
Foreign currency transaction losses (gains)	14	30	(14)	38
Other operating income	—	(47)	(24)	(21)
Total operating expenses	10,863	9,802	4,252	12,296
Income (loss) from operations	(4,776)	(3,682)	234	(10,289)
Other income (expense)				
Investment income	—	—	—	8
Interest expense	(611)	(321)	(111)	(20)
Total other income (expense)	(611)	(321)	(111)	(12)
Net income (loss)	(5,387)	(4,003)	123	(10,301)
Accretion and dividends on convertible preferred stock and preferred units	(422)	(815)	(467)	(1,747)
Net gain on extinguishment of preferred stock	—	—	—	759
Net loss applicable to common stockholders and members	$ (5,809)	$(4,818)	$ (344)	$ (11,289)
Net loss attributable to common stockholders per share:				
Basic and diluted	$ (2.50)	$ (1.82)	$ (0.13)	$ (4.21)
Weighted average common shares outstanding:				
Basic and diluted	2,320	2,643	2,643	2,679

(in thousands)	As of December 31, 2013	As of December 31, 2014	As of June 30, 2015
Balance sheet data:			
Cash and cash equivalents	$ 1,119	$ 1,121	$ 85,215
Working capital (deficit)	(2,446)	(6,158)	81,051
Total assets	2,510	3,491	88,800
Accrued expenses	194	1,115	3,062
Other related party payables	3,503	3,761	1,919
Total liabilities	4,653	9,189	6,172
Convertible preferred stock and preferred units	11,778	12,593	111,392
Total stockholders' and members' deficit	(13,921)	(18,291)	(28,764)

MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes thereto included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks and uncertainties. As a result of many factors, including those factors set forth in the "Risk Factors" section of this prospectus, our actual results could differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis. All amounts are expressed in thousands other than share and per share amounts.

Overview

We are a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. In AAV gene therapy, the viral genes are removed from the AAV, a small, non-pathogenic cold virus, creating a biological delivery vehicle called a vector. A therapeutic gene sequence is then inserted, creating a recombinant vector. Our proprietary AAV gene delivery platform (our NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10 (NAV Vectors). Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing gene therapy products administered directly into the body, or *in vivo*, based on our NAV Technology Platform. We seek to accomplish our mission through a combination of our internal development efforts and the efforts of our third-party licensees (NAV Technology Licensees). Our NAV Technology Platform is currently being applied in the development of 23 product candidates for a variety of diseases, including five internally developed product candidates and 18 partnered product candidates developed by our NAV Technology Licensees.

We are applying our NAV Technology Platform to generate a broad pipeline of best-in-class and often first-in-class AAV gene therapy treatments. Our NAV Technology Platform is covered by more than 100 licensed patents and patent applications worldwide. Our product candidates, which are designed for a variety of diseases, incorporate proprietary advances in AAV gene therapy that significantly enhance their profiles as potential therapeutics. The benefits of our NAV Technology Platform have been observed across several clinical trials and studies conducted by our development partners and third-party investigators. Approximately 70% of all AAV gene therapy clinical trials relating to new treatment INDs posted on the United States' government clinical trials database from 2012 through 2014 used our NAV Vectors.

Financial Overview

Revenue

We classify our revenue into three categories: license revenue, grant revenue and reagent sales. To date, we have generated limited revenue through our licensing agreements with our NAV Technology Licensees for research, development and commercialization of product candidates using our proprietary technology. Additionally, we have generated limited revenue from grant programs and sales of licensed reagents to customers for use in research and development. We have not generated any revenue from sales of approved products or drug therapies. If we fail to complete the development of our product candidates in a timely manner, or fail to obtain their regulatory approval, our ability to generate future revenue will be compromised.

License Revenue

We have granted a number of intellectual property licenses to other biotechnology and pharmaceutical companies.

The terms of our license agreements require delivery of a license for use of our intellectual property in either research only, or in research and commercial development of drug therapies for various diseases. License agreements generally have a term equal to the life of the intellectual property, but are terminable at the option of

the licensee. Non-refundable payments to us under these arrangements may include: (i) up-front license fees, (ii) option fees to exercise options to obtain commercial licenses, (iii) annual maintenance fees, (iv) sublicense fees, (v) payments based on the achievement of certain milestones and (vi) royalties on product sales. Due to the contingent nature of option fees, sublicense fees, milestone payments and future royalties on product sales under our licensing arrangements, future license revenue is dependent on the successful development and commercialization of products by our licensees, which is uncertain and may fluctuate significantly from period to period.

Nonrefundable up-front license fees are recognized as revenue upon delivery of the license, provided there are no undelivered elements in the arrangement and the necessary criteria under ASC 605-45, *Revenue Recognition—Principal Agent Considerations* for revenue recognition have been met.

License revenue from a related party consists of license fees from licenses granted to Dimension Therapeutics, Inc. (Dimension).

Grant Revenue

Grant revenue is generated through research and development grant programs offered by the United States federal government and the European Union.

In December 2012, as part of a consortium of research and development entities called MeuSIX, we were awarded a long-term grant by the European Commission's Seventh Framework Program to perform preclinical and clinical research and development services for the treatment of MPS VI, a severe lysosomal storage disorder. Under the grant agreement, we are reimbursed by the grantor for 75% of qualified research and development costs, up to €2,273 (approximately $2,927 based on the average conversion rate for the grant period to date through June 30, 2015) of such costs over the five-year grant period.

Additionally, we have received grant awards from various agencies of the United States federal government to support our research and development projects. We were awarded five grants from the National Institute of Health (NIH) between the years of 2010 and 2013. As of February 2015, all NIH grants were completed.

Grant revenue is expected to decrease in 2015 and in future periods as we expect to incur less costs under the MeuSIX grant. We are not currently seeking any further grant awards.

Reagent Sales

Reagent sales consist of the sales of licensed reagents to third-parties for use in research and development. We do not consider reagent sales a core aspect of our business model and we do not dedicate significant resources to sales efforts for reagents. Accordingly, future revenue from sales of reagents is uncertain and may fluctuate significantly from period to period.

Expenses

We classify our expenses into three categories: costs of revenue, research and development and general and administrative expenses. Personnel costs including salaries, benefits, bonuses and stock-based compensation expense, comprise a significant component of research and development and general and administrative expenses. We allocate expenses associated with our facilities, information technology costs, depreciation and other overhead costs between research and development and general and administrative categories based on employee headcount and the nature of work performed by each employee.

Costs of Revenue

Costs of revenue primarily consist of our expenses related to the generation of revenue from our intellectual property licensing arrangements and sales of reagents. These expenses fall into the following categories: sublicense fees that are included in licensing costs to related parties, and royalties and production costs that are included in costs of reagent sales. Future costs of revenue are uncertain due to the nature of our license agreements and reagent sales, and significant fluctuations in costs of revenue may occur from period to period.

Research and Development Expense

Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits and any stock-based compensation, for our scientific personnel performing research and development activities;
- costs related to executing preclinical studies and clinical trials;
- costs related to acquiring, developing and manufacturing materials for preclinical studies and clinical trials;
- fees paid to consultants and other third-parties who support our internal product candidate development;
- other costs in seeking regulatory approval of our internal product candidates; and
- allocated facility-related costs and overhead.

Up-front fees incurred in obtaining technology licenses for research and development activities are expensed as incurred if the technology licensed has no alternative future use.

We typically utilize our employee, consultant and infrastructure resources across our development programs. We do not allocate personnel costs, other internal costs or external consultant costs to specific product candidates or development programs.

In 2013 and 2014, research and development expense primarily consisted of expenses incurred under our grant programs, as well as externally sourced research and development services and fees incurred under our services agreement with FoxKiser LLP (FoxKiser), a related party. Under the FoxKiser services agreement, we paid a fixed monthly fee plus an additional support fee, as determined by FoxKiser on a monthly basis, as consideration for all personnel and overhead costs including office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance, and other services provided to us by FoxKiser. We allocated a portion of the service and support fees under the agreement with FoxKiser to research and development. The services agreement with FoxKiser was terminated on January 31, 2015 and all further costs associated with the nature of the services previously received under the agreement are now paid directly by us rather than through the services agreement.

Our internal product candidate development efforts focus on a specific set of metabolic, neurodegenerative and retinal diseases. Our internal product development candidates are RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), RGX-111 for the treatment of Mucopolysaccharidosis Type I (MPS I), RGX-121 for the treatment of Mucopolysaccharidosis Type II (MPS II), RGX-314 for the treatment of wet age-related macular degeneration (wet AMD) and RGX-321 for the treatment of X-linked retinitis pigmentosa. Prior to 2015, we incurred minimal expenditures on these projects. In 2015, we received gross proceeds of $30,000 from the sale and issuance of our Series C convertible preferred stock (Series C Preferred Stock) in January 2015 and $70,500 from the sale and issuance of Series D convertible preferred stock (Series D Preferred Stock) in May 2015. As a result of the capital we raised in 2015, and increased planned expenditures on our internal product development programs, we expect research and development expenses to increase significantly beginning in 2015. We plan to increase our research and development expense for the foreseeable future as we continue our effort to develop and to further advance the development of our gene therapy candidates, subject to the availability of additional funding.

During the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015, we incurred the following external research and development expenses:

- $3,501, $2,677, $988 and $3,481, respectively, for external, preclinical research and development as well as grant activities related to our Lead Product Candidates, and the advancement of our technology and other potential product candidates;

- $132, $344, $0 and $747, respectively, for the development of general manufacturing processes, which we intend to use in the manufacturing of materials for clinical trials for RGX-111, RGX-121, RGX-314 and RGX-321; and
- $14, $320, $174 and $1,252, respectively, for manufacturing of materials to be used in clinical trials for RGX-111, RGX-121 and RGX-314.

The remainder of research and development expenses for the years ended December 31, 2013 and 2014, and for the six months ended June 30, 2014 and 2015 were not allocated to our programs and include personnel costs and overhead, and other unallocated research and development costs including consultants and other externally sourced research and development services.

General and Administrative Expense

General and administrative expense consists primarily of salaries and personnel-related costs, including employee benefits and any stock-based compensation, for employees performing functions other than research and development. This includes personnel in executive, finance and administrative support functions. Other general and administrative expenses include facility-related costs not otherwise allocated to research and development expense, professional fees for accounting and legal services, expenses associated with obtaining and maintaining patents and costs of our information systems.

In 2013 and 2014, general and administrative expense included service fees incurred under our services agreement with FoxKiser. Under the FoxKiser services agreement, we paid a fixed monthly fee plus an additional support fee, as determined by FoxKiser on a monthly basis, as consideration for all personnel and overhead costs including office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance, and other services provided to us by FoxKiser. We allocated a portion of the service and support fees under the agreement with FoxKiser to general and administrative expenses. The services agreement with FoxKiser was terminated on January 31, 2015 and all further costs associated with the nature of the services previously received under the agreement are now paid directly by us.

We expect that our general and administrative expense will increase as we begin to operate as a publicly-traded company and continue to develop and potentially commercialize our internal product candidates. We believe that these increases likely will include increased costs for director and officer liability insurance, costs related to the hiring of additional personnel and increased fees for outside consultants, lawyers and accountants. We also expect to incur increased costs to expand our accounting and finance team with knowledgeable personnel to comply with reporting requirements applicable to public companies and maintain adequate internal control over financial reporting.

Other Income (Expense)

Other income (expense) primarily includes interest expense incurred on our then-outstanding borrowings from FoxKiser.

Amounts outstanding under the FoxKiser services agreement in excess of 30 days from their due date accrued interest at one and half percent per month, compounding monthly. At December 31, 2013 and 2014, amounts due by us to FoxKiser under the services agreement were $655 and $1,423, respectively. The FoxKiser services agreement was terminated on January 31, 2015. Interest expense incurred under this agreement for the years ended December 31, 2013 and 2014 was $611 and $190, respectively.

On July 31, 2014, we received $1,800 in exchange for a promissory note issued to FoxKiser. On September 15, 2014, we received $600 in exchange for a second promissory note issued to FoxKiser. Both promissory notes accrued interest at the Short-Term Applicable Federal Rate (0.34% at December 31, 2014),

compounding annually, and were payable on demand by FoxKiser at the earlier of December 31, 2014 or the next issuance of preferred equity securities by us. We determined that the promissory notes with FoxKiser bear interest at below-market rates. Accordingly, we imputed interest on the promissory notes and recorded an aggregate discount of $128 on the promissory notes, which was amortized using the effective interest method through December 31, 2014, at which date the notes became payable upon demand by FoxKiser. Amortization of the discount is recorded as interest expense in the statements of operations. Interest expense, including imputed interest, incurred under the promissory notes for the year ended December 31, 2014 was $131.

On January 13, 2015, FoxKiser exercised its share settlement options and converted the aggregate principal and interest due under both the promissory notes of $2,403, as well as $1,389 outstanding under the services agreement, into 585,578 shares of Series C Preferred Stock. We expect other income (expense) to decrease significantly beginning in 2015 as a result of the settlement of these debt instruments, termination of the FoxKiser services agreement, and no further debt outstanding as of June 30, 2015.

Income Taxes

To date, we have not been required to pay U.S. federal or state income taxes because we have not generated taxable income.

Critical Accounting Policies and Significant Judgments and Estimates

This Management's Discussion and Analysis of our Financial Condition and Results of Operations is based on our financial statements, which we have prepared in accordance with accounting principles generally accepted in the United States. The preparation of our financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of our financial statements, as well as the reported revenues and expenses during the reported periods. We evaluate these estimates and judgments on an ongoing basis. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing elsewhere in this prospectus, we believe that the following accounting policies are critical to the process of making significant judgments and estimates in the preparation of our financial statements and understanding and evaluating our reported financial results.

Revenue

We generate revenue primarily through license agreements with our NAV Technology Licensees for research, development, and commercialization of product candidates using our proprietary technology. Additionally, we have generated revenue from grant programs and sales of licensed reagents to customers for use in research and development.

We recognize revenue when all of the following criteria are met:

- persuasive evidence of an arrangement exists;
- delivery has occurred or services have been rendered;
- our price to the buyer is fixed or determinable; and
- collectability is reasonably assured.

We defer amounts we receive prior to satisfying the revenue recognition criteria until such time as the revenue recognition criteria are met.

License Revenue and License Revenue from Related Party

The terms of our license agreements require delivery of an intellectual property license for use of our intellectual property in either research only, or in research and commercial development of product candidates for various diseases. We have determined that none of our license agreements contain multiple deliverables from us. We recognize nonrefundable up-front license fees when we deliver the license provided there are no undelivered elements in the arrangement and we have met all of the necessary criteria for revenue recognition. When we determine an option to exercise a commercial license is substantive, we recognize the option fee as revenue upon exercise and delivery of the underlying commercial license, provided there are no undelivered elements in the arrangement and we have met all of the necessary criteria for revenue recognition. Annual maintenance fees do not represent a separate deliverable other than the delivery of the license. We recognize annual maintenance fees as revenue under our license agreements when the price is fixed or determinable and collectability is reasonably assured, provided that we have satisfied all other revenue recognition criteria, which is typically upon each anniversary date of the underlying license agreement.

Sublicense fees are payable to us upon the receipt of certain fees by the licensee from any sublicensees. We recognize sublicense fees as revenue when the price is fixed and determinable and collectability is reasonably assured, provided that we have satisfied all other revenue recognition criteria.

We recognize milestone payments as revenue upon achievement of the milestone by the licensee, provided that we have satisfied all other revenue recognition criteria. At the inception of an arrangement that includes milestone payments, we evaluate whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. We evaluate factors such as the scientific, clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. We have evaluated each, and concluded that all of the clinical, regulatory and commercial milestones pursuant to our license agreements are substantive. Milestone payments are recognized as revenue upon achievement of the milestone by the licensee, provided that all other revenue criteria are satisfied.

We will recognize royalty revenue in the period of sale of the related product(s) based on the underlying contract terms, provided that we can reliably measure the reported sales, we have no remaining performance obligations, and we have satisfied all other revenue recognition criteria. See “—License Revenue and License Revenue from a Related Party.”

Grant Revenue

We generate grant revenue through research and development grant programs offered by the United States federal government and the European Union. We recognize revenue related to government grants in the period during which the related costs are incurred and the related services are rendered, provided that we have met the applicable performance obligations under the grants. If we are the principal and the primary obligor under the arrangements, we record the funds we receive under the grants as revenue. If we are not the principal or primary obligor, we record the grant proceeds as a reduction to research and development expense.

Our grants contain refund provisions in the case of non-compliance with the provisions of the grant, which include, but are not limited to, the eligibility of costs, calculation of personnel rates, selection of subcontractors and other provisions included in the underlying grant agreements. We review those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is recognized as revenue. If the probability of repayment is determined to be more than remote, we record the amount of potential repayment of the grant as a liability, until such time that the grant requirements have been satisfied. Funds received in advance of the performance of the services are recorded as deferred revenue.

Reagent Sales

Our reagent sales consist of the sales of licensed reagents to third-parties for use in research and development. We recognize revenue from reagent sales upon delivery to customers, provided that we have satisfied all other revenue recognition criteria.

Accrued Research and Development Expenses

We estimate our accrued research and development expenses as of each balance sheet date. This process involves reviewing contracts and purchase orders with service providers, identifying services that have been performed on our behalf, and estimating the level of service performed, expected remaining period of performance, and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. Expenses that are paid in advance of performance are deferred as a prepaid expense and expensed as the services are provided.

Examples of estimated accrued research and development expenses include fees paid to:

- contract research organizations (CROs) in connection with preclinical development and clinical studies;
- vendors related to process development and manufacturing of materials for use in preclinical development and clinical studies;
- service providers for professional service fees such as consulting and other research and development related services.

Our understanding of the status and timing of services performed relative to the actual status and timing may vary and may result in us reporting changes in estimates in any particular period. To date, there have been no material differences from our estimates to the amount actually incurred.

Stock-Based Compensation

Our 2014 Stock Plan (the Plan) provides for issuance of stock options, restricted stock awards, and unrestricted stock awards to our employees, members of the board of directors, and consultants. We have not granted restricted or unrestricted stock awards under the Plan since its inception, and we did not grant any stock option awards prior to 2014.

Our stock-based awards are subject to either service or performance-based vesting conditions. We record compensation expense for awards to employees and directors with service-based vesting conditions based on the estimated grant date fair value of the awards. We recognize compensation expense for employee awards on a straight-line basis over the requisite service period, which is generally the vesting term. We record compensation expense for awards to non-employees with service-based vesting conditions based on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, which is generally the vesting term, on a straight-line basis. We recognize compensation expense for non-employee awards with performance-based vesting conditions based on the then current fair value at each financial reporting date prior to the measurement date over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. We recognize compensation expense for employee awards with performance-based vesting conditions based on the estimated grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

We have reported stock-based compensation expense in our statements of operations as follows:

	Year Ended December 31, 2014	Six Months Ended June 30, 2015
Research and development	$ 60	$ 312
General and administrative	259	399
	$ 319	$ 711

We did not grant any stock options prior to September 24, 2014. Accordingly, no stock-based compensation was recorded for the year ended December 31, 2013 and the six months ended June 30, 2014.

Determination of the Fair Value of Stock-Based Compensation Grants

We calculate the fair value of stock options using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the use of subjective assumptions, including the expected volatility of our common stock, the assumed dividend yield, the expected term of our stock options, the risk-free interest rate for a period that approximates the expected term of our stock options, and the fair value of the underlying common stock on the date of grant. In applying these assumptions, we considered the following factors:

- We do not have sufficient history to estimate the volatility of our common stock. We calculate expected volatility based on reported data for selected similar publicly traded companies for which the historical information is available. For the purpose of identifying peer companies, we consider characteristics such as enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected term of the stock-based awards. We focus our peer group company selection on companies that operate within the biotechnology industry, and specifically on companies that use gene therapy, or similar technologies, for treating diseases and/or are focused on treating diseases in our development pipeline, or our licensees' pipelines. We ensure that the companies selected have sufficient trading history to provide meaningful data to estimate the expected volatility of our common stock over the expected term of stock options we have granted. We carefully consider the size of the selected peer group companies relative to us, and its potential impact on our expected volatility. We have performed analyses of our expected volatility under various scenarios in which we have altered our peer group selection, or applied weighting, such that the computation focuses more closely on companies closer to our estimated size over the expected term of the stock options we have awarded. As a result of these analyses, we have determined that potential changes to the peer group company used, or other changes in computations to account for the difference in our size relative to our peers, would have an immaterial effect on our expected volatility and stock-based compensation expense. We plan to continue to use the guideline peer group volatility information until the historical volatility of our common stock is sufficient to measure expected volatility for future option grants;
- The assumed dividend yield of zero is based on our expectation of not paying dividends for the foreseeable future;
- We determine the average expected life of "plain vanilla" stock options based on the simplified method in accordance with Securities and Exchange Commission (SEC) Staff Accounting Bulletin Nos. 107 and 110, as our common stock to date has not been publicly traded. We expect to use the simplified method until we have sufficient historical exercise data to provide a reasonable basis upon which to estimate expected term;
- We determine the risk-free interest rate by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant; and
- We estimate forfeitures based on our historical analysis of actual stock option forfeitures. To date, we have had minimal forfeitures, accordingly, we have assumed no forfeiture rate.

The following summarizes the assumptions we used to estimate the fair value of stock options that we granted to employees and non-employees for the period indicated:

	Year Ended December 31, 2014	Six Months Ended June 30, 2015
Employees		
Expected volatility	64%	64%
Expected term (in years)	6.0	6.1
Risk-free interest rate	2.0%	1.7%
Expected dividend yield	0.0%	0.0%
Non-employees		
Expected volatility	65%	67%
Expected term (in years)	9.9	9.8
Risk-free interest rate	2.4%	1.7%
Expected dividend yield	0.0%	0.0%

Based upon an assumed initial public offering price of $ per share, the midpoint of the price range set forth on the cover of this prospectus, the aggregate intrinsic value of outstanding options to purchase shares of our common stock as of June 30, 2015 was $ million, of which $ million related to vested options and $ million to unvested options.

Determination of Exercise Price of Stock Options and the Fair Value of Common Stock on Grant Dates

The following table summarizes by grant date the number of shares of our common stock subject to stock options granted during 2014 and 2015 as well as the associated per-share exercise price and the estimated fair value per share of our common stock on the grant date:

Grant Date	Number of Shares Underlying Options Granted	Exercise Price per Share	Estimated Fair Value per Share of Common Stock	Estimated Fair Value per Share of Options(a) (b)
September 24, 2014	1,884,500	$ 0.85	$ 0.85	$ 0.52
November 4, 2014	247,900	$ 0.85	$ 0.85	$ 0.49
May 15, 2015	1,063,900	$ 3.76	$ 3.76	$ 2.27

(a) The Estimated Fair Value per Share of Options reflects the weighted-average fair value of options granted on each grant date, determined using the Black-Scholes option-pricing model.

(b) For purposes of recording stock-based compensation for grants of options to non-employees, we measure the fair value of the award on the service completion date (vesting date). At the end of each reporting period prior to completion of the services, we remeasure the value of any unvested portion of the option based on the then-current fair value of the option and adjust the expense accordingly. The weighted-average fair value amounts presented in this column for grants to employees, directors and non-employees reflect only the grant-date fair value of options granted to non-employees and not any subsequently remeasured fair value of those options.

In setting the exercise price of the stock options at each grant date, our board of directors or its compensation committee uses the estimated fair value of the common stock on the date of grant.

In connection with the preparation of the financial statements necessary for the filing of the registration statement of which this prospectus forms a part, we undertook valuations of the fair value of our common stock as of July 31, 2014, April 30, 2015 and June 15, 2015 for financial reporting purposes. We used the estimated fair value per share of our common stock as determined by this valuation to measure the stock-based compensation expense for options granted for the dates shown in the table above.

There are significant assumptions and estimates required in determining the fair value of our common stock. Following the closing of this offering and the commencement of public trading of our common stock, the fair value per share of our common stock for purposes of determining stock-based compensation will be the closing price of our common stock as reported on the applicable grant date.

Common Stock Valuation Methodology

To estimate the fair value of our common stock, given the absence of a public trading market for our common stock, valuation estimates are prepared by management, and provided to our board of directors, in accordance with the framework of the *American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation* (the AICPA Practice Guide), as well as independent third-party valuations. Our contemporaneous valuations of our common stock as of July 31, 2014, April 30, 2015 and June 15, 2015 were based on a number of objective and subjective factors, including external market conditions affecting our industry sector and the prices at which we sold shares of preferred stock, the superior rights and preferences of securities senior to our common stock, and the likelihood of achieving a liquidity event such as an initial public offering (IPO).

July 31, 2014 Valuation

For our valuation as of July 31, 2014, we determined the aggregate equity value of our business using a combination of the market multiple approach (20% weighting) and back-solve method of the option-pricing method (OM) (80% weighting).

The market multiple approach estimates the fair value of a company by applying market multiples of comparable publicly-traded companies and publicly disclosed financial data to arrive at estimated fair value. We applied a market multiple of revenue of comparable publicly-traded companies to our estimated revenue for the year ended December 31, 2014 to arrive at an estimated equity value. We gave consideration to differences between us and the selected guideline public companies in terms of size, anticipated profitability, market size and other critical characteristics that generally reflect an investor's assessment of the business and financial risks inherent in our industry. In particular we gave consideration to the fact that we had no clinical-stage therapy candidates currently in our development pipeline compared to comparable publicly-traded companies that have advanced pipelines and approved drugs.

The OM back-solve method derives the implied equity value for one type of equity security from a contemporaneous transaction involving another type of equity security. We applied the OM back-solve method to solve for the equity value and corresponding value of common stock based on the price per unit of our Series B Preferred Units issued in October 2013 by ReGenX Biosciences, LLC (our predecessor entity). The issuance of Series B Preferred Units was led by an unrelated investor that had not previously invested in us. We believe the per unit issuance price of the Series B Preferred Units provides an indication of the fair value of our equity as of July 31, 2014.

The OM treats common stock and convertible preferred stock as call options on an equity value, with exercise prices based on the liquidation preference of the convertible preferred stock. Therefore, the common stock has value only if the funds available for distribution to the stockholders exceed the value of the liquidation preference at the time of a liquidity event such as a merger, sale or IPO, assuming the enterprise has funds available to make a liquidation preference meaningful and collectible by the stockholders. The common stock is modeled to be a call option with a claim on the enterprise at an exercise price equal to the remaining value immediately after the convertible preferred stock is liquidated. The OM uses the Black-Scholes option-pricing model to price the call options. The OM is appropriate to use when the range of possible future outcomes is so difficult to predict that forecasts would be highly speculative.

We estimated the time to liquidity as 3.0 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The estimated time to liquidity considered that at the valuation date we had only raised $10,891 in equity capital since inception and we had no clinical phase drug candidates or collaborative partnerships with more capitalized pharmaceutical companies. The risk-free rate was estimated as the interpolated 3.0 year yield on government bonds.

We estimated volatility to be 65% at the valuation date given our early stage of development. To arrive at this number, historical volatilities of comparable publicly-traded companies were analyzed, most of which are significantly more developed than we are.

We applied a discount for lack of marketability (DLOM) to the value indicated for our common stock. A discount is appropriate because our common stock is unregistered, and the holder of a minority interest in the common stock may not influence the timing of a liquidity event for us. Our estimate of the appropriate discount for lack of marketability took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount of 41%, which we selected as an appropriate DLOM.

The following table summarizes the significant assumptions used to determine the fair value of our common stock of $0.85 per share as of July 31, 2014:

Equity value	$23,300
Years to liquidity event	3.0
Annual volatility	65%
Risk-free interest rate	1.02%
Discount for lack of marketability (DLOM)	41%

April 30, 2015 Valuation

For our valuation as of April 30, 2015, we used a hybrid of the probability-weighted expected return method (PWERM) (15% weighting) and the OM (85% weighting), which we refer to as the hybrid method.

Under the PWERM, share value is derived from the probability-weighted present value of expected future investment returns, considering possible outcomes available to us, as well as the economic and control rights of each share class. The PWERM in our April 30, 2015 valuation assumes an IPO date five months from the valuation date based on our board of directors' assessment of our prospects, our investors' motivations and market conditions. The PWERM considers two possible outcomes: (i) a future equity value upon an IPO at the high end of an estimated range and (ii) a future equity value upon an IPO at the lower end of an estimated range. In order to estimate the range of potential future equity values upon an IPO for the PWERM, we considered the pre-money enterprise values at the IPO date of comparable companies that had undergone IPOs in recent periods prior to April 30, 2015. We placed a 35% weighting on the higher end of the range of expected future equity values, and a 65% weighting on the lower end of the range, based on the stage of development of our internal drug candidates versus the comparable publicly-held companies which generally had further developed drug pipelines at the date of their IPOs. The future equity value at the expected IPO date under each scenario was allocated to each series of preferred stock and the common stock assuming conversion of all preferred series to common. We then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. We assumed a risk-adjusted rate of 20% for the common shares. We selected these risk-adjusted rates based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid.

Under the PWERM, we applied a DLOM to the value indicated for our common stock. Our estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount between 11% and 20%, which we used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

Under the OM, we applied the OM back-solve method to solve for the equity value and corresponding value of common stock based on the price per share of our Series D Preferred Stock issued in May 2015. Given the proximity to the Series D Preferred Stock financing, and the fact that the Series D Preferred Stock issuance included and was led by unrelated investors, we believe the per share issuance price of the Series D Preferred Stock provides an indication of the fair value of our equity as of April 30, 2015. The values indicated for the preferred and common shares by the IPO scenario and the OM scenario were probability weighted to calculate the weighted value as of the April 30, 2015 valuation date.

Under the OM, we estimated the time to liquidity as 2.5 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The anticipated timing of a liquidity event was management's estimate in the event our planned IPO does not occur. The risk-free rate was estimated as the interpolated 2.5 year yield on government bonds.

Under the OM, we estimated volatility to be 82% at the valuation date given our early stage of development. To arrive at this number, historical volatilities of comparable publicly-traded companies were analyzed, most of which are significantly more developed than we are.

Under the OM, we applied a DLOM to the value indicated for our common stock. Our estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount between 29% and 57%, which we used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

For the April 30, 2015 valuation, we estimated the fair value of our common stock by assigning an 85% weighting to the estimated fair value using the OM back-solve method and a 15% weighting to the PWERM method. We believe that the 85% weighting on the OM back-solve method is appropriate due to the proximity of the sale and issuance of our Series D Preferred Stock in May 2015. The 15% weighting for the IPO scenario was deemed appropriate because at the time of the valuation, we believed that there was the possibility of following a successful Series D Preferred Stock financing with an IPO.

June 15, 2015 Valuation

For our valuation as of June 15, 2015, we used a hybrid of the PWERM (40% weighting), and the OM (60% weighting).

The PWERM in our June 15, 2015 valuation assumes an IPO date 3.5 months from the valuation date based on our board of directors' assessment of our prospects, our investors' motivations and market conditions. The PWERM considers two possible outcomes: (i) a future equity value upon an IPO at the high end of an estimated range and (ii) a future equity value upon an IPO at the lower end of an estimated range. In order to estimate the range of potential future equity values upon an IPO for the PWERM, we considered the pre-money enterprise values at the IPO date of comparable companies that had undergone IPOs in recent periods prior to June 15, 2015. We placed a 35% weighting on the higher end of the range of expected future equity values, and a 65% weighting on the lower end of the range, based on the stage of development of our internal drug candidates versus the comparable publicly-held companies which generally had further developed drug pipelines at the date of their IPOs. The future equity value at the expected IPO date under each scenario was allocated to each series of preferred stock and the common stock assuming conversion of all preferred series to common. We then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. We assumed a risk-adjusted rate of 20% for the common shares. We selected these risk-adjusted rates based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid.

Under the PWERM, we applied a DLOM to the value indicated for our common stock. Our estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A

put option model indicated a discount between 9% and 15%, which we used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

Under the OM, we applied the OM back-solve method to solve for the equity value and corresponding value of common stock based on the price per share of our Series D Preferred Stock issued in May 2015. Given the proximity to the Series D Preferred Stock financing, and the fact that the Series D Preferred Stock issuance included and was led by unrelated investors, we believe the per share issuance price of the Series D Preferred Stock provides an indication of the fair value of our equity as of June 15, 2015. The values indicated for the preferred and common shares by the IPO scenario and the OM scenario were probability weighted to calculate the weighted value as of the June 15, 2015 valuation date.

Under the OM, we estimated the time to liquidity as 2.5 years based on then-current plans and estimates of our board of directors and management regarding a liquidity event. The anticipated timing of a liquidity event was management's estimate in the event our planned IPO does not occur. The risk-free rate was estimated as the interpolated 2.5 year yield on government bonds.

Under the OM, we estimated volatility to be 81% at the valuation date given our early stage of development. To arrive at this number, historical volatilities of comparable publicly-traded companies were analyzed, most of which are significantly more developed than we are.

Under the OM, we applied a DLOM to the value indicated for our common stock. Our estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount between 29% and 57%, which we used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

For the June 15, 2015 valuation, we estimated the fair value of our common stock by assigning a 60% weighting to the estimated fair value using the OM back-solve method and a 40% weighting to the PWERM method. We believe that the 60% weighting on the OM back-solve method is appropriate due to the proximity of the sale and issuance of our Series D Preferred Stock in May 2015. The 40% weighting for the IPO scenario was deemed appropriate because at the time of the valuation, we believed that there was a higher probability of following our Series D financing with a successful IPO than there was at our previous valuation date of April 30, 2015.

The following table summarizes the significant assumptions used to determine the fair value of our common stock of $3.76 and $6.90 per share as of April 30, 2015 and June 15, 2015, respectively, using the hybrid method:

	April 30, 2015		June 15, 2015	
	OM	PWERM	OM	PWERM
Weighting	85%	15%	60%	40%
Equity value	$130,700	$338,100	$130,000	$346,000
Years to liquidity event	2.5	0.4	2.5	0.3
Annual volatility	82%	N/A	81%	N/A
Risk-free interest rate	0.75%	N/A	0.91%	N/A
Weighted average cost of capital	N/A	20%	N/A	20%
Discount for lack of marketability	35%	15%	35%	10%
Estimated per share fair value of common stock	$ 2.08	$ 13.25	$ 2.03	$ 14.21

Cost Method Investments

Cost method investments consist of holdings in certain corporations and are stated at cost. We account for our investments in other entities using the cost method if our ownership interest is below 20% and we do not have significant influence over the operations of the entities.

Declines in the fair value of cost method investments below their carrying value that are deemed to be other-than-temporary are reflected in earnings as realized losses. In estimating other-than temporary impairment losses, management considers, among other things, (i) the length of time and the extent to which the fair value has been less than cost, (ii) the financial condition and near term prospects of the issuer, and (iii) our intent and ability to retain our investments in the issuer for a period of time sufficient to allow for the anticipated recovery in fair value.

We have not identified any events or changes in circumstances that would have an adverse effect on the carrying value of our cost method investments as of December 31, 2013 and 2014, and June 30, 2015.

Variable Interest Entity Analysis

Upon the initial investment in an entity, the inception of a commercial license agreement, or upon any reconsideration event, we must determine if the entity meets the definition of a variable interest entity (VIE) and, if so, if we are the primary beneficiary and required to consolidate the entity.

We consider an entity to be a VIE if (i) its investors do not have sufficient equity at risk for the legal entity to finance its activities without additional subordinated financial support, or (ii) as a group, the holders of the equity investment at risk do not have both the power to direct the activities of the legal entity that most significantly impact the entity's economic performance, and the obligation to absorb the expected losses or the right to receive expected residual returns of the legal entity.

We are considered the primary beneficiary of a VIE if we have both the power to direct the activities that most significantly affect the VIE's economic performance and the obligation to absorb the losses of, or right to receive benefits from, the VIE that could be potentially significant to the VIE. If we, or any of our related parties that have a variable interest in the VIE, individually lack the necessary power and benefits criteria, but the related party group as a whole has the necessary power and benefits, we determine which of the related party group members is most closely associated with the VIE and consider that party to be the primary beneficiary.

As a result of our analyses, we have concluded that we are not the primary beneficiary of any VIEs and, therefore, we have not consolidated any VIEs.

Utilization of Net Operating Loss Carryforward

As of December 31, 2014, we had federal net operating loss (NOL) carryforwards of approximately $2,979 which may be available to offset future income tax liabilities and expire at various dates through 2034. We also have U.S. state NOL carryforwards of approximately $13,406, which may be available to offset future income tax liabilities and which expire at various dates through 2034.

Under the provisions of the Internal Revenue Code, the NOL carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL carryforwards may become subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50%, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on our value immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. We have completed several financings since our inception, which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

If we experience such an ownership change in connection with this offering, previous offerings, or future offerings, the tax benefits related to the NOL carryforwards may be further limited or lost.

We account for income taxes in accordance with Financial Accounting Standards Board (FASB) ASC Topic 740, *Income Taxes*, which provides for deferred taxes using an asset and liability approach. We recognize deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

We have evaluated the positive and negative evidence bearing upon the realizability of our deferred tax assets, including our NOLs. Based on our history of operating losses, we believe that it is more likely than not that the benefit of our deferred tax assets will not be realized. Accordingly, we have provided a full valuation allowance for deferred tax assets as of December 31, 2013 and 2014 and June 30, 2015.

Convertible Preferred Stock and Preferred Units

We evaluate convertible preferred stock and preferred units upon issuance in order to determine classification as to permanent or temporary equity and whether or not the instruments contain an embedded derivative that requires bifurcation. This analysis followed the whole instrument approach which compares an individual feature against the entire convertible preferred stock or preferred unit instrument which includes that feature. This analysis was based on a consideration of the economic characteristics and risk of each series of convertible preferred stock and preferred units.

We evaluated all of the stated and implied substantive terms and features, including: (i) whether the convertible preferred stock and preferred units included redemption features, (ii) how and when any redemption features could be exercised, (iii) whether the holders of convertible preferred stock and preferred units were entitled to dividends and how those dividends were calculated, (iv) the voting rights of the convertible preferred stock and preferred units and (v) the existence and nature of any conversion rights.

As a result of this analysis, we concluded that the convertible preferred stock and preferred units represent a debt host and, therefore, the redemption feature of each series of convertible preferred stock and preferred units is considered to be clearly and closely related to the associated debt host instrument and is not considered an embedded derivative that requires bifurcation.

We also concluded that the conversion rights under the convertible preferred stock are not clearly and closely related to the debt host instruments, however the conversion features do not meet the net settlement criteria of a derivative and, therefore, are not considered embedded derivatives that require bifurcation.

As a result of this analysis, we concluded that it was appropriate to classify the outstanding shares of convertible preferred stock and preferred units outside of permanent equity and within temporary equity, due to their associated redemption features and liquidation preferences which are considered to be outside of our control. At each reporting date, each series of outstanding convertible preferred stock and preferred units is accreted and stated at the amounts in which each series is currently redeemable, which is also equal to the aggregate liquidation preference at that date.

Extinguishment of Preferred Stock

In connection with the issuance of the Series C Preferred Stock in January 2015, the rights, preferences, and privileges of the Series A convertible preferred stock (Series A Preferred Stock) and the Series B convertible preferred stock (Series B Preferred Stock) then outstanding were modified. More specifically, holders of Series C Preferred Stock received preference over Series A Preferred Stock, Series B Preferred Stock and Common Stock in regards to dividends and liquidation. The dividend rights changed from cumulative dividend rights to noncumulative dividend rights for all series of convertible preferred stock, and all accrued but unpaid cumulative

dividends on the Series A Preferred Stock and Series B Preferred Stock as of January 13, 2015 were forfeited. As a result of this modification, the redemption values and liquidation preferences of Series A Preferred Stock and Series B Preferred Stock, which were previously equal to original issue price plus accrued but unpaid cumulative dividends, was reduced to original issue price plus non-cumulative dividends declared. Additionally, the redemption date of Series A Preferred Stock and Series B Preferred Stock was changed from October 30, 2018 to December 31, 2019.

We accounted for the amendment to the rights, preferences, and privileges of the Series A Preferred Stock and Series B Preferred Stock as an extinguishment of the old convertible preferred stock and issuance of new convertible preferred stock due to the significance of the modifications to the substantive contractual terms of the convertible preferred stock and the associated fundamental changes to the nature of the convertible preferred stock. Accordingly, we recorded a loss of $1,317 on the Series A Preferred Stock and a gain of $2,076 on the Series B Preferred Stock within stockholders' deficit equal to the difference between the fair value of the new shares of convertible preferred stock issued and the carrying amount of the old shares of preferred stock extinguished. We allocated the entire net gain on extinguishment of convertible preferred stock of $759 to additional paid-in capital. The net gain on extinguishment is reflected in the calculation of net loss available to common stockholders in accordance with FASB ASC Topic 260, *Earnings per Share*. The fair value of the Series A Preferred Stock and Series B Preferred Stock was determined using the OM back-solve method on the per share price of Series C Preferred Stock to estimate aggregate equity value. We used the OM to allocate equity value to the Series A Preferred Stock and Series B Preferred Stock using Black-Scholes option-pricing model.

A summary of the changes within each class of convertible preferred stock for the six months ended June 30, 2015 are as follows:

	Series A Convertible Preferred Stock	Series B Convertible Preferred Stock	Series C Convertible Preferred Stock	Series D Convertible Preferred Stock
Carrying amount at December 31, 2014	$ 3,963	$ 8,630	$ —	$ —
Accretion of convertible preferred stock prior to issuance of Series C convertible preferred stock	9	22	—	—
Issuance of Series C convertible preferred stock, net of transaction costs	—	—	29,813	—
Loss (gain) on extinguishment of convertible preferred stock	1,317	(2,076)	—	—
Issuance of Series D convertible preferred stock, net of transaction costs	—	—	—	67,998
Accretion (decretion) to redemption value	(2,289)	1,316	187	2,502
Carrying amount at June 30, 2015	$ 3,000	$ 7,892	$ 30,000	$ 70,500

Related Party Transactions

The Trustees of the University of Pennsylvania

In February 2009, we entered into a license agreement, as amended, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents and patent applications owned by the Penn. In consideration for the license, we issued to Penn 24.5% of our then-outstanding membership interest on a fully diluted basis after issuance which is now represented by 213,150 shares of our common stock. Under the agreement, we pay Penn royalties on net sales and sublicense fees. Additionally, we are obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents. In addition to our license agreement, Penn also provides services to us including manufacturing of reagents and preclinical research and development related to our grant programs and internal drug candidates.

GlaxoSmithKline LLC

In March 2009, we entered into a license agreement, as amended, with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents owned by Penn and exclusively licensed to GSK. In consideration for the license, we issued to GSK 19.9% of our then-outstanding membership interest on a fully diluted basis after issuance which is now represented by 1,085,824 shares of our common stock. Under the agreement, we pay GSK royalties on net sales and sublicense fees. We are obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents. Additionally, we are obligated to pay GSK up to $1,650 upon the achievement of various milestones, none of which have been achieved as of June 30, 2015.

Dimension Therapeutics, Inc.

In October 2013, we granted an exclusive, sublicensable, worldwide commercial license to Dimension for preclinical and clinical research and development, and commercialization of drug therapies using our licensed patents for the treatment of hemophilia A and hemophilia B, as well as a one-year option to obtain exclusive licenses for the commercialization of two other diseases to be elected by Dimension in the future. The agreement requires on-going annual maintenance fees payable to us, for each disease indication licensed to Dimension, beginning in October 2014. The agreement also requires Dimension to pay us royalties on net sales, if any, intended to be approximately equal to the amount of royalties that will be due by us to Penn and GSK on such sales. In consideration for the license granted, Dimension issued us, and a number of our members, directors, and executives, an aggregate total of 10,000,000 shares of its common stock, with an estimated fair value of $2,700. We recorded $2,700 as revenue upon delivery of the license. Of the 10,000,000 shares, a total of 10,000 shares were issued to us, with an estimated fair value of $3, which is included in cost method investments on the balance sheets. In consideration for the efforts by the various members, directors and executives which were responsible for executing the license agreement with Dimension, we recorded expenses equal to the estimated fair value of the 9,990,000 shares of common stock of Dimension received by those parties of $2,697, which is included in general and administrative expenses in the statements of operations. In accordance with our revenue recognition policy, we determined that the $2,700 in revenue from the license granted to Dimension should be recognized in full upon the delivery of the license, as we have no further significant performance obligations under the agreement. Additionally, we determined that the $2,697 of general and administrative expenses to related parties should be recognized in full upon the execution of the agreement with Dimension, as those parties have no further performance obligations to us as a result of the transaction.

In addition to our related parties holding common stock in Dimension as a result of the license agreement, three of our board members served on the board of directors of Dimension on the effective date of the license. We evaluated consolidation guidance under ASC 810 and determined that Dimension is considered a variable interest entity. However, we do not consolidate Dimension because we lack the power to direct the activities of the VIE that most significantly impact the VIE’s economic performance. We hold an equity interest in Dimension and also have a license agreement granting Dimension the right to use our licensed intellectual property.

In connection with the license agreement granted to Dimension, we entered into an arrangement with Penn and Dimension in which we helped coordinate and manage research and development activities performed by Penn on behalf of Dimension. Under the arrangement, Dimension reimbursed us for all costs incurred and paid to Penn, and we retain rights to certain intellectual property discovered under the contracted research and development performed by Penn. Due to the uncertainty of any future intellectual property rights that may be discovered by Penn and retained by us, and because such intellectual property would have no future alternative use due to the stage of development of the drug therapies under development, we have not recognized any benefit as consideration paid by Dimension to us as a result of the license agreement. We have evaluated the facts and circumstances of the arrangement with regards to ASC 605-45, *Revenue Recognition-Principal Agent Considerations* and determined that the reimbursements from Dimension should be recorded on a net basis. Accordingly, proceeds received from Dimension under the arrangement were recorded as a reduction of research

and development expense in the statements of operations. As of June 30, 2015 (unaudited), we received the final payments from Dimension under this arrangement and paid all amounts owed to Penn, and the arrangement was ended.

In September 2014, Dimension elected OTC deficiency as its third disease indication under the license agreement, and the license was amended to extend the term of the option to elect the fourth and final disease indication for an additional six months. In consideration for the extension of the option, Dimension paid us an extension fee. In January 2015, Dimension elected glycogen storage disease type Ia as its fourth and final disease indication under the license.

In March 2015, we entered into an option and license agreement granting Dimension options to an exclusive commercial license for four new disease indications to be elected by Dimension in the future. If elected, each option carries an option fee payable to us upon exercise and annual maintenance fees. Additionally, for each option exercised, Dimension is obligated to pay us upon achievement of various substantive milestones, as well as mid to upper-single-digit percentage royalties on net sales of licensed products and mid-single-digit to low-double-digit percentage sublicense fees, if any. Dimension exercised its first two options under the option and license agreement in May 2015 and August 2015.

During the year ended December 31, 2014, we received $200 from Dimension for the purchase of manufacturing materials owned by us which we use to manufacture materials for research and development and future clinical trials. The material is delivered to Dimension upon their request. Since the sale of the material is not a recurring revenue stream or core aspect of our business, we deferred the recognition of the $200 as an advance payment, and recognize a gain on disposal of the material as the material is delivered by us to Dimension.

FoxKiser LLP

During 2013 and 2014, we were party to a services agreement, as amended from time to time, with FoxKiser, one of our stockholders, which was terminated in January 2015. Under the agreement, we paid a fixed monthly fee plus an additional support fee, as determined by FoxKiser on a monthly basis, as consideration for office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance, and other services provided to us. Amounts outstanding to FoxKiser in excess of 30 days from their due date accrued interest at one and half percent per month, compounding monthly. We allocated the service and support fees under the agreement with FoxKiser between research and development and general and administrative expense.

Amounts owed by us to FoxKiser under the services agreement were settled through the issuance of Series B Preferred Units of ReGenX Biosciences, LLC (our predecessor entity) on October 30, 2013 and subsequently Series C Preferred Stock on January 13, 2015. In January 2015, the services agreement was terminated and the remaining amounts due to FoxKiser under the agreement were paid in full in cash.

Emerging Growth Company Status

Under Section 107(b) of the JOBS Act, emerging growth companies can delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Recent Accounting Pronouncements

In February 2015, the FASB issued Accounting Standards Update (ASU) 2015-2, *Consolidation (Topic 810): Amendments to the Consolidation Analysis*, which provides clarification regarding the guidance surrounding consolidation of certain legal entities. This guidance is effective for annual and interim periods beginning after December 15, 2015. We are evaluating the application of this ASU, but we have not yet determined the potential effects it may have on our financial statements.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, requiring management to evaluate whether events or conditions could impact our ability to continue as a going concern and to provide disclosures if necessary. Management will be required to perform the evaluation within one year after the date that the financial statements are issued. Disclosures will be required if conditions give rise to substantial doubt and the type of disclosure will be determined based on whether management's plans will be able to alleviate the substantial doubt. The ASU will be effective for the first annual period ending after December 15, 2016, and for annual periods and interim periods thereafter with early application permitted. We are evaluating the application of this ASU, but we have not yet determined the potential effects it may have on our financial statements.

In June 2014, the FASB issued ASU No. 2014-12, *Compensation—Stock Compensation (Topic 718): Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could be Achieved after the Requisite Service Period*, which requires us to assess share-based awards with performance targets that could be achieved after the requisite service period for potential treatment as a performance condition. Compensation expense is to be recognized when the performance target is deemed probable and should represent the compensation expense attributable to the periods for which service has already been rendered. If the performance target is reached prior to achievement of the service period, the remaining unrecognized compensation cost should be recognized over the remaining service period. The ASU is effective for annual and interim periods beginning after December 15, 2015 with early adoption permitted. We have evaluated the application of this ASU, and determined that it does not have a material effect on our financial statements.

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers (Topic 606)*, which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition. This ASU is based on the principle that revenue is recognized to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. The ASU was originally effective January 1, 2017, however, on April 1, 2015, the FASB voted to propose a deferral of the effective date by one year until January 1, 2018, but will permit entities to adopt the standard as of the original effective date. We are evaluating the application of this ASU, but we have not yet determined the potential effects it may have on our financial statements.

Results of Operations

(in thousands)	Years Ended December 31, 2013	2014	Change	Six Months Ended June 30, 2014	2015	Change
Revenue						
License revenue	$ 1,055	$ 4,355	$ 3,300	$3,705	$ 570	$ (3,135)
License revenue from related party	2,700	220	(2,480)	—	1,000	1,000
Reagent sales	368	326	(42)	291	148	(143)
Grant revenue	1,964	1,219	(745)	490	289	(201)
Total revenues	6,087	6,120	33	4,486	2,007	(2,479)
Expenses						
Costs of revenue						
Licensing costs to related parties	151	885	734	741	314	(427)
Costs of reagent sales	173	122	(51)	102	49	(53)
Research and development	5,051	4,961	(90)	1,787	6,803	5,016
General and administrative	5,474	3,851	(1,623)	1,660	5,113	3,453
Foreign currency transaction losses (gains)	14	30	16	(14)	38	52
Other operating income	—	(47)	(47)	(24)	(21)	3
Total expenses	10,863	9,802	(1,061)	4,252	12,296	8,044
Income (loss) from operations	(4,776)	(3,682)	1,094	234	(10,289)	(10,523)
Other Income (Expense)						
Investment income	—	—	—	—	8	8
Interest expense	(611)	(321)	290	(111)	(20)	91
Total other income (expense)	(611)	(321)	290	(111)	(12)	99
Net income (loss)	$ (5,387)	$ (4,003)	$ 1,384	$ 123	$ (10,301)	$ (10,424)

Comparison of the Six Months Ended June 30, 2014 and 2015

License Revenue and License Revenue from Related Party. License revenue and license revenue from a related party decreased by $2,135, from $3,705 for the six months ended June 30, 2014 to $1,570 for the six months ended June 30, 2015. This decrease is primarily attributable to up-front fees received for five new licenses granted during the six months ended June 30, 2014 and only one new license during the six months ended June 30, 2015. The decrease in up-front license fees was partially offset due to an increase of annual recurring maintenance fees of $115 related to licenses granted during the six months ended June 30, 2014. License revenue included $1,000 for the six months ended June 30, 2015 recognized from a license granted to Dimension. In accordance with our revenue recognition policy, we recognize up-front license fees as revenue immediately because we have no further performance obligations under the license agreements, and all other necessary revenue recognition criteria have been met. Additionally, we recognize annual maintenance fees as revenue when the price is fixed or determinable, obligations are satisfied and collectability is deemed reasonably assured.

Reagent Sales. Reagent sales decreased by $143, from $291 for the six months ended June 30, 2014 to $148 for the six months ended June 30, 2015. This decrease is primarily attributable to a decrease in customers, and volume of customer orders, for purchases of reagents during the six months ended June 30, 2015 relative to the six months ended June 30, 2014. We do not consider reagent sales a core aspect of our business model, and accordingly, we do not expect reagent sales to be a significant source of revenue in the future.

Grant Revenue. Grant revenue decreased by $201, from $490 for the six months ended June 30, 2014 to $289 for the six months ended June 30, 2015. This increase is due to higher research and development costs incurred under our grant with the European Union, which are reimbursable to us at 75% of cost.

Licensing Costs to Related Parties. Licensing costs to related parties decreased by $427, from $741 for the six months ended June 30, 2014 to $314 for the six months ended June 30, 2015. This decrease is primarily attributable to sublicense fees payable to Penn and GSK related to up-front fees received from five new licenses granted by us during the six months ended June 30, 2014. We recognized corresponding sublicense fees for one new license to Penn or GSK for up-front license fees during the six months ended June 30, 2015.

Costs of Reagent Sales. Costs of reagent sales decreased by $53, from $102 for the six months ended June 30, 2014 to $49 for the six months ended June 30, 2015. This decrease is relatively consistent with the decrease in reagent sales between these periods and is a result of a decrease in customers, and volume of customer orders, for purchases of reagents during the six months ended June 30, 2015 relative to the six months ended June 30, 2014. Due to the relatively low volume of reagent sales transactions, costs of reagent sales as a percentage of reagent sales may fluctuate from period to period. We do not consider reagent sales a core aspect of our business model, and accordingly, we do not expect costs of reagent sales to be a significant cost in the future.

Research and Development Expense. Research and development expense increased by $5,016, from $1,787 for the six months ended June 30, 2014 to $6,803 for the six months ended June 30, 2015. This increase was primarily attributable to the following:

- increase of $3,502 for externally sourced research and development, process development, and manufacturing of material for clinical trials related primarily to our RGX-111 program for MPS I, RGX-121 program for MPS II and RGX-314 program for wet AMD;
- increase of $748 for additional personnel costs as a result of increased headcount and stock compensation expense; and
- increase of $265 for research and development costs incurred under our grant programs.

General and Administrative Expense. General and administrative expense increased by $3,453, from $1,660 for the six months ended June 30, 2014 to $5,113 for the six months ended June 30, 2015. This increase is primarily attributable to increases of $1,775 for professional fees for legal, accounting and consulting services, an increase of $996 for additional personnel costs as a result of increased headcount and stock-based compensation expense, and an increase of $133 for the maintenance of intellectual property licensed from related parties.

Foreign Currency Transactions Losses (Gains). Foreign currency transactions losses increased by $52, from a gain of $14 for the six months ended June 30, 2014 to a loss of $38 for the six months ended June 30, 2015 due to fluctuations in the foreign currency exchange rate between the Euro to the United States Dollar and increased grant activity under our grant with the European Union.

Interest Expense. Interest expense decreased by $91, from $111 for the six months ended June 30, 2014 to $20 for the six months ended June 30, 2015. This decrease primarily was attributable to a decrease in interest expense due to the conversion of $3,792 outstanding to FoxKiser into Series C Preferred Stock in January 2015 and the termination of the services agreement with FoxKiser on January 31, 2015.

Comparison of the Years Ended December 31, 2013 and 2014

License Revenue and License Revenue from Related Party. License revenue and license revenue from a related party increased by $820, from $3,755 for the year ended December 31, 2013 to $4,575 for the year ended December 31, 2014. This increase in license revenue is primarily attributable to an increase in revenue recognized from up-front fees of $750 driven by six new licenses granted by us in 2014, as well as an increase in recurring annual maintenance fees of $185 for licenses granted prior to 2014. License revenue included $2,700 and $220 for the years ended December 31, 2013 and 2014 recognized from a license granted to Dimension.

Reagent Sales. Reagent sales decreased by $42, from $368 for the year ended December 31, 2013 to $326 for the year ended December 31, 2014. This decrease is primarily due to a significant sale of reagents to a single customer in 2013 which did not occur in 2014. We do not consider reagent sales a core aspect of our business model, and accordingly, we do not expect reagent sales to be a significant source of revenue in the future.

Grant Revenue. Grant revenue decreased by $745, from $1,964 for the year ended December 31, 2013 to $1,219 for the year ended December 31, 2014. The decrease is primarily due to significantly less research and development activity conducted under our U.S. federal grant programs, resulting in a corresponding decrease of $916 of grant revenue. The decrease in U.S. federal grant revenue was partially offset by increased costs incurred for research and development under our grant with the European Union, which resulted in a $171 increase in grant revenue.

Licensing Costs to Related Parties. Licensing costs to related parties increased by $734, from $151 for the year ended December 31, 2013 to $885 for the year ended December 31, 2014. This increase is due primarily to an increase in sublicense fees payable to Penn and GSK related to up-front fees received by us for six new licenses granted in 2014. Additionally, in 2013, $3,000 of license revenue from up-front license fees was paid to us in the form of non-cash consideration, for which we were not required to pay corresponding sublicense fees to Penn or GSK.

Costs of Reagent Sales. Costs of reagent sales decreased by $51, from $173 for the year ended December 31, 2013 to $122 for the year ended December 31, 2014. This decrease is relatively consistent with the decrease in reagent sales between these periods and is a result of a significant sale of reagents to a single customer in 2013 that did not occur in 2014. Due to the relatively low volume of reagent sales transactions, costs of reagent sales as a percentage of reagent sales may fluctuate from period to period. We do not consider reagent sales a core aspect of our business model, and accordingly, we do not expect costs of reagent sales to be a significant cost in the future.

Research and Development Expenses. Research and development expense decreased by $90, from $5,051 for the year ended December 31, 2013 to $4,961 for the year ended December 31, 2014. This decrease was primarily attributable to the following:

- decrease of $924 for externally sourced research and development performed by Penn; and
- decrease of $575 for costs incurred under our grant programs.

The decrease was partially offset by the following:

- increase of $1,037 for externally sourced research and development, process development, and manufacturing activities;
- increase of $172 for service fees from FoxKiser allocated to research and development as a result of increased headcount;
- increase of $116 for consulting services;
- increase of $60 for stock compensation expense for research and development personnel; and
- increase of $25 for license fees to access technology for use in research and development.

General and Administrative Expenses. General and administrative expense decreased by $1,623, from $5,474 for the year ended December 31, 2013 to $3,851 for the year ended December 31, 2014. This decrease is primarily attributable to a decrease of $2,450 in compensation to related parties for transaction services related to the license with Dimension as well as professional fees for legal, accounting and consulting services. The decrease is partially offset by increases of $259 in stock compensation expense, $227 in services from FoxKiser allocated to general and administrative expenses as a result of higher headcount, $124 in license maintenance fees, and $102 in recruiting costs for the hiring of additional personnel.

Foreign Currency Transaction Losses. Foreign currency transaction losses increased by $16, from $14 for the year ended December 31, 2013 to $30 for the year ended December 31, 2014. This is due to fluctuations in foreign currency exchange rate of the Euro to the United States Dollar and increased grant activity under our grant with the European Union.

Other Operating Income. Other operating income increased by $47, from $0 for the year ended December 31, 2013 to $47 for the year ended December 31, 2014. This increase is due to the sale of manufacturing materials to Dimension, a related party, that we previously purchased for use in our own research and development.

Interest Expense. Interest expense decreased by $290 from $611 for the year ended December 31, 2013 to $321 for the year ended December 31, 2014. This decrease was due to the conversion of $5,892 of debt to FoxKiser into Series B Preferred Units, which occurred in October 2013 resulting in less debt outstanding during 2014.

Liquidity and Capital Resources

We have funded our research and development and operating activities principally from the sale of preferred units and convertible preferred stock, and the issuance of debt with share settlement options. Additionally, we have supplemented our cash flows with up-front fees received from granting commercial licenses to our proprietary technology to other biotechnology and pharmaceutical companies.

As of December 31, 2014, we had cash and cash equivalents of $1,121 and amounts outstanding to FoxKiser of $1,423 under the services agreement and $2,403 in promissory notes. On January 13, 2015, we completed the sale and issuance of 4,631,774 shares of Series C Preferred Stock, par value $0.0001 per share, at a per share price of $6.477 for aggregate gross proceeds of $30,000. The aggregate purchase price of $30,000 included $26,208 of cash proceeds and the conversion $3,792 of debt by FoxKiser. On May 15, 2015, we completed the sale and issuance of 7,366,849 shares of Series D Preferred Stock, par value $0.0001 per share, at a per share price of $9.5699 generating aggregate gross proceeds of $70,500. As of June 30, 2015, we had cash and cash equivalents of $85,215 and had no debt outstanding.

We have incurred losses since our inception and, as of June 30, 2015, had an accumulated deficit of $39,110. We anticipate that we will continue to incur losses for at least the next several years. We expect that our research and development and general and administrative expenses will continue to increase and, as a result, we will need additional capital to fund our operations, which we may obtain through one or more equity offerings, debt financings or other third-party funding, including potential strategic alliances and licensing or collaboration arrangements.

Cash Flows

	Year Ended December 31,		Six Months Ended June 30,	
	2013	2014	2014	2015
Net cash provided by (used in) operating activities	$(3,012)	$(2,399)	$1,017	$ (9,677)
Net cash used in investing activities	—	—	—	(315)
Net cash provided by financing activities	1,965	2,401	—	94,086
Net increase (decrease) in cash and cash equivalents	$(1,047)	$ 2	$1,017	$84,094

Operating Activities

The significant decrease in cash provided by operating activities for the six months ended June 30, 2015, compared to the six months ended June 30, 2014, is primarily attributable to a decrease in license revenue and

significant increases in research and development and general and administrative expenses as a result of increased head count, spending on the advancement of our lead product candidates and overhead. The decrease in cash used in operating activities during the year ended December 31, 2014, compared to the year ended December 31, 2013, was primarily attributable to up-front license fees received from six new licenses granted during the year ended December 31, 2014 which were not present during the year ended December 31, 2013.

For the six months ended June 30, 2015, our net cash used in operating activities of $9,677 consisted of a net loss of $10,301, primarily attributable to general and administrative and research and development expenses, decreased by changes in working capital of $108, offset by $732 in adjustments for non-cash items. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $711. The change in working capital was primarily attributable to an increase in accounts payable and accrued expenses of $1,652, a decrease in related party receivables of $750, and a decrease in unbilled receivables of $327 primarily consisting of reimbursements due to us under our grant with the European Union partially offset by a decrease in amounts due to a related party of $1,876 and an increase in prepaid expenses of $1,142.

For the six months ended June 30, 2014, our net cash provided by operating activities of $1,107 consisted of net income of $123, primarily attributable to increased license revenue from up-front fees on licenses granted during the period and $894 of cash provided by changes in working capital. The change in working capital was primarily attributable to an increase in service fees payable to FoxKiser of $1,400, a decrease in related party receivables of $924, partially offset by a decrease in other related party payables of $1,051, increases in trade accounts receivables of $668 and unbilled receivables of $235 primarily consisting of up-front license fees due to us by licensees and reimbursements due to us under our grant with the European Union.

For the year ended December 31, 2014, our net cash used in operating activities of $2,399 consisted of a net loss of $4,003, primarily attributable to general and administrative and research and development expenses, offset by $494 in adjustments for non-cash items and $1,110 of cash provided by changes in working capital. Adjustments for non-cash items primarily consisted of stock-based compensation expense of $319. The change in working capital was primarily attributable to an increase in accounts payable and accrued expenses of $797, an increase in service fees payable to FoxKiser and other related party payables of $1,026, an increase in advance payments of $153, a decrease in related party receivables of $174, partially offset by an increase in trade receivables of $799 and unbilled receivables of $213 primarily consisting of reimbursements due to us under our grant with the European Union.

For the year ended December 31, 2013, our net cash used in operating activities of $3,012 consisted of a net loss of $5,387, primarily attributable to general and administrative and research and development expenses, decreased by $289 in adjustments for non-cash items and partially offset by $2,664 of cash provided by changes in working capital. Adjustments for non-cash items primarily consisted of $303 of non-cash consideration received for licenses granted. The change in working capital was primarily attributable to an increase in service fees payable to FoxKiser of $3,192, an increase in accounts payable and accrued expenses of $364, a decrease in unbilled receivables of $109, partially offset by an increase in related party receivables of $924.

Investing Activities

For the six months ended June 30, 2015, net cash used in investing activities consisted of $315 to purchase property and equipment.

We had no cash flows from investing activities for the years ended December 31, 2013 and 2014 and the six months ended June 30, 2014.

Financing Activities

For the six months ended June 30, 2015, net cash provided by financing activities primarily consisted of $26,021 in net proceeds from the sale and issuance of Series C Preferred Stock and $67,998 in net proceeds from the sale and issuance of Series D Preferred Stock.

For the year ended December 31, 2014, net cash provided by financing activities primarily consisted of $2,400 in net proceeds from the issuance of promissory notes to FoxKiser.

For the year ended December 31, 2013, net cash provided by financing activities consisted of $1,965 in net proceeds from the sale and issuance of Series B Preferred Units of ReGenX Biosciences, LLC (our predecessor entity).

We had no cash flows from financing activities for the six months ended June 30, 2014.

Future Funding Requirements

To date, we have generated a limited amount of revenue through license agreements with strategic partners for research, development, and commercialization of product candidates using our proprietary technology. Additionally, we have generated revenue from grant programs and sales of licensed reagents to customers for use in research and development, for which we do not expect significant future revenue. We do not expect to generate significant recurring revenue unless and until we obtain regulatory approval for and commercialize our product candidates. In addition, we expect our expenses to increase in connection with our ongoing development activities, particularly as we continue to expand the research, development and clinical trials of, and seek regulatory approval for, our internally developed product candidates. Following the closing of this offering, we expect to incur additional costs associated with operating as a public company. In addition, subject to obtaining regulatory approval for our internally developed product candidates, we expect to incur significant commercialization expenses for product sales, marketing, manufacturing and distribution. We anticipate that we will need substantial additional funding in connection with our continuing operations.

Based upon our current operating plan, we believe that the net proceeds from this offering, together with our existing cash and cash equivalents, will enable us to fund our operating expenses and capital expenditure requirements through at least the next 24 months. We intend to devote the majority of the net proceeds from this offering for clinical development and regulatory approval of our internally developed product candidates. We have based our estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of product candidates.

Our future capital requirements will depend on many factors, including:

- our planned expansion of the licensing of our NAV Technology Platform;
- the results of our preclinical studies for our Lead Product Candidates and any subsequent clinical trials;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials, if any, for our other product candidates;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- revenue, if any, received from commercial sale of our products, should any of our product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements remaining in effect;

- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the costs associated with being a public company.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our product revenues, if any, and any commercial milestones or royalty payments under our license agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. In addition, revenue from our NAV Technology Platform sublicensing is dependent in part on the clinical and commercial success of our licensing partners. Neither we nor any of our NAV Technology Licensees have commercialized any products using our NAV Technology Platform. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

To the extent that additional capital is raised through the sale of equity or equity-linked securities, the issuance of those securities could result in substantial dilution for our existing stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our existing stockholders. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline and existing stockholders may not agree with our financing plans or the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with collaborative partners or otherwise that may require us the relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Contractual Obligations, Commitments and Contingencies

Our principal commitments consist of obligations under vendor contracts to provide research services and other purchase commitments with our vendors. Additionally, our commitments consist of obligations to our licensors under our in-license agreements, which include sublicense fees, milestones fees, royalties and reimbursement of patent maintenance costs. These amounts are not fixed and determinable.

The amount and timing of when these payments will actually be made is uncertain and the payments are contingent upon the initiation and completion of future activities, including services to be provided by our vendors. Sublicense fees are due to the licensors when we sublicense licenses to third-parties; the fees are based on a percentage of the sublicense fees received from the sublicensees. Milestone fees are payable by us upon our future achievement of certain development and regulatory milestones. Royalty fees are based on a percentage of net sales of licensed products. Maintenance costs are reimbursements to the licensors for maintaining licensed patents. For further information regarding these agreements and amounts that could become payable in the future, please see “License Agreements and Research Collaborations—Platform Licenses” located elsewhere in this document.

As of December 31, 2014, we had no contracts or other commitments with minimum payment obligations.

In March 2015, we entered into a lease agreement for our corporate headquarters in Rockville, Maryland. Additionally, we have entered into a short-term lease agreement for laboratory space in Philadelphia, Pennsylvania. Future minimum lease payments are as follows:

	Total	Less Than 1 Year	Years 1-3	Years 4-5	More Than 5 Years
Future minimum lease payments	$1,577	$ 83	$908	$586	$ —

Off-Balance Sheet Arrangements

During the periods presented, we did not have, nor do we currently have, any off-balance sheet arrangements as defined under Securities and Exchange Commission rules.

Quantitative and Qualitative Disclosure about Market Risk

Interest Rate Sensitivity

Our primary exposure to market risk for our cash and cash equivalents is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. However, we do not believe a sudden change in the interest rates would have a material impact on our financial condition or results of operations.

We were subject to interest rate risk in connection with our debt instruments outstanding at December 31, 2014 bearing variable interest rates. We have no debt instruments outstanding at June 30, 2015.

Concentrations of Credit Risk

We deposit our cash with financial institutions that we consider to be of high credit quality and purchase marketable securities which are generally investment grade, liquid, short-term fixed income securities and money-market instruments denominated in U.S. dollars. Our marketable securities consist of certificates of deposit, commercial paper, corporate notes and U.S. government agency notes.

Three customers accounted for approximately 76% of our total revenue for the year ended December 31, 2013. No other customer accounted for more than 10% of revenue in 2013. Two customers accounted for approximately 47% of our total revenue for the year ended December 31, 2014. No other customer accounted for more than 10% of revenue in 2014. We do not consider reagent sales a core aspect of our business model and we do not dedicate significant resources to sales efforts for reagents. Accordingly, future revenue from sales of reagents is uncertain and may fluctuate significantly from period to period.

Foreign Currency Risk

Our results of operations and cash flows are subject to fluctuations due to changes in foreign currency exchange rates. A substantial majority of our expenses are denominated in U.S. Dollars, with the remainder in Euros. Our results of operations and cash flow are, therefore, subject to fluctuations due to changes in foreign currency exchange rates and may be harmed in the future due to changes in foreign exchange rates. To date, we have not entered into any hedging arrangements with respect to foreign currency risk or other derivative instruments. The effect of a hypothetical 10% change in foreign currency exchange rates applicable to our business would not harm our operations.

BUSINESS

Overview

We are a leading biotechnology company focused on the development, commercialization and licensing of recombinant adeno-associated virus (AAV) gene therapy. In AAV gene therapy, the viral genes are removed from the AAV, a small, non-pathogenic cold virus, creating a biological delivery vehicle called a vector. A therapeutic gene sequence is then inserted, creating a recombinant vector. Our proprietary AAV gene delivery platform (our NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10 (NAV Vectors). Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing gene therapy products administered directly into the body, or *in vivo*, based on our NAV Technology Platform. We seek to accomplish our mission through a combination of our internal development efforts and the efforts of our third-party licensees (NAV Technology Licensees). Our NAV Technology Platform is currently being applied in the development of 23 product candidates for a variety of diseases, including five internally developed product candidates and 18 partnered product candidates developed by our NAV Technology Licensees. Most of our NAV Technology Licensees have licensed specific NAV Vectors for the indications they are pursuing. We maintain rights to all unlicensed indications as well as retaining the right to our NAV Technology Platform for unlicensed vectors in disease indications for which we have granted licenses.

We are applying our NAV Technology Platform in an effort to generate a broad pipeline of best-in-class and often first-in-class AAV gene therapy treatments. Our NAV Technology Platform is covered by more than 100 licensed patents and patent applications worldwide. Our product candidates, which are designed for a variety of diseases, incorporate proprietary advances in AAV gene therapy that significantly enhance their profiles as potential therapeutics. The benefits of our NAV Technology Platform have been observed across several clinical trials and studies conducted by our development partners and third-party investigators. Approximately 70% of all AAV gene therapy clinical trials relating to new treatment Investigational New Drug applications (INDs) posted on the United States government clinical trials database from 2012 through 2014 used NAV Vectors.

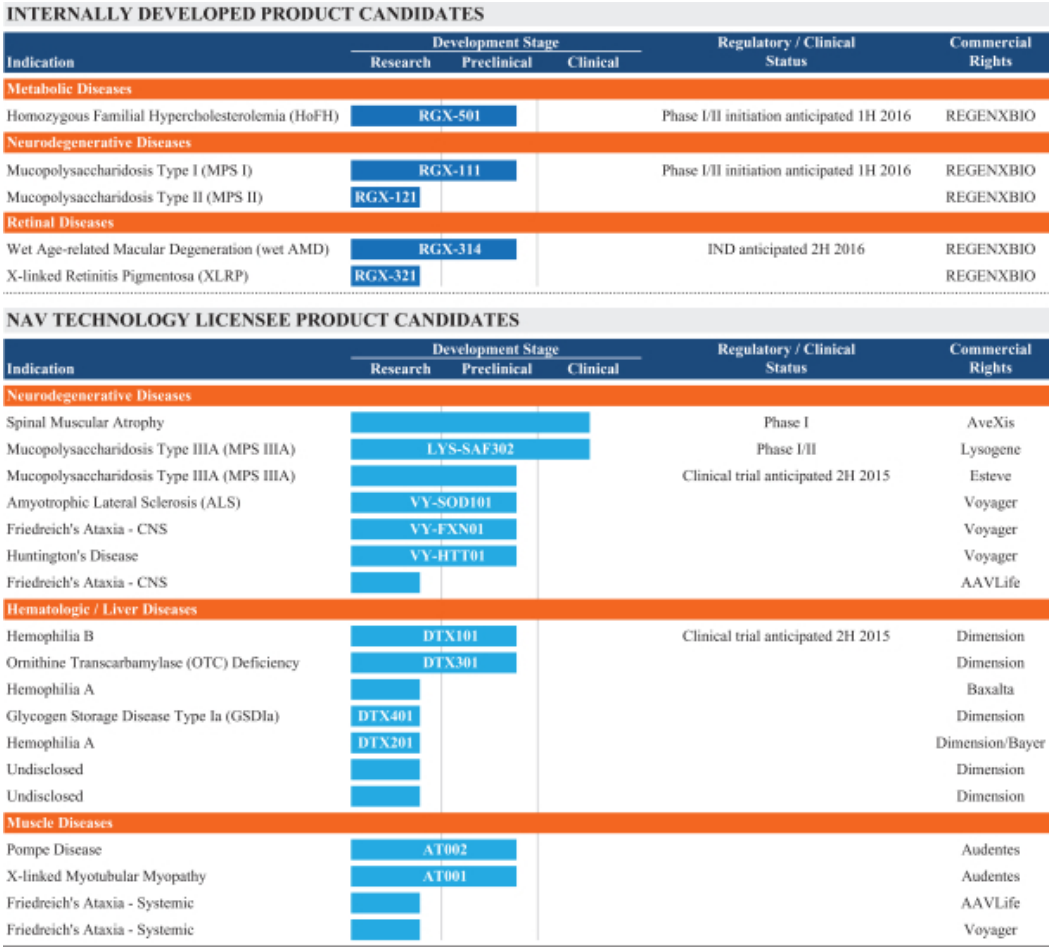
The foundation of our NAV Technology Platform was discovered in an effort to identify next generation AAV vectors that could overcome the limitations of earlier generation AAV vectors (AAV1 through AAV6). We believe the key benefits of NAV Vectors over earlier generation AAV vectors include:

- higher gene expression;
- longer-term gene expression;
- broad and novel tissue selectivity;
- lower immune response; and
- improved manufacturability.

We believe that gene therapies using our NAV Technology Platform (NAV Gene Therapy) have the potential to transform the treatment paradigm for patients with a wide range of severe diseases with significant unmet medical needs. NAV Vectors have demonstrated stable expression in animals for over eight years. Moreover, AAV8 vectors have demonstrated stable expression for over four years in a clinical trial for the treatment of hemophilia B.

In certain monogenic, recessive diseases, NAV Gene Therapy may provide clinical benefits for patients that are substantially greater than currently available therapies. In other types of diseases, such as hemophilia, NAV Gene Therapy has the potential to replace a lifetime of continuous treatment of standard protein replacement therapy and other treatment approaches with a single treatment, which could reduce health care system costs while also improving patients' quality of life. We believe that the potential efficiency and broad applicability of our NAV Technology Platform may allow us to develop NAV Gene Therapy treatments that are injected or infused into the bloodstream, spinal fluid or directly into the target tissue to treat a wide range of diseases.

Our internal and partnered product development program pipeline is shown below.



INTERNALLY DEVELOPED PRODUCT CANDIDATES

Indication	Development Stage: Research	Development Stage: Preclinical	Development Stage: Clinical	Regulatory / Clinical Status	Commercial Rights
Metabolic Diseases					
Homozygous Familial Hypercholesterolemia (HoFH)	RGX-501			Phase I/II initiation anticipated 1H 2016	REGENXBIO
Neurodegenerative Diseases					
Mucopolysaccharidosis Type I (MPS I)	RGX-111			Phase I/II initiation anticipated 1H 2016	REGENXBIO
Mucopolysaccharidosis Type II (MPS II)	RGX-121				REGENXBIO
Retinal Diseases					
Wet Age-related Macular Degeneration (wet AMD)	RGX-314			IND anticipated 2H 2016	REGENXBIO
X-linked Retinitis Pigmentosa (XLRP)	RGX-321				REGENXBIO

NAV TECHNOLOGY LICENSEE PRODUCT CANDIDATES

Indication	Development Stage: Research	Development Stage: Preclinical	Development Stage: Clinical	Regulatory / Clinical Status	Commercial Rights
Neurodegenerative Diseases					
Spinal Muscular Atrophy				Phase I	AveXis
Mucopolysaccharidosis Type IIIA (MPS IIIA)	LYS-SAF302			Phase I/II	Lysogene
Mucopolysaccharidosis Type IIIA (MPS IIIA)				Clinical trial anticipated 2H 2015	Esteve
Amyotrophic Lateral Sclerosis (ALS)	VY-SOD101				Voyager
Friedreich's Ataxia - CNS	VY-FXN01				Voyager
Huntington's Disease	VY-HTT01				Voyager
Friedreich's Ataxia - CNS					AAVLife
Hematologic / Liver Diseases					
Hemophilia B	DTX101			Clinical trial anticipated 2H 2015	Dimension
Ornithine Transcarbamylase (OTC) Deficiency	DTX301				Dimension
Hemophilia A					Baxalta
Glycogen Storage Disease Type Ia (GSDIa)	DTX401				Dimension
Hemophilia A	DTX201				Dimension/Bayer
Undisclosed					Dimension
Undisclosed					Dimension
Muscle Diseases					
Pompe Disease	AT002				Audentes
X-linked Myotubular Myopathy	AT001				Audentes
Friedreich's Ataxia - Systemic					AAVLife
Friedreich's Ataxia - Systemic					Voyager

We currently plan to build internal gene therapy franchises in the metabolic, neurodegenerative and retinal therapeutic areas, and develop multiple product candidates in each area. Our most advanced programs are for the treatment of two severe genetic diseases, homozygous familial hypercholesterolemia (HoFH) and Mucopolysaccharidosis Type I (MPS I). We expect these programs to enter Phase I/II clinical trials in the first half of 2016. We also have a program for wet age-related macular degeneration (wet AMD) that is in the preclinical stage and for which we expect to file an IND in the second half of 2016.

Our partnered development pipeline benefits from the disease-specific expertise of our NAV Technology Licensees. Our partnering strategy provides us the flexibility to sublicense development of treatments designed to address significant unmet medical needs, while remaining focused on our core programs and therapeutic areas internally, which we believe enables us to achieve maximum value. We believe that the broad applicability of our NAV Technology Platform and any clinical successes of the treatments utilizing NAV Vectors will create new internal and partnered pipeline opportunities.

As an innovator in AAV gene therapy development, our intellectual property strategy is designed to provide us with extensive protection for our product candidates and our NAV Technology Platform. We currently have exclusive rights to over 100 patents and patent applications worldwide covering our NAV Vectors, including composition of matter claims for AAV7, AAV8, AAV9 and AAVrh10, as well as methods for their manufacture and therapeutic uses. We believe this patent portfolio forms a strong foundation for our current programs and with our ongoing research and development, we expect to continue to expand this substantial patent portfolio. Our licensed patents not only seek to protect our key assets - our NAV Technology Platform and our internal product candidates - they also form the basis for licensing and partnering arrangements.

Our company was formed from a successful collaboration that began in February 2009 between FoxKiser LLP, the University of Pennsylvania (together with The Trustees of the University of Pennsylvania, Penn) and gene therapy pioneer James Wilson, M.D., Ph.D. We have built on the foundation of this collaboration to produce what we believe to be compelling NAV Gene Therapy product candidates derived from discoveries and research in Dr. Wilson's lab. As our team has grown, we have continued to build on our scientific foundation, adding depth in gene therapy and biotechnology leadership. Our management team includes leaders who are experienced in building and operating innovative healthcare ventures and have expert knowledge in the development of AAV gene therapy. We believe the strength of our team coupled with the depth of knowledge of our scientific founder and advisors position us to succeed in developing and bringing to market, independently or with our development partners, unique, best-in-class gene therapy treatments for a range of severe diseases with significant unmet medical needs.

Our Strategy

Our mission is to transform the lives of patients suffering from severe diseases with significant unmet medical needs by developing and commercializing *in vivo* gene therapy products based on our NAV Technology Platform. We are seeking to develop, manufacture, commercialize and license product candidates across multiple therapeutic areas while continuing to expand our NAV Technology Platform. To achieve our mission, we are pursuing the following strategies:

- **Apply our proprietary, next generation AAV vector technology to develop *in vivo* gene therapies for patients.** We believe *in vivo* gene therapy is an ideal treatment paradigm for many diseases with sub-optimal or non-existent therapies because of its potential to correct an underlying genetic defect, rather than just treating a patient's symptoms. We believe our NAV Technology Platform will prove to be a significant advancement over earlier AAV vectors. Based on data derived from third-party clinical studies using our NAV Vectors, we believe our NAV Technology Platform possesses unique, beneficial properties that are not seen in earlier generation AAVs. We believe that our NAV Technology Platform, which underpins our internal development programs and the programs of our NAV Technology Licensees, will enable us and our partners to develop best-in-class gene therapy candidates for a wide range of disease targets due to these unique properties.
- **Focus on rapidly advancing our internal lead proprietary development programs in metabolic, neurodegenerative and retinal diseases.** Both HoFH and MPS I are diseases with high unmet clinical need and current treatments that are sub-optimal or non-existent. We plan to file an IND for HoFH in the second half of 2015 and initiate a Phase I/II clinical trial starting in the first half of 2016. We expect to file an IND and initiate a Phase I/II clinical trial for MPS I starting in the first half of 2016. If we are successful in achieving proof-of-concept in the Phase I/II clinical trials for these diseases, we will pursue registration trials and commercialization of such product candidates. In addition, we plan to progress our product development program for wet AMD toward clinical trials and expect to file an IND in the second half of 2016.
- **Establish gene therapy franchises in our current core therapeutic areas of metabolic, neurodegenerative and retinal diseases.** After human proof-of-concept is achieved in a disease, we believe we will be able to apply what we have learned and use our NAV Technology Platform to more rapidly develop new product candidates for many similar diseases. Once an appropriate vector and

route of administration for a particular disease type have been established, a new gene can be inserted into the appropriate vector and the established route of administration can be used for other similar diseases. We expect to use this approach to further build the foundation for our neurodegenerative disease franchise by quickly moving to IND-filing and clinical trials for our MPS II program if we are able to demonstrate human proof-of-concept in MPS I. We believe that this approach is also applicable to metabolic and retinal diseases, as well as many other therapeutic areas, and will allow us to efficiently generate product candidates for diseases in and beyond our current areas of therapeutic focus.

- **Further grow the pipeline of products based on our NAV Technology Platform through strategic in-licensing and sublicensing of new programs.** We also plan to grow the pipeline of commercial product development programs using our NAV Technology Platform through licensing. For example, we plan to pursue in-licensing for programs we deem to be the most promising research programs using our NAV Vectors. We intend to continue to selectively sublicense our NAV Technology Platform for specific vector and indication combinations to additional NAV Technology Licensees. Strategic sublicensing allows us to maintain our internal product development focus in our core disease indications and therapeutic areas while still expanding the NAV Gene Therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue.
- **Maintain and grow our extensive intellectual property portfolio.** We plan to leverage our intellectual property rights and substantial expertise in AAV gene therapy in order to develop and commercialize NAV Gene Therapy treatments. We have licensed exclusive rights to a broad portfolio of certain fundamental AAV gene therapy patents and patent applications. In securing these rights, we have focused on obtaining robust rights for those intellectual property assets we believe will be most important in providing us with a competitive advantage with respect to AAV gene therapy treatments. We plan to continue to seek to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business.

Our Strengths

We believe our technology, expertise and know-how will allow us to maintain our leadership position in the gene therapy field. Our strengths include the following:

- Our NAV Technology Platform, for which we have an exclusive worldwide license.
- Strong clinical data supporting proof-of-concept of our NAV Technology Platform from three separate reported Phase I/II third-party clinical trials using AAV8 for the treatment of hemophilia B and a clinical trial using AAV9 for the treatment of spinal muscular atrophy (SMA).
- The largest pipeline of programs in AAV gene therapy with 23 total product candidates that use our NAV Technology Platform, consisting of our five internal programs and 18 partnered product candidates being developed by our NAV Technology Licensees.
- Two internal programs, RGX-501 for the treatment of HoFH and RGX-111 for the treatment of MPS I, which we expect to advance into clinical trials in the first half of 2016.
- Two ongoing clinical trials being conducted by our NAV Technology Licensees targeting diseases, SMA and Mucopolysaccharidosis Type IIIA (MPS IIIA), for which there are no currently approved treatments.
- Our NAV Technology Platform expertise, which allows us to apply what we may learn in a specific disease program to similar diseases, thus allowing us to rapidly develop additional product candidates for related disease indications.
- Our long-standing relationships with academics, leading research institutions, scientists and scientific advisors who have vast experience in the field of gene therapy and contribute key insights and significant developments to the field.

The Broad Potential and Application of Gene Therapy

The concept of developing human therapies involving the delivery of external genes has existed for decades, driven by the arrival of recombinant technology and the early demonstrations by scientists of the ability to deliver and drive expression of external gene sequences in mammalian cells.

We believe that gene therapy has the potential to become a new and important class of treatment because it may offer the following benefits:

- **Ability to treat a broad range of diseases.** Given the availability of the sequence of the entire human genome, it could be possible to design gene therapy to express or effect expression of any human protein whose presence, absence or activity causes disease.
- **Ability to target mechanisms that cannot be targeted effectively by existing drug classes.** Many proteins that play roles in disease cannot be targeted effectively with small molecules and therapeutic proteins. These limitations on small molecule and protein drugs may not apply to gene therapy, which we believe can be designed to target any gene in the genome.
- **Inherently specific, natural and therefore potent mechanism of action.** Gene therapy is designed to result in proteins specifically targeting the underlying cause of a disease and that are produced naturally in humans. This mechanism has the inherent theoretical benefit of creating more potent treatments with a reduced risk of inactivation.
- **Simplified discovery of treatment candidates.** Identification of small molecule and protein drug candidates typically requires screening of a large number of potential candidates to find prospective leads. Identification of gene therapy candidates has the potential to be simpler and take considerably less time because it can involve relatively standard processes that can be applied in a similar fashion to many successive product candidates.
- **Ability to create convenient treatment profiles.** Because gene therapies are designed to deliver a long-term effect with a single administration, a single gene delivered via gene therapy could potentially do the same work of administering conventional drugs for many years.

Historically, the primary challenge for gene therapy has been the delivery of genes into cells. Genes are made of deoxyribonucleic acid (DNA), which is a large, highly charged molecule that is difficult to transport across a cell membrane and deliver to the nucleus, where it can be transcribed and translated into protein. The genetic material needs to be delivered efficiently and to the desired target tissues and cell types, which will vary depending on the disease to be treated. Based on this need, scientists have designed and developed a variety of gene vectors in order to facilitate gene delivery in cells.

To date, the study of gene vectors as treatments in humans has involved approaches with *in vivo* and *ex vivo* techniques using a variety of different gene vectors. Each approach presents different features and benefits for the treatment of a particular disease. *Ex vivo* gene therapy approaches generally are employed to target correction in blood and bone marrow. These methods typically involve harvesting and isolating a patient's own cells. Both the patient and cells undergo several preparatory steps to allow for modification of the cells by gene vectors. Ultimately, the modified cells are re-administered to the patient. *In vivo* gene therapy approaches involve directly administering (e.g., by infusion or injection) gene vectors into patients in order to reach desired cells in target tissues (e.g., liver, brain, eye, muscle, heart). These methods rely on a combination of the route of administration and the gene vectors themselves to facilitate the correction in the target tissues. We focus on *in vivo* gene therapy.

Among vectors available for *in vivo* gene therapy, viral vectors have been adopted with the greatest frequency because they have demonstrated the greatest efficiency in gene delivery to date. This efficiency exists because viral vectors are derived from naturally occurring viruses whose normal life-cycle relies on gene

delivery of their own genomes. In other words, they are naturally optimized to deliver genes to cells. Many viral vectors have presented sub-optimal safety profiles for *in vivo* treatment in humans because the viruses from which they are derived are pathogenic (causing disease), immunogenic (causing immune response) or create genomic toxicity (delivering a gene to a place where it interrupts normal function). Vectors derived from adenovirus, herpes virus and retroviruses have been tested as *in vivo* viral vectors.

Vectors derived from AAV have among the best safety profiles for gene therapy given that AAVs are not known to be associated with disease in humans. The earlier generation AAV vectors were designed by scientists in the mid-1980s and the first clinical trials using AAV began in the mid-1990s. There were only a handful of AAV vectors available to scientists at the time of the first clinical trials because AAV vectors were designed based on the capsid (the protein shell of a virus that encloses the genetic material of the virus) of AAV viruses known to be in existence and only six distinct serotypes (groups within a single species of microorganisms, such as bacteria or viruses, which share distinctive surface structures) had been discovered at that time. These earlier generation AAV vectors were shown to be limited in their application due to a variety of limitations and challenges, including:

- low or unmeasurable gene expression, meaning the delivered gene was enabling production of low or unmeasurable amounts of the therapeutic protein;
- short-term gene expression, meaning if gene expression was measurable, it was transient;
- limited tissue selectivity, meaning concentrated gene expression was not observed in the target organ; and
- high levels of immune response, meaning the body may neutralize the gene delivery vector with pre-existing antibodies or generate T-cells that inhibit the therapeutic effect.

Discovery of Next Generation AAV

In recognition of the limitations and challenges of earlier generation AAV vectors, an effort was undertaken in the early 2000s at Penn to discover other naturally occurring AAV sequences. The identification of such sequences was based on the observation that wild-type AAV (in contrast to recombinant AAV) can undergo a latent cycle in which the AAV genome stays within the cell, meaning the virus, including its capsid gene sequence, remains intact within the cell but does not reproduce. This allowed for identification of new sequences not by purifying viruses from tissues, but by searching for capsid gene sequences in a variety of tissues isolated from non-human primates and from humans, based on regions of the AAV capsid gene that did not vary between the known AAV vector. By searching for capsid gene sequences in this manner, many more capsid protein sequences were discovered than would have been found by purifying viruses from tissues.

More than 100 new capsid sequences were identified by the process. The first few were initially designated AAV7, AAV8 and AAV9, after which, other sequences were identified by species from which it was isolated (e.g., “rh” indicating rhesus macaque) followed by a number (e.g., 10, for rh10). Early characterization of the initial discoveries of AAV7, AAV8, AAV9 and AAVrh10 suggested that these vectors may be significantly more efficient in various applications important for clinical translation than other previously known AAVs.

After patenting the next generation AAV vectors, Penn initiated a distribution program through a material-transfer process that enabled researchers to access the next generation AAV vectors for research use only, under specific restrictions. Thousands of custom reagents were sent to independent researchers, who began to characterize and validate the beneficial features of AAV vectors in animal models of disease. In 2010, the first clinical trials were conducted using the next generation AAV vectors and initial proof-of-concept and safety in humans was established from these trials. These clinical trials also produced longer-term efficacy results which reinforced our belief that these next generation vectors have beneficial properties not seen in the earlier generation AAV vectors.

We believe the next generation AAV vectors, which form the basis of our NAV Technology Platform, have many improved properties relative to earlier generation AAV vectors for development and commercialization of AAV treatments, including:

- higher gene expression;
- longer-term gene expression;
- broad and novel tissue selectivity;
- lower immune response; and
- improved manufacturability.

Our Proprietary NAV Technology Platform for Gene Delivery

Our NAV Technology Platform has been used in several clinical trials conducted by our partners and third-party investigators. In 2009, we licensed rights to the next generation AAV vectors discovered at Penn. Our NAV Vectors form the foundation of our NAV Technology Platform.

We are developing therapeutics using NAV Vectors that contain genes which are synthesized to code for the expression of therapeutic proteins in target cells to correct the underlying causes of the diseases we seek to treat. Each product candidate is designed with a NAV Vector for a specific cell target and to express a specific protein. We incorporate proprietary modifications to both the AAV and the gene which enhance properties such as potency, stability and tissue distribution. Our proprietary modifications, including the use of vectors derived from novel sequences of AAV such as AAV7, AAV8, AAV9 and AAVrh10, are protected by over 100 licensed patents and patent applications. The rights to our NAV Technology Platform provide our product candidates with what we believe to be a competitive advantage over product candidates developed with earlier generation AAV vectors due to the novel and beneficial properties of our NAV Vectors.

Clinical Validation of Our NAV Technology Platform

History of the Development of AAV8 in the Treatment of Hemophilia B

Hemophilia is a genetic bleeding disorder that prevents the blood from clotting normally. The main symptom is uncontrolled, often spontaneous bleeding. Internal bleeding into the joints can result in pain, swelling and, if left untreated, can cause permanent damage. Hemophilia B is caused by mutations in the gene encoding the clotting factor, Factor IX (FIX).

A collaboration among scientists and clinicians at St. Jude Children's Research Hospital and University College London established the first human proof-of-concept using AAV8 to deliver and express a gene in the liver. The results of these translational studies and clinical trial present an informative translational road map.

- **Mice studies demonstrate correction of bleeding episodes.** In 2006, preclinical studies were reported involving a single intravenous administration of an AAV8 vector encoding the human Factor IX (hFIX) gene that resulted in greater than normal levels of hFIX and correction of the bleeding diathesis in FIX knock-out mice.
- **Non-human primate studies demonstrate long-term hFIX expression.** In 2011, preclinical studies were reported involving a single intravenous administration of an AAV8 vector encoding the hFIX gene that resulted in peak levels of hFIX of approximately 420% of normal.
- **Human clinical trial demonstrates reduction in disease severity.** In 2011, the New England Journal of Medicine published results from a clinical trial involving a single, intravenous administration of an AAV8 vector encoding the hFIX gene to six subjects with severe hemophilia B. This trial resulted in increased levels of hFIX sufficient to improve severe hemophilia B to a mild or moderate disease state.

Researchers at St. Jude Children's Research Hospital, studying hemophilia B patients, used AAV8 encoding the FIX protein delivered intravenously to target the liver, and used the liver as a depot for producing and secreting the needed FIX. In the study, six patients with FIX levels of less than one percent of normal were treated in an ascending dose study and then the highest dose was extended to another four patients. Reports indicate that the treatment was well-tolerated and demonstrated therapeutic, sustained levels of FIX expression in all patients. All patients were expressing levels of FIX post-treatment at levels at or above two percent of normal, converting them to patients with moderate disease. Several high dose patients have sustained levels at eight percent of normal, placing them in the mild disease group. Most patients have been able to stop prophylactic FIX infusions. The main vector-related adverse event was an elevated serum alanine aminotransferase level, which we believe may be attenuated by a short, tapering course of steroid.

The clinical trial described above was the strongest example of efficacy evidence in any AAV vector clinical trial. Expression has been stable, with the earliest dosed patient showing expression and long-lasting amelioration of bleeding episodes for over four years. A previous clinical trial in hemophilia B using AAV2 did not have observed evidence of efficacy, so the St. Jude clinical trial was notable for reporting evidence of preliminary efficacy in this disease and in suggesting the importance of NAV Vectors.

Subsequently, two additional groups have reported human proof-of-concept using AAV8-mediated gene therapy to deliver and express a gene in the liver for the treatment of hemophilia B. NAV Vectors have been further validated in a more recent clinical trial by Baxalta US Inc. (formerly Baxter Healthcare) (Baxalta) in hemophilia B. Baxalta employs an AAV8 vector which differs by encoding a gene for a naturally occurring mutant of FIX which has higher activity. Baxalta has recently reported that two patients in the mid-dose cohort have experienced no bleeds without regular infusions of FIX and one of these patients has had sustained FIX expression levels of 20% to 25% of normal FIX levels for 12 months. In the highest dose cohort, FIX expression levels have peaked above 50%, though the two patients in this cohort experienced an immune response which has led to decreased FIX expression, with one patient resuming regular FIX infusions. Baxalta has exclusive rights to use AAV8 for the treatment of hemophilia B from a license executed directly with GSK which predates our licensing of the NAV Technology Platform.

Clinical Use of NAV Technology

Our NAV Technology has been used by our NAV Technology Licensees in clinical trials. In 2010, major milestones were achieved with the initiation of two investigator-sponsored studies using NAV Vectors. As noted above, clinical trials for hemophilia B using AAV8 have met safety and efficacy endpoints. The hemophilia B study has generated evidence of durable gene expression in patients for over four years. Since the initial investigator-sponsored trials began in 2010, we believe 12 additional clinical trials have been initiated using NAV Vectors by our NAV Technology Licensees or other third parties. All diseases targeted in 14 clinical trials using NAV Vectors of which we are aware are set forth in the graphic below.

2010–2011	2012–2013	2014–2015
▪ Mucopolysaccharidosis Type IIIA	▪ Limb Girdle Muscular Dystrophy Type 2D	▪ Duchenne Muscular Dystrophy
▪ Batten's Disease (LINCL)	▪ Metachromatic Leukodystrophy	▪ Giant Axonal Neuropathy
▪ Hemophilia B	▪ Hemophilia B	▪ X-linked Retinoschisis
	▪ Hemophilia B	▪ Alpha-1 Antitrypsin Deficiency
		▪ Pompe Disease
		▪ Spinal Muscular Atrophy Type I
		▪ Hepatitis C

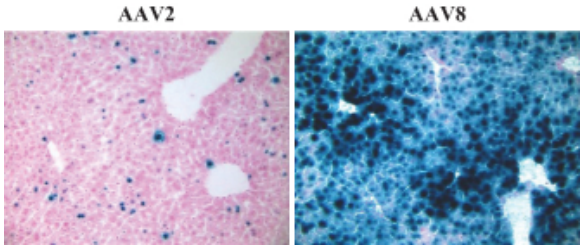
Key Potential Benefits of NAV Technology

The properties that make NAV Vectors unique from and potentially an improvement to earlier generation AAV vectors, as well as provide support that they are potentially best-in-class for development and commercialization of AAV treatments, are set forth in the pages that follow.

Higher Gene Expression

NAV Vectors have been shown to generate higher levels of gene expression in animals than earlier generation AAV vectors such as AAV2. In mice livers, one of our NAV Vectors, AAV8, produced levels of gene expression that were 10- to 100-fold higher than was achieved with AAV2. The figure below shows the contrast in the amount of gene expressed using the two vectors.

AAV Transduction in Mouse Liver



In this experiment, the reporter gene LacZ, a gene which encodes a protein that turns a clear substrate blue in a specific medium, was included in the transgene sequence delivered by the vector so that cells expressing the transgene are stained blue, visually denoting expression level. It was possible to transduce the entire mouse liver and achieve long-term expression with AAV8. Higher gene expression creates the possibility of achieving therapeutic benefit in more diseases than was possible using earlier AAV vectors, as more therapeutic protein is generated with vectors that enable higher expression.

Longer-Term Gene Expression

We believe the longer-term gene expression seen using NAV Vectors is due to more stable genomic persistence and reduced cellular immunity, which are a function of novel capsid structure and lower dosing required using NAV Vectors due to the greater gene expression discussed earlier. NAV Vectors have demonstrated stable expression in animals for over eight years. Moreover, AAV8 vectors have demonstrated stable expression for over four years in clinical trials for hemophilia B patients.

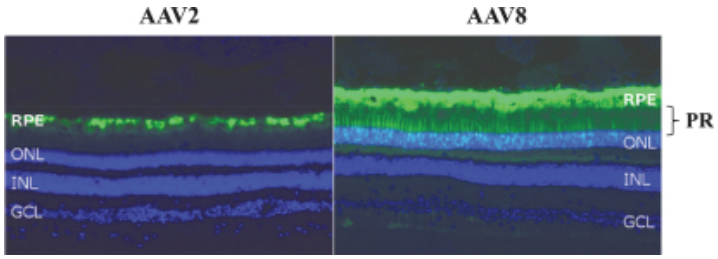
Broad and Novel Tissue Selectivity

NAV Vectors also display high levels of tissue specificity. This property is important because it allows for development of therapeutics to target cells that earlier generation AAV vectors do not target or do not target well. In the CNS, AAV9 has emerged as a vector that enables efficient gene delivery when directly injected into the brain. This was aided by the ability of AAV9 to be transported throughout the brain, enabling broader delivery with a single injection.

NAV Gene Therapy has demonstrated novel tissue selectivity for the CNS when delivered intravenously. Intravenous delivery of AAV9 resulted in efficient gene expression in the brain and spinal cord, and this route of administration produced results in both small and large animals, including non-human primates. This was the first time a gene therapy vector was demonstrated to cross the blood-brain barrier. This route of administration has recently been used clinically by one of our NAV Technology Licensees to treat SMA.

NAV Vectors have also shown novel properties in the eye when investigated for the treatment of acquired disease and inherited retinal degenerations. AAV8 expressing a fluorescent protein was administered by subretinal injection in the non-human primate eye in order to show gene expression in the retina itself, which contains the cell types to be treated. As is depicted in the graphic below, a cross-section of the non-human primate retina below showed more efficient gene delivery (as demonstrated by the much greater amount of the fluorescent protein expressed) with AAV8 as compared to AAV2 in the retinal pigment epithelium (RPE) and to the photoreceptor (PR) layer. The majority of genes associated with retinal degeneration are located in the RPE and PR layer. These genes influence the cell's development or function and are therefore critical to most inherited retinal degenerations.

AAV Transduction of Layers in the Non-Human Primate Eye(1)



(1) Science Translational Medicine: *Dosage Thresholds for AAV2 and AAV8 Photoreceptor Gene Therapy in Monkey*, Luk H. Vandenberghe, et al. (2011). Reprinted with permission from the American Association for the Advancement of Science.

Lower Immune Response

Lower immune response to the gene therapy vector used to deliver the transgene is important for longer-term gene expression, higher expression and higher potency. Data indicate that more than 50% of certain human populations have a high level of neutralizing antibodies (NAbs) for the earlier generation vector AAV2. This represents a major obstacle to the effective use of these earlier generation AAV vectors due to the inhibition of gene delivery via particle neutralization in circulation, meaning pre-existing antibodies neutralize the vector with the transgene before it can reach the target cells. By contrast, frequency of neutralizing antibodies for AAV8 is consistently lower than for AAV2. In a French study, for example, AAV2 NAbs occurred at a frequency of 59% compared to 19% for AAV8. Thus, AAV8 is a candidate for liver-directed gene delivery in a higher proportion of the population than AAV2.

Additionally, reduced effect from the generation and reactivity of T-cells to NAV Vectors has been demonstrated, relative to earlier generation AAV vectors. Activation of T-cells to the capsid of AAV2 vectors has been implicated in liver toxicity in a clinical trial for the treatment of hemophilia B. A patient in this clinical trial developed an elevation of liver enzymes and subsequently lost expression. This led to a hypothesis that capsid protein antigens and memory T-cell activation may lead to clearance of AAV-transduced cells. To further investigate this kind of toxicity, scientists reported a study that evaluated T-cell responses to AAV vectors after administration to mice and nonhuman primates. In this study, high levels of T-cells specific to capsids of AAV2 were detected. AAV8, however, did not lead to activation of capsid-specific T-cells. In a more recent clinical trial for the treatment of hemophilia B, using AAV8, there was less of an effect from T-cells generated and reactive with AAV8. We believe this is likely a function of the lower doses that can be used as well as the structure of the vector itself.

Improved Manufacturability

The manufacturing process for NAV Vectors can be designed to reduce the number of difficult processing steps required for the earlier AAV vectors, improving overall yield at larger scale. NAV Vectors are derived from naturally "fit" viruses, which are stable structures that efficiently assemble, in contrast to the earlier generation AAV vectors. During production, NAV Vectors are secreted by AAV producer cells, eliminating the need for lysing (breaking down of the membrane of a cell, often by viral, enzymic or osmotic mechanisms that compromise the cells integrity) of cells, which can complicate purification and impact yield. This is a novel aspect of NAV Vectors that increases yield and efficiency in production.

Our NAV Gene Therapy Product Candidates

We have developed an internal pipeline of product candidates across the therapeutic areas of metabolic, neurodegenerative and retinal diseases. Below is a table summarizing our current internal development programs.

INTERNALLY DEVELOPED PRODUCT CANDIDATES

Indication	Development Stage: Research	Development Stage: Preclinical	Development Stage: Clinical	Regulatory / Clinical Status
Metabolic Diseases				
Homozygous Familial Hypercholesterolemia (HoFH)	RGX-501			Phase I/II initiation anticipated 1H 2016
Neurodegenerative Diseases				
Mucopolysaccharidosis Type I (MPS I)	RGX-111			Phase I/II initiation anticipated 1H 2016
Mucopolysaccharidosis Type II (MPS II)	RGX-121			
Retinal Diseases				
Wet Age-related Macular Degeneration (wet AMD)	RGX-314			IND anticipated 2H 2016
X-linked Retinitis Pigmentosa (XLRP)	RGX-321			

Metabolic Diseases

Our product development pipeline includes treatment candidates for liver-targeted expression of genes. The selected candidates for our programs seek to leverage lessons learned from previous reports of preclinical and human proof-of-concept studies conducted by third-party investigators and our partners using our NAV Technology Platform. Based on these studies and our own research, we believe our NAV Technology Platform demonstrates promising properties for applications that involve gene delivery to liver cells that may result in long-term, high-level expression of protein.

Historically, a clinical trial for the treatment of hemophilia B using AAV2 vectors that were administered to achieve expression of genes in the liver did not produce evidence of efficacy. Reported data from this study generally did not show any measureable levels of expression sufficient to correct disease symptoms. In subjects where measureable expression levels were reported, gene expression faded over a short period of time. We believe selecting different AAV vectors will increase the levels and duration of expression.

The first clinical milestone of AAV-mediated liver gene therapy occurred in 2011 in the trial described previously for the treatment of hemophilia B using AAV8 in which some patients were able to discontinue prophylactic FIX injections. In 2014, the same group reported in a study update that the treatment was shown to

be durable for over four years and that long-lasting efficacy results were reported in the patients treated. Subsequently, two additional groups have reported human proof-of-concept using AAV8-mediated gene therapy to deliver and express a gene in the liver for the treatment of hemophilia B.

Recently, our academic collaborators demonstrated in a MPS I feline model that liver directed a-l-iduronidase (IDUA) gene delivery using AAV8 resulted in persistent, normal levels of IDUA in the blood. In most cases, the treatment also resulted in cross-correction (cells that are transduced with vector can release enzyme, which is taken up by non-transduced cells) in most tissues including complete resolution of disease pathology in some tissues normally not responsive to enzyme replacement therapy (ERT).

We intend to advance a pipeline of programs in certain metabolic diseases that will be enhanced by the benefits of NAV-mediated liver gene therapy. Our initial focus will be on a severe lipid disorder, HoFH.

RGX-501 for the Treatment of HoFH Caused by LDLR Mutations

Overview of HoFH

HoFH is a monogenic disorder caused by abnormalities in the function or expression of the low-density lipoprotein receptor (LDLR) gene. LDLR plays an important role in the regulation of cholesterol by facilitating uptake and degradation of low-density lipoprotein (LDL) in the liver. LDL is the primary carrier of cholesterol in the blood and has been implicated in the development of plaque buildup in the arteries. HoFH patients have very low levels or are completely deficient of LDLR, resulting in very high total blood cholesterol levels which are typically greater than 500 milligrams per deciliter (mg/dl). This leads to premature and aggressive plaque buildup, life threatening coronary artery disease (CAD) and aortic valve disease. Over time, patients with HoFH develop atherosclerosis, or narrowing and blockage of the arteries, which leads to a high incidence of heart attacks in children and teenagers, among other severe symptoms. If untreated, HoFH patients usually die of causes related to CAD or aortic valve disease before the age of 30.

Recently published medical literature suggests that the worldwide prevalence of HoFH is estimated to be as high as 1 in 200,000, which would correspond to approximately 35,000 individuals, based on worldwide population figures. Multiple studies have compared HoFH patients based on LDLR activity and have shown small differences in residual activity can lead to significant reductions in cholesterol levels and better long term outcomes.

Current Therapies for HoFH

The current standard of care in HoFH focuses on early initiation of aggressive treatment because of the severe clinical effects of elevated LDL-C. Unfortunately, available treatment options are limited. Lipoprotein apheresis, a physical method of purging the plasma of LDL-C, requires weekly or biweekly treatment in order to maintain effect. The procedure is laborious, requiring frequent intravenous access that can be challenging, expensive and not readily available. Other available treatments include statins, a class of pharmaceuticals commonly used to lower cholesterol levels, cholesterol absorption inhibitors and other cholesterol lowering medications. Recently, two new drugs have been approved by the United States Food and Drug Administration (the FDA) as add-on therapy specifically for HoFH: lomitapide and mipomersen. Both result in a reduction of LDL-C, but their use is associated with an array of adverse events that may affect tolerance and long term adherence. These therapies do not provide a cure for the disease and their use is limited due to tolerability and drug availability. Despite the implementation of an aggressive multi-drug therapy approach, the LDL-C levels of HoFH patients remain elevated and mean life expectancy remains at approximately 32 years. With all current therapies, even in combination, providing sub-optimal treatment for patients, a better solution is needed. We believe HoFH is a promising target for gene therapy.

In July 2015, the European Commission and the FDA approved Repatha (Amgen) and Praluent (Sanofi-Aventis), respectively, for the treatment of high cholesterol. The European Commission also approved Repatha

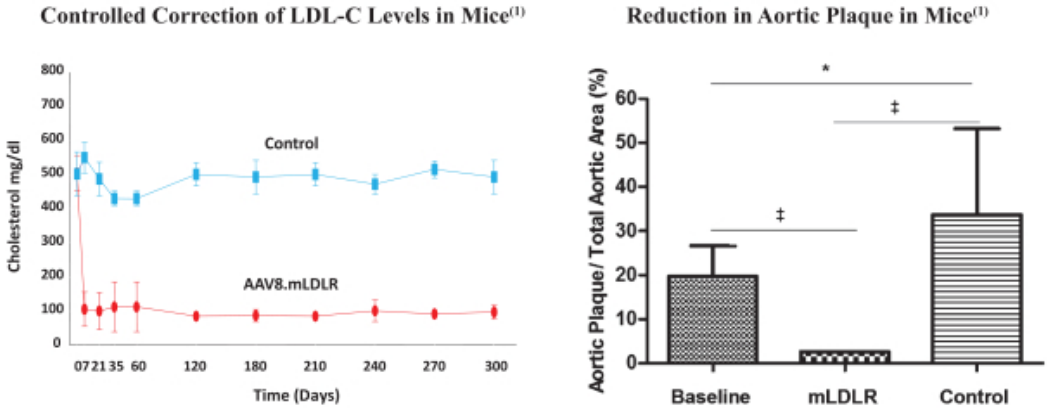
for the treatment of HoFH, among other indications. Repatha and Praluent represent the first drug approvals in a new class of drug called PCSK9 inhibitors. PCSK9 inhibitors are designed to bind to a protein called PCSK9 and inhibit PCSK9 from binding to LDLR on the liver surface. In the absence of PCSK9, there is more LDLR on the surface of the liver to remove LDL-C from the blood. We believe that the emergence of PCSK9 inhibitors as therapy will increase the opportunity and awareness for the profile of RGX-501 by helping to identify more patients who may benefit from its product profile. A clinical trial evaluating a PCSK9 inhibitor demonstrated that its effectiveness relies on patients having functional LDLR. We believe that a substantial unmet medical need remains for the population of HoFH patients who are LDLR negative or severely deficient in LDLR function. We believe that RGX-501, by restoring or increasing LDLR function, may enhance the impact of PCSK9 inhibitors in the treatment of many patients with high cholesterol and as prescribers explore combination therapies.

RGX-501

RGX-501 is our product candidate for the treatment of HoFH, which uses the AAV8 vector to deliver the human LDLR gene to liver cells. We believe that the liver is the preferred target organ for gene therapy of HoFH since LDLRs produced in the liver contribute to greater than 90% of the capture and breakdown of LDL, making the liver by far the most important LDLR producing organ. Additionally, the liver is also the only organ capable of excreting cholesterol from the body, a function that is critical to the maintenance of cholesterol balance. Finally, studies have shown that liver transplantation in HoFH patients corrects the disease, providing strong support that correction of hepatic LDL receptor activity by gene therapy is sufficient for metabolic correction of the disease.

Preclinical Proof of Concept for RGX-501

In order to evaluate the potential for RGX-501 for the treatment of HoFH, mouse LDLR liver-directed gene therapy with AAV8 was evaluated in mouse models of HoFH by our scientific collaborators at Penn. Mice were injected intravenously with the vector and followed for metabolic correction and reversal of pre-existing atherosclerotic lesions. Animals were also evaluated for gross clinical toxicity and abnormalities in serum transaminases, an indicator of liver damage. Animals in the Penn study receiving the vector showed a near complete normalization of hypercholesterolemia that remained stable for almost a year, as well as a substantial regression of atherosclerosis over two months as assessed by two independent methods of quantification at two different sites within the aorta. There was no vector induced toxicity of the liver based on histopathology and clinical chemistry.



(1) PLOS One: *Gene Therapy in a Humanized Mouse Model of Familial Hypercholesterolemia Leads to Marked Regression of Atherosclerosis*, Sadik H. Kassim and Hui Li, et al. (October 2010).

Planned Clinical Development of RGX-501

With our development partners at Penn, we intend to file an IND in the second half of 2015 to support the initiation of a dose-escalation Phase I/II clinical trial of intravenously administered RGX-501 in the United States in patients with HoFH. The design is expected to be a single ascending dose design with a formal safety assessment of the lower dose group prior to dose escalation. The trial design is expected to call for enrollment of approximately 10 subjects and is intended to be a single center study. The primary endpoint will be a safety assessment. The secondary endpoints will likely be biomarkers (e.g., LDL-C) and other outcome measures. Based on previous clinical trials and recent approvals in HoFH, we believe reduction in LDL-C is an endpoint that is an acceptable measure on which regulatory approval could be based.

We had a Pre-Pre-IND meeting on RGX-501 in August 2010 and a Pre-IND meeting on RGX-501 in November 2010. The FDA made pre-clinical, Chemistry, Manufacturing and Controls (CMC) and protocol recommendations at these meetings which have been incorporated into our product development and will be reflected in the RGX-501 IND. We also requested input and received agreement from the FDA on proposed testing of CMC assays for RGX-501 in May and June of 2015, respectively.

The United States National Institutes of Health (NIH) Office of Biotechnology Activities' Recombinant DNA Advisory Committee (the RAC) reviewed the draft protocol for our HoFH Phase I/II clinical trial which we submitted in January 2012. In March 2012, we presented the protocol to the RAC and received subsequent communication from the RAC in March 2012 endorsing the protocol with comments. We are incorporating the RAC's recommendations into the final protocol. Results from this Phase I/II clinical trial will guide us in finalizing the design of a pivotal Phase III clinical trial. If successful, we believe the results of this Phase III clinical trial could support submission of a Biologics License Application (BLA) to the FDA in the United States and a Marketing Authorization Application (MAA) to the EMA in Europe for RGX-501.

We have received orphan drug product designation from the FDA for RGX-501.

Neurodegenerative Diseases

We are focused on developing NAV Gene Therapy for treatments for diseases with significant unmet medical need that involve neurodegeneration in the brain and spinal cord—which together comprise the CNS. We believe our NAV Technology Platform has optimal features for gene delivery to the CNS. In addition, our programs involve novel strategies for improved delivery of NAV Gene Therapy treatments to the CNS that enhance our candidate profiles.

For neurodegenerative disease, AAV2 vectors were historically applied via focal delivery in the brain by adopting existing direct injection techniques. In certain cases, investigators have attempted to use direct injection of vector into multiple sites of the brain to address neurodegenerative disorders that require gene delivery to larger areas. Although there are some examples in animal models in which focal delivery can be therapeutic, these techniques have not produced efficacy in humans.

For most neurodegenerative diseases, we believe that global delivery to the CNS will achieve optimal therapeutic efficacy. Widespread transduction of the CNS in animal models has been achieved by administration of NAV Vectors into the ventricles, cisterna magna, as well as lumbar puncture, which allows the vector to circulate through the cerebrospinal fluid (CSF). We are progressing similar delivery approaches through the CSF in humans to achieve global delivery to the CNS.

Additionally, one of our NAV Vectors, AAV9, has produced early evidence of potentially unique and beneficial properties for gene delivery in the CNS by having the ability to cross the blood-brain barrier. As a result, treatments may be delivered via intravenous injection to target the CNS. One of our NAV Technology Licensees is currently using this approach in a clinical trial for the treatment of a neurodegenerative disease called SMA.

Based on these studies and our own research, we believe our NAV Technology Platform demonstrates promising properties for applications that involve gene delivery to the CNS that we believe will result in long-term, high-level expression of protein. We intend to advance a pipeline of programs in neurodegenerative diseases that will be enhanced by the benefits of using our NAV Technology Platform.

RGX-111 for the Treatment of MPS I Caused by Autosomal Recessive IDUA Mutations

Overview of MPS I

MPS I is a rare autosomal recessive, or non-sex-linked, genetic disease caused by deficiency of IDUA, an enzyme required for the breakdown of polysaccharides heparan sulfate and dermatan sulfate in lysosomes, which are intracellular structures that dispose of waste products inside cells. These polysaccharides, called glycosaminoglycans (GAGs), accumulate in tissues of MPS I patients, resulting in characteristic storage lesions and diverse clinical signs and symptoms. MPS I patients may exhibit short stature, bone and joint deformities, coarsened facial features, enlargement of both the liver and spleen (hepatosplenomegaly), cardiac valve disease, obstructive sleep apnea, recurrent upper respiratory infections, hearing impairment, carpal tunnel syndrome and vision impairment due to corneal clouding. In addition, many patients develop symptoms related to GAG storage in the CNS, which can include excessive accumulation of fluid in the brain, spinal cord compression and cognitive impairment. MPS I patients span a broad spectrum of disease severity and extent of CNS involvement. The severe form of MPS I is also referred to as Hurler syndrome. Hurler patients typically present with symptoms before two years of age and universally exhibit severe cognitive decline after an initial period of normal development.

MPS I is estimated to occur in 1 in 100,000 births, an important metric when considering disease prevalence because severe MPS I patients experience a short life span. Based on global population, this equates to over 1,000 MPS I patients born each year worldwide. Studies suggest that severe forms of MPS I represent between one-half and two-thirds of all MPS I patients.

Current Therapies for MPS I

The first disease modifying therapy developed for severe MPS I was bone marrow transplant (BMT). Though BMT has demonstrated improvements in survival, growth, cardiac and respiratory function, mobility and intellect, it is also associated with substantial morbidity and an estimated 15% to 25% mortality. Accordingly, the procedure is reserved for patients with severe disease before two years of age because the risk-benefit ratio is thought to be more favorable in younger patients who have not yet experienced advanced cognitive decline. Another critical limitation of BMT is that cognitive decline continues for up to a year after transplant before stabilizing, leaving permanent cognitive deficits. In an effort to find approaches that treat the CNS manifestations of neurodegenerative diseases, clinical trials to evaluate direct administration of ERT into the spinal fluid (intrathecal administration) for the treatment of MPS I and direct administration of ERT into the brain (intracerebroventricular administration) for Batten's Disease (a neurodegenerative disease) have been initiated. These approaches, however, do not address the underlying cause of these neurodegenerative diseases. Furthermore, we believe the need for frequent (bi-weekly or monthly) intrathecal or intracerebroventricular administration is likely to lead to patient compliance issues, further reducing the treatment potential of this method of ERT.

More recently, a recombinant form of human IDUA (Aldurazyme) has been approved for the treatment of MPS I. Given as a weekly intravenous infusion, this ERT has demonstrated improvement in hepatosplenomegaly, growth, mobility and respiratory function. However, as the enzyme cannot cross the blood-brain barrier, ERT does not treat the CNS manifestations of MPS I.

Overall, the limitations of BMT and ERT leave a significant unmet need for a method to safely achieve long term IDUA reconstitution in the CNS.

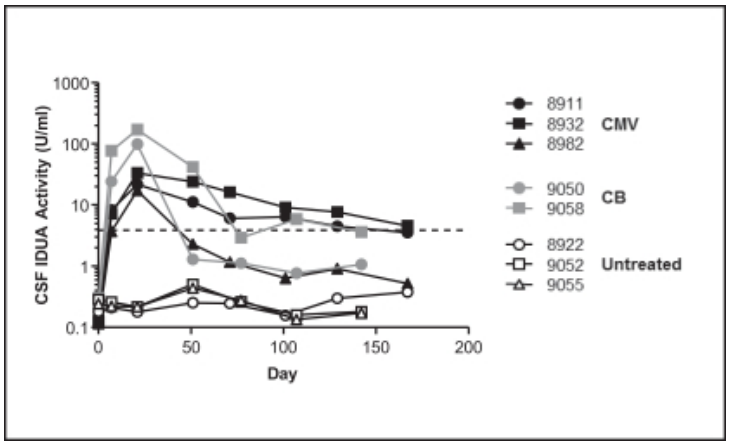
RGX-111

RGX-111 is our product candidate for the treatment of MPS I which uses the AAV9 vector to deliver the human IDUA gene to the CNS. Delivery of the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDUA beyond the blood-brain barrier, allowing for long term cross-correction of cells throughout the CNS. This strategy could also provide rapid IDUA delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occurs in Hurler patients following BMT.

Preclinical Proof of Concept of RGX-111

To assess the feasibility of achieving widespread IDUA expression and correction of storage pathology throughout the brain of MPS I patients, we carried out proof-of-concept studies of intrathecal AAV9 delivery of IDUA using large animal models of MPS I. These studies demonstrated that AAV9 delivery can safely restore IDUA expression to levels equivalent to or greater than non-affected animals. As can be seen in the diagram below, animals treated with an intracisternal injection of an AAV9 vector expressing feline IDUA from a CB promoter (gray symbols) or CMV promoter (black symbols) showed IDUA expression levels above those of untreated animals and in some cases above those of wild-type animals (the dotted line represents mean CSF IDUA expression for two wild-type animals). Storage correction was observed throughout the CNS. Some animals had IDUA activity at lower levels than wild-type animals post-treatment but also achieved significant correction relative to diseased animals. The extent of CNS correction in our studies was substantially greater than that observed in a previous study of MPS I cats treated with BMT at similar ages, thus demonstrating that gene delivery can achieve rapid onset and high levels of IDUA delivery. These findings provide proof of concept of AAV9 delivery of IDUA for treating the CNS pathology associated with MPS I.

IDUA Expression in Feline CSF Following IT AAV9 Delivery(1)



(1) Molecular Therapy: *Intrathecal gene therapy corrects CNS pathology in a feline model of mucopolysaccharidosis I*, Peter Bell, et al. (July 2014).

Planned Clinical Development of RGX-111

We intend to file an IND in the first half of 2016 to support the initiation of an early phase dose-escalation clinical trial of RGX-111 based gene delivery via CNS administration in subjects with MPS I. The Phase I/II clinical trial currently being considered is expected to be a single ascending dose design with a formal safety

assessment of the lower dose group prior to dose escalation. The trial design is expected to call for enrollment of approximately 10 adult subjects. The primary endpoint will be a safety assessment. The secondary and exploratory endpoints will be evaluation of biomarkers and clinical outcomes.

The RAC conducted an initial review of the draft protocol for our MPS I clinical trial, which we submitted in July 2015, and has requested that we present the protocol to the RAC in September 2015.

We submitted a request for orphan drug product designation from the FDA for RGX-111 in July 2015.

RGX-121 for the Treatment of MPS II Caused by X-Linked Recessive IDS Mutations

Overview of MPS II

MPS II, also known as Hunter syndrome, is a rare, X-linked recessive, or sex-linked, disease caused by a deficiency in the lysosomal enzyme iduronate-2-sulfatase (IDS). IDS is another enzyme responsible for the breakdown of polysaccharides heparan sulfate and dermatan sulfate in the lysosomes of cells resulting in a progressive, multisystem disorder with a similar phenotype to MPS I. In severe forms of the disease, early developmental milestones may be met, but developmental delay is readily apparent by 18 to 24 months. Developmental progression begins to plateau between three and five years of age, with regression reported to begin around six and a half years. By the time of death, most patients with CNS involvement are severely mentally handicapped and require constant care.

MPS II is estimated to occur in approximately 1 in 200,000 births. Based on global population, this equates to approximately 500 to 1,000 MPS II patients born each year worldwide.

Current Therapies for MPS II

In 2006, recombinant IDS (Elaprase), an ERT, was approved by the FDA for the treatment of Hunter syndrome and has subsequently been approved for use internationally. ERT in MPS II patients is not expected to result in improvement of CNS dysfunction since IDS is not expected to cross the blood-brain barrier. Specific treatment to address the neurological manifestations of MPS II and prevent or stabilize cognitive decline remains a significant unmet medical need. Overall, the limitations of ERT leave a significant unmet need for a method to safely achieve long term IDS reconstitution in the CNS.

RGX-121

RGX-121 is our product candidate for the treatment of MPS II, which uses the AAV9 vector to deliver the human IDS gene to the CNS. Delivery of the gene encoding the enzyme that is deficient within cells in the CNS could provide a permanent source of secreted IDS beyond the blood-brain barrier, allowing for long term cross-correction of cells throughout the CNS. We believe this strategy could also provide rapid IDS delivery to the brain, potentially preventing the progression of cognitive deficits that otherwise occur in Hunter syndrome patients.

As noted above, this approach has been successfully used in the treatment of animal models of monogenic CNS diseases. Previously conducted studies of AAV9 directed gene therapy in the CNS with MPS I animal models have shown that AAV9 can successfully be used to achieve wide biodistribution within the CNS, robust expression of transgene product that benefits from cross-correction and overall acceptable safety profile. We believe these studies have validated the use of AAV9 in the development of CNS directed gene therapy products and that by using AAV9 for the development of both RGX-111 and RGX-121, we will be able build upon the learnings and experience generated in our RGX-111 program to rapidly and efficiently focus our development efforts for RGX-121.

Preclinical Development of RGX-121

To assess the feasibility of achieving widespread IDS expression and correction of storage pathology throughout the brain of MPS II patients, we carried out proof-of-concept studies of CNS AAV9 delivery using a mouse model of MPS II. There are no known large animal models of MPS II. MPS II mice were administered

with AAV9 vector encoding a gene for IDS in the CNS, which resulted in higher levels of IDS enzyme activity in the brain. Treated animals showed levels of tissue GAG in the brain and peripheral tissues that were lower than untreated MPS II animals and similar to wild-type littermates. These results show the potential therapeutic benefit of AAV9-mediated IDS gene delivery to the CNS through the CSF to address neurological manifestations of MPS II.

Retinal Diseases

We are developing applications of our NAV Technology Platform to treat inherited and acquired forms of retinal disease that can result in visual loss or complete blindness. The retina is the light-sensitive layer of cells that lines the inside of the eye and sends visual messages to the brain. The effects of retinal diseases are isolated to the eye, which is an ideal target for gene therapy due to its immunoprivileged state, small size and relative physical isolation from the rest of the body. The molecular basis of many retinal diseases is becoming well-understood and many retinal diseases are monogenic diseases whose complementary DNA has already been successfully cloned. Also, diagnosis with many forms of inherited blindness is becoming quicker and simpler, due to improved research and application of technology to characterize the variable, unique patterns of different retinal diseases. We believe our NAV Gene Therapy will have improved profiles for achieving therapeutic efficacy where highly efficient gene delivery to the retina is required.

Third party studies reported early evidence of the safety and efficacy of subretinal injection of AAV2 in clinical trials for a retinal disease called Leber congenital amaurosis type 2 (LCA2). Other programs are studying the safety and efficacy profile of AAV2 to treat neovascularization in wet AMD. For LCA2, retinal function was restored by reconstituting gene function in the retinal pigment epithelium (RPE). However, for most retinal degeneration disorders, photoreceptor cells are the primary cell type involved and have historically been a more difficult cellular target in the retina for AAV gene therapy. We believe our NAV Technology Platform will be more efficient at gene delivery into many retinal cell types, particularly photoreceptor cells, than earlier generation AAV vectors such as AAV2. Data from mice, dogs and non-human primates suggests that, compared to other AAVs, NAV Vectors can safely and more effectively target a diverse set of retinal cells, including RPE cells and photoreceptors, when compared to other AAVs. For instance, in most retinal cells NAV-mediated gene delivery reaches maximal levels of expression much sooner than AAV2-mediated delivery. Furthermore, in the same set of retinal cells, NAV Vectors achieve equivalent expression to AAV2 at a dose that is ten times less. Our NAV Technology Platform has been used successfully in a gene therapy approach in animal models of achromatopsia, LCA2, autosomal recessive retinitis pigmentosa, retinoschisis and wet AMD.

We believe that retinal diseases are an ideal target for NAV Gene Therapy due to early evidence indicating efficiency at achieving gene delivery in a wide-array of cell types in the retina. We believe the first use of our NAV Technology Platform in a clinical trial for retinal diseases could result in robust safety and efficacy data but could also serve as a stepping stone for using NAV Gene Therapy in other human retinal diseases.

RGX-314 for the Treatment of Wet AMD

Overview of Wet AMD

Age-related macular degeneration (AMD) is a disease that results in diminution and eventual loss of central vision due to progressive damage to the macula. A subset of AMD patients have wet AMD which is characterized by loss of vision due to the formation of new blood vessels into space between two layers of cells in the retina. This excess blood vessel formation results in fluid leakage that can result in physical changes in the structure of the retina and changes in vision. As this process becomes more severe, blindness can result from scar formation due to hemorrhaging.

Wet AMD is a leading cause of total and partial vision loss in the United States, Europe and Japan. Wet AMD consists of approximately 10% of all cases of AMD, but accounts for approximately 90% of the vision loss associated with AMD. As indicated by the name, the risk for developing AMD increases with age and we

anticipate the diagnosis rate will continue to increase as the population continues to trend towards an aging population. In the United States, the prevalence of wet AMD is estimated to be nearly 600,000 individuals. Globally, the prevalence of wet AMD may exceed three million individuals based on extrapolations using global population figures. In developed countries, an estimated two-thirds of people with AMD have been diagnosed, of whom about two-thirds are treated.

Current Therapies for Wet AMD

Anti-vascular endothelial growth factor (VEGF) therapies have significantly changed the landscape for treatment of wet AMD. They have quickly become the standard of care due to their ability to either halt or significantly impede the loss of vision in the majority of patients with wet AMD. Currently there are three VEGF inhibitors that are commonly used for the treatment of wet AMD. All of these therapies require repetitive intravitreal injections typically ranging from every four to eight weeks in frequency to maintain efficacy, and patients often experience vision loss with reduced frequency of treatment. Due to a variety of factors, including inconvenience and discomfort associated with frequent injections in the eye, patient compliance is a significant concern with anti-VEGF therapies.

We are aware of multiple gene therapy product candidates currently in development to address the unmet medical need described above for wet AMD by targeting VEGF inhibition using AAV2 as the gene therapy vector. Recently, an ongoing clinical trial using one of these AAV2 gene therapy vectors reported data that indicated there may be patients who benefited with reduced injection frequency. We believe these data may also indicate that some patients may benefit from greater inhibition of VEGF activity and that utilizing NAV Technology could allow us to achieve better VEGF inhibition than our competitors using AAV2 to treat wet AMD.

RGX-314

RGX-314 is our product candidate for the treatment of wet AMD, which acts by neutralizing the activity of VEGF and modifying the pathway for formation of new, leaky blood vessels and retinal fluid accumulation. We plan on delivering RGX-314 subretinally using an AAV8 vector encoding a gene for a monoclonal antibody fragment which binds to VEGF and neutralizes VEGF activity. Ranibizumab is an FDA-approved monoclonal antibody fragment that binds to VEGF and has been extensively shown to be both efficacious and safe in wet AMD patients when delivered repeatedly through intraocular injections.

Planned Development of RGX-314

We intend to initiate IND-enabling studies for RGX-314 in 2015, followed by a planned IND filing in the second half of 2016.

X-linked Retinitis Pigmentosa (XLRP)

Retinitis pigmentosa (RP) is the most common inherited form of blindness, with an estimated 100,000 patients in the United States. XLRP accounts for approximately 10% of RP, with 75% to 80% of XLRP cases due to mutations in the gene for retinitis pigmentosa GTPase regulator (RPGR). Mutations in RPGR are associated with a more severe form of the disease, causing early onset of disease, and a relatively fast progression. No therapies exist for RP beyond vitamin supplementation and sun protection, which may or may not slow disease progression. We currently have a preclinical program in development, RGX-321, for the treatment of XLRP.

License Agreements and Commercial Licenses

Platform Licenses

We have exclusively licensed many of our rights in our NAV Technology Platform from Penn and GlaxoSmithKline LLC (GSK), which together we refer to as our Platform Licenses. We currently use our NAV

Technology Platform to develop treatments for metabolic, neurodegenerative, and retinal diseases. We also sublicense our NAV Technology Platform to third parties in order to develop and bring to market NAV Gene Therapy for a range of severe diseases with significant unmet medical needs outside of our core disease indications and therapeutic areas. For further information regarding our commercial sublicenses, please see "License Agreements and Commercial Licenses—Commercial Licenses to NAV Technology Licensees" located elsewhere in this prospectus.

The Trustees of the University of Pennsylvania. In February 2009, we entered into an exclusive, worldwide license agreement with Penn for patent and other intellectual property rights relating to a gene therapy technology platform based on AAVs discovered at Penn in the laboratory of our Chief Scientific Advisor, James M. Wilson, M.D., Ph.D. This license was amended in September 2014. In February 2009, we also entered into a sponsored research agreement (SRA) with Penn (2009 SRA) under which we funded the nonclinical research of Dr. Wilson relating to AAV gene therapy and obtained an option to acquire an exclusive worldwide license in certain intellectual property created pursuant to such 2009 SRA. In December 2014, we entered into another sponsored research agreement with Penn funding related nonclinical research of Dr. Wilson (2014 SRA). We entered into an additional sponsored research agreement (2013 SRA) with Penn in November 2013 which was funded entirely by our NAV Technology Licensee, Dimension Therapeutics, Inc. (Dimension).

Our license agreement with Penn, as amended, provides us with an exclusive, worldwide license under certain patents and patent applications in order to make, have made, use, import, offer for sale and sell products covered by the claims of the licensed patents and patent applications as well as all patentable inventions (to the extent they are or become available for license) that:

- were discovered by Dr. Wilson or other Penn researchers working under his direct supervision at Penn prior to September 2014;
- are related to the AAV technology platform discovered by Dr. Wilson at Penn prior to February 2009 or pursuant to a sponsored research agreement or subsequent amendment to a sponsored research agreement; and
- are owned by Penn and available for licensing.

Prior to entering into the license agreement with us, Penn had previously entered into two license agreements with third parties with respect to certain of the licensed patents and patent applications. Our license from Penn is subject to those preexisting license grants. With respect to the first third party license granted by Penn, our license is non-exclusive with respect to the patents and patent applications licensed to the third party for so long as that preexisting license grant remains in effect and will become exclusive upon the expiration or termination of that existing license agreement. The pre-existing licenses also include a license agreement Penn entered into with GSK in May 2002 granting a license to certain patents and patent applications, of which we subsequently sublicensed certain rights to from GSK in March 2009. For further information regarding our GSK sublicense, please see "License Agreements and Commercial Agreements—Platform Licenses—GlaxoSmithKline LLC" located elsewhere in this prospectus. Our license agreement with Penn provides that should the rights Penn licensed to GSK ever revert to Penn, such rights shall automatically be included in our license agreement with Penn.

The Penn license agreement, as amended, also provides us with a non-exclusive, worldwide license to use all know-how that:

- was developed by Dr. Wilson, or other Penn researchers working under his direct supervision at Penn; and
    - is related to the AAV technology platform discovered by Dr. Wilson prior to February 2009; or
    - is related to the AAV technology platform discovered by Dr. Wilson at Penn after February 2009 pursuant to the 2009 SRA, the 2013 SRA or subsequent amendment to a sponsored research agreement; and

- is owned by Penn and available for licensing; and
- is necessary or useful for the practice of the licensed patent rights.

Under the terms of the Penn license agreement, we issued equity to Penn now represented by 213,150 shares of our common stock. We are also obligated to pay Penn:

- low- to mid-single digit royalties on net sales of licensed pharmaceutical products sold by us or our affiliates;
- low-single digit to low-double digit royalty percentages of net sales on products intended for research purposes only;
- low- to mid-double digit royalty percentage on royalties received from third parties on net sales of licensed pharmaceutical products by such third parties;
- low-double digit to mid-teen digit percentages of sublicense fees we receive for the licensed intellectual property rights from sublicensees; and
- reimbursements for ongoing patent prosecution and maintenance expenses.

As of June 30, 2015, we have incurred expenses of $1.7 million to Penn under the license agreement. There are no future potential milestones to be paid under the license agreement. Our Penn license agreement, as amended, will terminate on a product-by-product and country-by-country basis on the date each particular licensed product ceases to be covered by at least one valid claim, issued or pending, under the licensed patent rights. We can terminate this license agreement by giving Penn prior written notice. Penn has the right to terminate:

- with notice if we are late in paying money due under the license agreement;
- with notice if we fail to achieve a diligence event on or before the applicable completion date or otherwise breach the license agreement;
- if we or our affiliates experience insolvency; or
- if we commence any action against Penn to declare or render any claim of the licensed patent rights invalid or unenforceable.

Under the current 2014 SRA, we fund research at Penn and pay certain intellectual property legal and filing expenses and receive the rights to the research results, if any. Under the Penn license agreement, as amended, and the 2014 SRA, all patentable inventions conceived, created, or conceived and reduced to practice pursuant to the 2014 SRA, together with patent rights represented by or issuing from the United States patents and patent applications, including provisional patent applications, automatically become exclusively licensed to us and all research results are automatically licensed to us as know-how. Our 2014 SRA with Penn will expire on December 31, 2016. We expect to seek to amend the SRA in order to continue to fund work and receive rights to the results of the research we fund at Penn.

GlaxoSmithKline LLC. In March 2009, we entered into a license agreement with GSK in order to secure the exclusive rights to patents and patent applications covering NAV Technology that GSK had previously licensed from Penn (subject to certain rights retained by GSK and Penn). Under this GSK license agreement, we receive an exclusive, worldwide sublicense under the licensed patent rights to make, have made, use, import, sell and offer for sale products covered by the licensed patent rights anywhere in the world. Our rights under this GSK license agreement are subject to certain rights retained by GSK for the benefit of itself and other third parties, including rights relating to: domain antibodies; RNA interference and antisense drugs; internal research purposes and GSK's discovery research efforts with non-profit organizations and GSK collaborators; AAV8 for the treatment of hemophilia B; AAV9 for the treatment of Muscular Dystrophy, congestive heart failure suffered by Muscular Dystrophy patients and cardiovascular diseases by delivery of certain genes; and non-commercial

research in the areas of Muscular Dystrophy, hemophilia B, congestive heart failure suffered by Muscular Dystrophy patients, and other cardiovascular disease. Under the terms of the license agreement, we issued to GSK 1,085,824 shares of our common stock. We are obligated to pay GSK:

- up to $1.65 million in aggregate milestone payments;
- low- to mid-single digit royalty percentages on net sales of licensed products;
- low- to mid-double digit percentages of any sublicense fees we receive from sublicensees for the licensed intellectual property rights; and
- reimbursements for certain patent prosecution and maintenance expenses.

As of June 30, 2015, we have incurred expenses of $3.3 million to GSK under the license agreement and no milestone payments have been made. Under our GSK license agreement, we are required to use commercially reasonable efforts to develop and commercialize licensed products. Our GSK license agreement will terminate upon the expiration, lapse, abandonment or invalidation of the last licensed claim to expire, lapse, become abandoned or unenforceable in all the countries of the world where the licensed patent rights existed. However, if no patent ever issues from patent rights licensed from GSK, this license agreement will terminate a specified number of years after the first commercial sale of the first licensed product in any country. We may terminate this license agreement for any reason upon a specified number of days' written notice. GSK can terminate this license agreement if:

- we are late in paying GSK any money due under the agreement and do not pay in full within a specified number of days of GSK's written demand;
- we materially breach the agreement and fail to cure within a specified number of days; or
- we file for bankruptcy.

Commercial Licenses to NAV Technology Licensees

We sublicense our NAV Technology Platform to third parties in order to develop and bring to market NAV Gene Therapy for a range of severe diseases with significant unmet medical needs. Sublicensing allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV Gene Therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue. Each sublicense specifies the vector or vectors and disease indication or indications as well as whether the sublicense is exclusive or non-exclusive. In determining whether to sublicense, we first evaluate whether the disease indication is of interest to us in which case we may develop a therapeutic for the disease indication internally using our NAV Technology Platform. If it is not, we consider the size of the potential market and unmet need, competition, licensee development history and licensee's ability to pay in evaluating whether to enter into a license agreement. We have granted nine commercial licenses covering 18 partnered product candidates in development by our NAV Technology Licensees, most under a license to specific NAV Vectors. Our license agreements include upfront fees, annual maintenance fees, milestone fees based on licensee candidate progression, and low-single to low-double digit royalties on sales. Such royalties are subject to customary reductions, such as if the licensee must obtain a license from a third party to avoid infringement of such third party's rights in order to exercise its rights under the license granted by us. We are obligated to make payments to our licensors with respect to the revenues we receive from our licensees for these sublicenses, in accordance with the terms of our agreements with our licensors.

Our NAV Technology Licensees currently have two on-going clinical trials using NAV Vectors. The chart below provides an overview of the development status of the programs of our NAV Technology Licensees.

NAV TECHNOLOGY LICENSEE PRODUCT CANDIDATES

Indication	Development Stage: Research	Development Stage: Preclinical	Development Stage: Clinical	Regulatory / Clinical Status	Commercial Rights
Neurodegenerative Diseases					
Spinal Muscular Atrophy				Phase I	AveXis
Mucopolysaccharidosis Type IIIA (MPS IIIA)		LYS-SAF302		Phase I/II	Lysogene
Mucopolysaccharidosis Type IIIA (MPS IIIA)				Clinical trial anticipated 2H 2015	Esteve
Amyotrophic Lateral Sclerosis (ALS)		VY-SOD101			Voyager
Friedreich's Ataxia - CNS		VY-FXN01			Voyager
Huntington's Disease		VY-HTT01			Voyager
Friedreich's Ataxia - CNS					AAVLife
Hematologic / Liver Diseases					
Hemophilia B		DTX101		Clinical trial anticipated 2H 2015	Dimension
Ornithine Transcarbamylase (OTC) Deficiency		DTX301			Dimension
Hemophilia A					Baxalta
Glycogen Storage Disease Type Ia (GSDIa)	DTX401				Dimension
Hemophilia A	DTX201				Dimension/Bayer
Undisclosed					Dimension
Undisclosed					Dimension
Muscle Diseases					
Pompe Disease		AT002			Audentes
X-linked Myotubular Myopathy		AT001			Audentes
Friedreich's Ataxia - Systemic					AAVLife
Friedreich's Ataxia - Systemic					Voyager

AAVLife. In April 2014, we entered into an exclusive license agreement with AAVLife for the development and commercialization of products to treat Friedreich's Ataxia using NAV Vectors. Under this license agreement, we granted AAVLife an exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell and offer for sale licensed products using AAVrh10 for Friedreich's Ataxia where the vector is administered by any route except directly to the central nervous system (Friedreich's Ataxia Systemic). Under the terms of this license agreement we also granted AAVLife an option (the "commercial option") to obtain a non-exclusive worldwide license to make, have made, use, import, sell and offer for sale licensed products using a single vector for each of Friedreich's Ataxia where the vector is administered directly to the central nervous system (Friedreich's Ataxia CNS) and Friedreich's Ataxia Systemic.

Under the terms of this license agreement, we received or are eligible to receive:

- an initial fee of $600,000 and an additional fee of $300,000 if the commercial option to Friedreich's Ataxia CNS is exercised;
- an annual maintenance fee per disease indication licensed;
- up to $13.85 million in combined milestone fees;
- mid- to high-single digit royalty percentages on net sales of licensed products; and
- mid-single to lower mid-double digit royalty percentages of any sublicense fees AAVLife receives from sublicensees for the licensed intellectual property rights.

AAVLife is obligated to achieve certain development milestones with respect to each licensed disease indication, including the filing of an IND for each licensed disease indication within a specified time period, which AAVLife may extend for additional time for a specified number of extensions upon the payment of a fee.

As of June 30, 2015, we have received $650,000 under the license agreement and have not received any milestone payments. This license agreement expires upon the expiration, lapse, abandonment, or invalidation of the last claim of the licensed intellectual property to expire, lapse, or become abandoned or unenforceable in all the countries of the world. The option to obtain a non-exclusive license for Friedreich's Ataxia CNS expires in April 2016 and the option to obtain a non-exclusive license to Friedreich's Ataxia Systemic expired in April 2015. AAVLife may terminate this license agreement upon six months' prior written notice. We may terminate this license agreement if AAVLife is a specified number of days late in paying money due under the license agreement, or if AAVLife, its affiliates, or any sublicensees become insolvent or, effective immediately, if they commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this licensed agreement for material breach if such breach is not cured within a specified number of days.

Audentes Therapeutics, Inc. In July 2013, we entered into an exclusive license agreement with Audentes Therapeutics, Inc. (Audentes) for the development and commercialization of products to treat X-Linked Myotubular Myopathy (XLMTM) and Pompe disease using AAV8 and AAV9. Under this license agreement, we granted Audentes an exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell, and offer for sale licensed products in the treatment of XLMTM and Pompe disease using AAV8 or AAV9.

Under the terms of this license agreement, we received or are eligible to receive:

- an initial fee of $600,000, half of which was paid in the form of 111,999 shares of Audentes' common stock;
- an annual maintenance fee;
- up to $17.7 million in combined milestone fees, a small portion of which may be paid in the form of shares of Audentes' common stock;
- mid- to high-single digit royalty percentages on net sales of licensed products; and
- mid-single to lower mid-double digit percentages of any sublicense fees Audentes receives from sublicensees for the licensed intellectual property rights.

Audentes is obligated to achieve certain development milestones with respect to each licensed disease indication, including the filing of an IND for the licensed indication within a specified time period, which Audentes may extend for additional time for a specified number of extensions upon the payment of a fee.

As of June 30, 2015, we have received $365,000 in cash payments under the license agreement and have not received any milestone payments. Our license agreement with Audentes will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed intellectual property to expire, lapse or become abandoned or unenforceable in all the countries of the world. Audentes may terminate this license agreement upon prior written notice. We may terminate this license agreement immediately if Audentes or its affiliates become insolvent, if Audentes is late by a specified number of days in paying money due under the license agreement, or, effectively immediately, if Audentes or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement for material breach that is not cured within a specified number of days.

AveXis, Inc. In March 2014, we entered into an exclusive license agreement with AveXis, Inc. (AveXis) for the development and commercialization of products to treat spinal muscular atrophy using AAV9. Under this license agreement, we granted AveXis an exclusive, sublicensable worldwide license under the licensed intellectual property to make, have made, use, import, sell and offer for sale licensed products in the field of spinal muscular atrophy using AAV9.

Under the terms of this license agreement, we received or are eligible to receive:

- an initial fee of $2.0 million;
- an annual maintenance fee;
- up to $12.25 million in milestone fees for all licensed products;
- mid-single to low-double digit royalty percentages on net sales of licensed products; and
- lower mid-double digit percentages of any sublicense fees AveXis receives from sublicensees for the licensed intellectual property rights.

Under the agreement, AveXis is obligated to achieve certain development milestones with respect to the licensed disease indication.

As of June 30, 2015, we have received $2.3 million under the license agreement which includes $250,000 in aggregate milestone payments. Our license agreement with AveXis will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed intellectual property to expire, lapse or become abandoned or unenforceable in all the countries of the world. AveXis may terminate this license agreement upon a specified period of prior written notice. We may terminate this license agreement if AveXis or its affiliates become insolvent, if AveXis is greater than a specified number of days late in paying money due under the license agreement, or, effective immediately, if AveXis, its affiliates, or sublicensees commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement for material breach if such breach is not cured within a specified number of days.

Baxalta US Inc. In November 2010, we entered into a non-exclusive license agreement with Chatham Therapeutics, LLC (Chatham) for the research and development of, and an option to obtain an exclusive worldwide license to commercialize, products to treat hemophilia A using AAV8. In December 2012, Chatham exercised the commercial option. In May 2014, Baxter Healthcare Corporation (Baxter) acquired Chatham and assumed the license agreement. In June 2015, Baxter assigned, transferred and conveyed all of its rights and obligations under the license agreement to Baxalta US Inc. (Baxalta). Under this license agreement, we granted Baxalta an exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell, and offer for sale licensed products in the treatment of hemophilia A using AAV8.

Under the terms of this license agreement, we received or are eligible to receive:

- an initial fee of $100,000;
- an annual maintenance fee in the mid-five digits, until Chatham exercised the commercial option, which required the additional payment of $2.0 million, and increased the annual maintenance fee up to a number in the lower mid-six digits;
- up to $7.5 million in milestone fees per each licensed product in the field;
- single digit royalty percentages on net sales of licensed products; and
- low- to mid-double digit percentages of any sublicense fees Baxalta receives from sublicensees for the licensed intellectual property rights.

As of June 30, 2015, we have received $2.6 million under the license agreement and have not received any milestone payments. Our license agreement with Baxalta will expire upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed intellectual property to expire, lapse, become abandoned or unenforceable. The license granted to Baxalta pursuant to the exercise of the commercial option will become a fully paid-up, non-exclusive, royalty-free license, on a country-by-country basis, upon the expiration, lapse, abandonment or invalidation of the last claim of the licensed intellectual property to expire, lapse, become

abandoned or unenforceable in the applicable country. Baxalta may terminate this license agreement upon prior written notice. We may terminate this agreement if Baxalta is greater than a specified number of days late in paying money due under the license agreement or if Baxalta, its affiliates, or sublicensees commence any action against Penn to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement if the other party becomes insolvent or materially breaches the license agreement and does not cure the breach within a specified number of days.

Dimension Therapeutics, Inc.

2013 License Agreement. In October 2013, we entered into an exclusive license agreement with Dimension which, as amended, granted Dimension the right to develop and commercialize products using our NAV Technology to treat hemophilia A and hemophilia B and an option to include up to two additional indications in the scope of the license, which were to be selected by Dimension on or before April 2015. This license agreement was amended in June 2014 and September 2014. For further information regarding our relationship with Dimension, please see "Certain Relationships and Related Party Transactions—Dimension Therapeutics, Inc." located elsewhere in this prospectus. Dimension selected ornithine transcarbamylase (OTC) deficiency and glycogen storage disease type Ia (GSDIa) as the additional licensed disease indications in September 2014 and January 2015, respectively. Under the license agreement, we granted Dimension an exclusive worldwide license under our NAV Technology to make, have made, use, import, sell, and offer to sell licensed products for the treatment of hemophilia A, hemophilia B, OTC and GSDIa. The rights granted to Dimension under this license are subject to certain terms and conditions, including the exclusion of rights to use AAV8 for the treatment of hemophilia A and hemophilia B, as well as the addition of any intellectual property in the licensed indications resulting from the 2013 SRA.

Under the terms of the agreement, we received or are eligible to receive:

- 10,000 shares of Dimension's common stock;
- an annual maintenance fee per disease indication licensed;
- low- to mid-single digit royalty percentages on net sales of licensed products; and

In addition, Dimension will pay any milestone fees owed by us to GSK or sublicense fees owed by us to Penn or GSK as a result of Dimension's activities under this license agreement.

The royalty payments owed to us by Dimension are subject to reduction if our royalty obligations under the Platform Licenses are reduced under certain circumstances, including if certain competitive products are launched by a third party or if the Platform Licenses are amended.

Dimension is required to develop licensed products in accordance with certain performance milestones, which include the receipt of certain financing and development milestones, and the filing of an IND for each of the two additional disease indications optioned by Dimension. In the event that Dimension fails to meet a particular development performance milestone, Dimension may extend the deadline to achieve such milestone for additional time for a specified number of extensions in exchange for separate payments to us.

As of June 30, 2015, we have received $220,000 in cash payments under the license agreement and have not received any milestone payments. Our license agreement with Dimension will expire upon the expiration, lapse, abandonment, or invalidation of the last claim of the licensed intellectual property to expire, lapse, or become abandoned or unenforceable in all the countries of the world. Upon expiration, Dimension's know-how license will become non-exclusive, perpetual, irrevocable and royalty-free with respect to licensed know-how in the field that we own and will continue with respect to all of our other know-how in the field under our GSK and/or Penn licenses for so long as our rights from these licensors continue. Dimension may terminate this license agreement upon a specified period of written notice. We may terminate the license agreement if Dimension or its affiliates

become insolvent, if Dimension is greater than a specified number of days late in paying money due under the license agreement, or, effective immediately, if Dimension or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement for a material breach that is not cured within a specified number of days.

2015 Option and License Agreement. In March 2015, we entered into an option and license agreement with Dimension that grants Dimension the option to exclusively license NAV Technology for the development and commercialization of products to treat up to four additional disease indications. Under this agreement, we granted Dimension four distinct options to obtain an exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell, and offer for sale licensed products with respect to a single disease indication. Dimension exercised options to exclusively license two disease indications, one in each of May 2015 and August 2015.

When Dimension exercises any or all of the commercial options, we received or are eligible to receive:

- an upfront fee of $1.0 million per commercial option;
- an annual maintenance fee per commercial option;
- up to $36.0 million in milestone fees for all disease indications;
- mid- to high-single digit royalty percentages on net sales of licensed products; and
- mid-single to low-double digit percentages of any sublicense fees Dimension receives from sublicensees for the licensed intellectual property rights.

Dimension is obligated to use diligent efforts to meet certain development and regulatory milestones for each optioned disease indication, which may be extended for additional time for a specified number of extensions upon the payment of an additional sum per licensed indication.

As of June 30, 2015, we have received $1.0 million under the license agreement and have not received any milestone payments. Our option and license agreement with Dimension will expire upon the expiration of the royalty obligations with respect to all licensed products for all licensed indications under all licenses granted under all exercised commercial options. Upon expiration, Dimension’s know-how license will become non-exclusive, perpetual, irrevocable and royalty-free with respect to licensed know-how in the field that we own and will continue with respect to all of our other know-how in the field under our GSK and/or Penn licenses for so long as our rights from these licensors continue. Dimension may terminate this option and license agreement upon a specified period of prior written notice. We may terminate the option and license agreement if Dimension or its controlling affiliates become insolvent, if Dimension is greater than a specified number of days late in paying money due under the option and license agreement, or, effective immediately, if Dimension or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this option and license agreement for a material breach that is not cured within a specified number of days.

Laboratorios Del Dr. Esteve. In March 2014, we entered into a non-exclusive license agreement with Laboratorios Del Dr. Esteve, S.A. (Esteve) for the development and commercialization of products to treat MPS IIIA using AAV9. Under the agreement, we granted Esteve a non-exclusive, sublicensable worldwide license under the licensed intellectual property to develop, make, have made, use, import, sell, and offer for sale licensed products in the MPS IIIA field using AAV9 and a non-exclusive license under the licensed intellectual property to practice the licensed patents for internal research and preclinical development of AAV9 agents, including the right to make and use research reagents for such internal research purposes, which research license is only sublicensable to the Universidad Autonoma de Barcelona and Esteve’s affiliates.

Under the terms of this license agreement, we received or are eligible to receive:

- an initial fee of $500,000;
- an annual maintenance fee;

- up to $8.5 million in milestone fees per licensed product;
- mid-single digit to low double-digit royalty percentages on net sales of licensed products; and
- low-double digit percentages of any sublicense fees Esteve receives from sublicensees for the licensed intellectual property rights.

As of June 30, 2015, we have received $550,000 under the license agreement and have not received any milestone payments. Our license agreement with Esteve will expire upon the expiration, lapse, abandonment, or invalidation of the last claim of the licensed intellectual property to expire, lapse or become abandoned or unenforceable in all the countries of the world. Esteve may terminate this license agreement upon a specified number of days' prior written notice. We may terminate the license agreement if Esteve, its affiliates, or sublicensees becomes insolvent, if Esteve is more than a specified number of days late in paying money due under the license agreement, or if Esteve or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement for a material breach uncured for more than a specified number of days.

Lysogene Société Anonyme. In December 2013, we entered into an exclusive license agreement with Lysogene Société Anonyme (formerly known as Lysogene Société par Actions Simplifiée) (Lysogene) for the development and commercialization of products to treat MPS IIIA using AAVrh10. Under this license agreement, we granted Lysogene an exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell, and offer for sale licensed products in the field of MPS IIIA using AAVrh10.

Under the terms of the license agreement, we received or are eligible to receive:

- an initial fee of $500,000;
- an annual maintenance fee;
- up to $7.75 million in milestone fees for the first licensed product to achieve the specified milestone events;
- mid-single to high-single digit royalty percentages on net sales of licensed products; and
- mid-teen to low-double digit percentages of any sublicense fees Lysogene receives from sublicensees for the licensed intellectual property rights.

Lysogene is obligated to achieve certain development milestones, including the first treatment in a Phase III clinical trial within a specified time period, which Lysogene may extend for additional time for a specified number of extensions upon the payment of a fee.

As of June 30, 2015, we have received $550,000 under the license agreement and have not received any milestone payments. Our license agreement with Lysogene will expire upon the expiration, lapse, abandonment, or invalidation of the last claim of the licensed intellectual property to expire, lapse, or become abandoned or unenforceable in all the countries of the world. Lysogene may terminate this license agreement upon a specified number of days' prior written notice. We may terminate this license agreement if Lysogene or its affiliates or sublicensees become insolvent, if Lysogene is greater than a specified number of days late in paying money due under the license agreement, or if Lysogene or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement if a material breach remains uncured for greater than a specified number of days.

Voyager Therapeutics, Inc. In May 2014, we entered into a license agreement with Voyager Therapeutics, Inc. (Voyager) for the development and commercialization of gene therapies to treat Amyotrophic Lateral

Sclerosis (ALS), Friedreich's Ataxia CNS, Friedreich's Ataxia Systemic and Huntington's disease (HD). Under this license agreement, we granted Voyager a non-exclusive worldwide license to make, have made and use NAV Technology solely for internal research and pre-clinical development for the identification of specific vectors which could be commercialized pursuant to an option to obtain a non-exclusive worldwide license under the licensed intellectual property to make, have made, use, import, sell, and offer for sale licensed products using a specified vector which can be exercised for each of ALS, Friedreich's Ataxia CNS, Friedreich's Ataxia Systemic, and/or HD indication(s) which we granted to Voyager. The rights granted to Voyager under this option are subject to certain limitations, such as the exclusion of rights to use AAVrh10 for the treatment of Friedreich's Ataxia Systemic.

Under the terms of this license agreement, we received or are eligible to receive:

- an upfront fee of $500,000;
- an annual maintenance fee;
- should Voyager exercise any or all of the commercial options by a specified date, we will receive an upfront fee ranging from $650,000 to $1.45 million and an annual maintenance fee ranging from low-five digits to low-six digits depending on the number of disease indication options exercised;
- up to $5.0 million in milestone fees per disease indication;
- mid- to high-single digit royalty percentages on net sales of licensed products; and
- mid-single digit percentages of any sublicense fees Voyager receives from sublicensees for the licensed intellectual property rights.

Voyager is also entitled to extend the duration of the commercial option by a specified length of time for each disease indication by making a payment to us.

As of June 30, 2015, we have received $500,000 under the license agreement and have not received any milestone payments. Our license agreement with Voyager will expire upon the expiration, lapse, abandonment, or invalidation of the last claim of the licensed intellectual property to expire, lapse, or become abandoned or unenforceable in all the countries of the world. The license agreement will automatically terminate with respect to all unexercised disease indications if Voyager does not exercise all of its commercial options under the agreement within a specified time period after entering into the license agreement, which may be extended. Voyager may terminate this license agreement upon a specified number of days prior written notice. We may terminate the license agreement if Voyager, its affiliates, or sublicensees experience insolvency, if Voyager is more than a specified number of days late in paying money due under the license agreement, or, effective immediately, if Voyager or its affiliates commence any action against us or our licensors to declare or render any claim of the licensed patent rights invalid or unenforceable. Either party may terminate this license agreement for material breach that is not cured within a specified number of days.

Other Licenses

Regents of the University of Minnesota. In November 2014, we entered into a license agreement with Regents of the University of Minnesota (Minnesota) for the exclusive rights to Minnesota's undivided interest in intellectual property jointly owned by Minnesota and us relating to the delivery of AAV vectors to the central nervous system for MPS I and MPS II. Under this Minnesota license agreement, we receive an exclusive license under the licensed patent rights to make, have made, use, offer to sell or sell, offer to lease or lease, import or otherwise offer to dispose or dispose of products covered by the licensed patent rights in all fields of use in any country or territory in which a licensed patent has been issued and is unexpired or a licensed patent application is pending.

Under the terms of the Minnesota license agreement, we are obligated to pay Minnesota:

- an upfront payment of $25,000;
- up to $125,000 in aggregate milestone payments per licensed product;

- low-single digit royalty percentages on net sales of licensed products;
- mid-single to low-double digit percentages of sublicense fees;
- annual maintenance fees; and
- patent-related maintenance expenses and fees.

We are obligated to achieve certain development performance milestones, each of which may be extended upon the payment of specified fees, related to our efforts to develop and commercialize products incorporating the licensed intellectual property.

As of June 30, 2015, we have incurred expenses of $35,102 paid to Minnesota under the license agreement. This license agreement expires when there is no licensed patent or pending patent application in any country. Upon expiration, our license becomes a royalty-free, fully-paid up, perpetual, and irrevocable license. Minnesota may terminate the license agreement if we materially breach or materially fail to perform one or more of our obligations under the license agreement and we have not cured in full within a specified number of days after delivery of notice of default for payment or a specified number of days if the default relates to any other matter. Minnesota may terminate the license agreement if we become bankrupt or if we commence or maintain an action challenging any patent or patent application licensed under the license agreement. We may terminate the agreement if Minnesota materially breaches or materially fails to perform one or more of its duties under this agreement. We may terminate for any reason upon a specified number of days' prior written notice but must pay an early termination fee.

ARIAD Pharmaceuticals, Inc. In November 2010, we entered into a license agreement with ARIAD Pharmaceuticals, Inc. (ARIAD) in order to secure the exclusive rights for certain gene expression regulation technology. Under this ARIAD license agreement, we receive an exclusive worldwide license under the licensed intellectual property to develop, make, have made, use, sell, offer for sale and import licensed products and perform licensed services in the field of human gene therapy and a non-exclusive license to conduct internal research using related technology. In exchange, we granted to ARIAD a non-exclusive, royalty free, worldwide license to certain improvements and inventions based on the licensed intellectual property for any and all uses outside of the field of human gene therapy. We also issued ARIAD 687,139 shares of our common stock. Under the terms of the ARIAD license agreement, we are obligated to pay ARIAD:

- up to $2.3 million in milestone payments;
- low-single digit royalty percentages on net sales of licensed products;
- an additional low- to mid-single digit royalty percentages on net sales of licensed products to reimburse ARIAD for royalty payments payable to ARIAD's licensors;
- low-double digit percentages of royalties received from our sublicensees;
- following achievement of a milestone event, annual maintenance fees to ARIAD for remittance to one of ARIAD's licensors; and
- reimbursement for ongoing patent prosecution and maintenance costs.

As of June 30, 2015, we have made no cash payments to ARIAD under the license agreement.

Our ARIAD license agreement will expire on a country-by-country, licensed product-by-licensed product, licensed service-by-licensed service basis on the later of ten years from the first commercial sale of the applicable licensed product or licensed service in such country or the date when there is no longer any valid claim covering such licensed product or licensed service in such country. Either party may terminate the ARIAD license agreement for material breach, effective a specified number of days after written notice in the event of nonpayment or a specified number of days for any other breach. We may terminate this license agreement upon a specified number of days' notice to ARIAD. Either party may terminate this license agreement if the other party files for bankruptcy.

Process Development and Manufacturing

We believe that we have access to the resources necessary to enable us to successfully commercialize NAV Gene Therapy products following regulatory approval, if any, by developing scalable processes to manufacture such products efficiently and at commercial quantity.

AAV Vector Expertise

We believe that Dr. Wilson's lab at Penn is among the leading centers in the world for the cloning, production and characterization of AAV vectors. Since our inception we have funded the research of Dr. Wilson relating to the development of manufacturing processes and the analytical characterization of NAV Vectors. We believe that our significant investments in process development and characterization at Penn will help us develop a scalable, proprietary manufacturing process for NAV Gene Therapy products.

We have also entered into two agreements with WuXi Apptec, Inc. (WuXi), a leading technology platform company, with expertise in characterization of biologics. In May 2015, we entered into a collaboration agreement with WuXi in order to establish a proprietary production process for our NAV Gene Therapy. The proprietary production process is designed to enable the manufacturing for our, as well as our NAV Technology Licensees', therapeutic programs from clinical trials through commercialization. Under the terms of the collaboration agreement, WuXi will work with us to establish standard processes applicable to our NAV Technology Platform which may be applied for the development, testing and manufacture of our products or those of our NAV Technology Licensees. WuXi will provide us and our NAV Technology Licensees substantially the same access to process development, testing and manufacturing resources to that received by WuXi's key commercial clients. WuXi will provide us with preferred scheduling and performance of services supporting our gene therapy programs and those of our NAV Technology Licensees. The collaboration agreement with WuXi will remain in force unless terminated in accordance with its terms. Either party may terminate the collaboration agreement upon a specified number of days' prior written notice, for a material breach uncured for more than a specified number of days or if the other party becomes insolvent.

We also entered into an agreement with WuXi in April 2015 setting forth the terms and conditions that would govern future work orders with WuXi. Under this agreement, WuXi would carry out services set forth in future work orders as agreed to by the parties. All work product developed as a result of WuXi's performance of the services under these future work orders would be our sole and exclusive property. This agreement will expire on the later of April 2017 or the completion of all services under the last work order executed by the parties prior to April 2017. Either party may terminate this agreement or future work order upon a specified number of days' prior written notice, for a material breach uncured for more than a specified number of days or if the other party becomes insolvent.

As part of our collaboration with WuXi, we have initiated production of NAV Vectors for use in our planned clinical trial for RGX-111 and have been invoiced $755,517 by WuXi as of June 30, 2015.

Proprietary Methods

We have obtained rights to all of the proprietary technology underlying our NAV Technology Platform through our Platform Licenses and our SRAs, under which we have exclusively licensed rights to certain manufacturing-related patents and non-exclusively licensed rights to certain know-how owned or developed by Penn. This intellectual property encompasses areas including scalable AAV production methods, methods of increasing the packaging yield of AAV and methods of purification of AAV vectors.

Through our SRAs with Penn, we have examined several methods of larger-scale manufacturing of AAV which have been optimized to yield high titer and quality vectors. However, further improvements to the efficiency and simplicity of the process remain important to address future needs for commercial applications.

We have paid particular attention to how the scale-up of AAV vector production occurs during downstream processing of the vector. Many production protocols have vector particles purified from a cell lysate, necessitating extensive downstream purification. These methods were largely developed using AAV2 vectors.

Scientists at Penn discovered that in contrast to earlier generation AAV2, most NAV Vectors were released primarily into the medium of production cultures and not retained in the cell. Because this distribution occurs in the absence of cell lysis, the production culture medium represents a relatively pure source of NAV Vectors and a lower level of cellular contaminants reduces the need for complicated purification steps. This method, for which we have licensed from Penn the exclusive patent rights, is high-yielding and versatile for the production of different NAV Vectors.

Other Capabilities

We have prepared and characterized a proprietary HEK293 master cell bank and other components (plasmid DNA banks) required for clinical vector production. Our master cell bank and other components are being used by us and certain of our NAV Technology Licensees for the production of NAV Vectors under cGMP for use in clinical trials that we expect will begin in 2015 and 2016. For example, as part of a European Union grant consortium, we were selected to manage the production of NAV Vectors for use in a clinical trial to be initiated in Italy for the treatment of MPS VI, a severe lysosomal storage disorder, expected to begin in 2016.

Intellectual Property

Our commercial success depends in part on our ability to obtain and maintain proprietary or intellectual property protection for our product candidates, our core technologies, and other know-how, to operate without infringing on the proprietary rights of others and to prevent others from infringing our proprietary or intellectual property rights. We strive to protect and enhance the proprietary technology, inventions, and improvements that are commercially important to the development of our business, including by seeking, maintaining and defending patent rights, whether developed internally or licensed from third parties. We also rely on trade secrets relating to our proprietary technology platform and on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen and maintain our proprietary position in the field of gene therapy. Additionally, we intend to rely on regulatory protection afforded through orphan drug designations, data exclusivity and market exclusivity as well as patent term extensions, where available.

We have exclusively licensed rights relevant to our NAV Technology which includes novel recombinant AAV vectors AAV7, AAV8, AAV9, and AAVrh10, among others. Our licensed patent portfolio includes exclusive rights to more than 100 patents and patent applications worldwide relating to composition of matter patents and/or patent applications for our novel AAV vectors, as well as methods for their manufacture and therapeutic uses. We also possess substantial know-how and trade secrets relating to NAV Technology.

Our patent portfolio includes the following licensed patents and patent applications relating to our novel AAV vectors:

- One issued U.S. patent relating to AAV7 vectors and uses thereof, currently scheduled to expire in 2026, including patent term adjustment;
- One granted European patent relating to AAV7 vectors and uses thereof, currently scheduled to expire in 2022;
- Five issued U.S. patents relating to AAV8 vectors and uses thereof, which are currently scheduled to expire in 2022 to 2026, including patent term adjustment;
- Two pending European patent applications relating to AAV8 vectors - any European patent that issues from these pending patent applications would currently be expected to expire in 2022;

- One issued U.S. patent relating to AAV9 vectors and uses thereof, currently scheduled to expire in 2026, including patent term adjustment;
- One granted European patent relating to AAV9 vectors and uses thereof, currently scheduled to expire in 2024;
- One pending U.S. patent application relating to AAVrh10 vectors - any U.S. patent that issues from this pending patent application is currently scheduled to expire in 2022; and
- One granted European patent relating to AAVrh10 vectors is currently scheduled to expire in 2022.

Our licensed patent portfolio also includes patents and patent applications relating to the following product candidates:

- A U.S. patent relating to RGX-501 that is currently scheduled to expire in 2026, including patent term adjustment; and
- Two International Patent applications filed pursuant to the Patent Cooperation Treaty (PCT) and pending U.S. patent applications relating to RGX-111 and RGX-121 - any U.S. patent and European patent that issues from these pending PCT applications relating to RGX-111 and RGX-121 is currently scheduled to expire in 2034.

Except as indicated above, the anticipated expiration dates referred to above are without regard to potential patent term extension, patent term adjustment or other market exclusivity that may be available to us.

In addition to our licensed patents and patent applications relating to composition of matter protection for novel AAV vectors having AAV7 capsid, AAV8 capsid, AAV9 capsid, and AAVrh10 capsid, our licensed patent portfolio includes composition of matter claims for novel AAV vectors having AAV11 and AAV12 capsids; Rh.1 to Rh.38, Rh.40, Rh.43, Rh.48 to Rh.62, and Rh.64; Cy.1 to Cy.6 capsids; bb.1 and bb.2 capsids; Ch.1 to Ch.4 capsids; hu.1 to hu.4, hu.6, hu.7, hu.9 to hu.25, hu.27 to hu.29, hu.31, hu.32, hu.34, hu.35, hu.37, hu.39 to hu.49, hu.51 to hu.58, hu.60 to hu.64, hu.66, and hu.67 capsids; pi.1 to pi.3 capsids; and AAV vectors that have amino acid sequences that are at least 95% identical to these capsids.

Our licensed patent portfolio also includes exclusive rights to patents and patent applications relating to:

- therapeutic compositions and methods involving the foregoing AAV vectors further comprising certain transgenes that encode therapeutic products, and their use in treating specified diseases;
- specific formulations or methods of delivery of the recombinant AAV vectors of interest for our in-house development programs;
- technology related to engineering AAV therapeutics including recombinant AAV vectors engineered to target conducting airway cells, methods of altering the targeting and cellular uptake efficiency of an AAV viral vector having a capsid containing an AAV9 cell surface binding domain, the design of recombinant AAV viral vectors that confer passive immunization to airborne pathogens (the aforementioned gene therapy systems can include the use of certain gene expression regulation technology; we have exclusively licensed the patents and patent applications relating to this technology);
- methods of detecting an AAV nucleotide sequence useful in diagnostics; and
- methods of manufacture of AAV, including patents and applications directed to scalable AAV production methods; methods of increasing the packaging yield, transduction efficiency, and gene transfer efficiency of an AAV, and methods of purification of viral vectors, such as AAV vectors.

We anticipate that our patent portfolio will continue to expand as a result of our sponsored research agreements with academic institutions, including the 2014 SRA with Penn where all patentable inventions

conceived, created, or conceived and reduced to practice pursuant to the 2014 SRA, together with patent rights represented by or issuing from the United States patents and patent applications (including provisional patent applications) related thereto automatically become exclusively licensed to us under our existing licensing agreement with Penn and all research results are automatically non-exclusively licensed to us as know-how under that existing license agreement. We also anticipate further expansion of our patent portfolio through our commercial licenses to NAV Technology Licensees which grant us non-exclusive, worldwide, royalty-free, perpetual licenses to use and practice, subject to certain limitations, any patentable modifications or improvements developed by our licensees, their affiliates, or sublicensees to any vector that is the subject of a claim within the licensed patents. For further information regarding our commercial sublicenses, please see “License Agreements and Commercial Licenses—Commercial Licenses to NAV Technology Licensees” located elsewhere in this prospectus.

Customers

Our revenue for the fiscal years ended December 31, 2014 and 2013 consisted of license revenue, reagent sales and grant revenue. Three customers, each based in the United States, accounted for approximately 76% of our total revenue for the year ended December 31, 2013. No other customer accounted for more than 10% of revenue in 2013. Two customers, both based in the United States, accounted for approximately 47% of our total revenue for the year ended December 31, 2014. No other customer accounted for more than 10% of revenue in 2014. Future revenue is uncertain and may fluctuate significantly from period to period.

Competition

The biotechnology and pharmaceutical industries, including in the field of gene therapy, are characterized by rapidly advancing technologies, intense competition and a strong emphasis on intellectual property. While we believe that our NAV Technology Platform, strong intellectual property portfolio and scientific expertise in the gene therapy field provide us with competitive advantages, we face potential competition from many different sources, including larger and better-funded pharmaceutical and biotechnology companies, new market entrants and new technologies.

We are aware of several companies focused on developing gene therapies in various disease indications, including Applied Genetic Technologies Corporation, Avalanche Biotechnologies, Inc., BioMarin Pharmaceutical Inc., bluebird bio, Inc., Genzyme Corporation (Genzyme), Sangamo BioSciences, Inc., Spark Therapeutics, Inc. and uniQure N.V. as well as several companies addressing other methods for modifying genes and regulating gene expression. Additionally, we have sublicensed our NAV Technology Platform for developing gene therapies in various disease indications to our NAV Technology Licensees. Not only must we compete with other companies that are focused on gene therapy products using earlier generation AAV technology and other gene therapy platforms, but any products that we may commercialize will have to compete with existing therapies and new therapies that may become available in the future.

There are other organizations working to improve existing therapies or to develop new therapies for our initially selected disease indications. Depending on how successful these efforts are, it is possible they may increase the barriers to adoption and success for our product candidates, if approved. These efforts include the following:

- **HoFH.** There are several companies with marketed products for the treatment of HoFH, including Aegerion (Juxtapid), Genzyme (Kynamro) and Amgen (Repatha, currently approved in Europe).
- **MPS I.** There is one principal competitor with a marketed product for the treatment of MPS I, Sanofi (Aldurazyme).
- **MPS II.** The principal marketed competition for MPS II is a systemic enzyme replacement therapy, Elaprase (idursulfase), which is marketed by Shire.

- **Wet AMD.** Marketed competition for wet AMD largely consists of anti-VEGF therapies developed by Roche/Genentech (Lucentis, Avastin) and Regeneron (Eylea).

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do. Our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance. Our competitors' treatments may be more effective, or more effectively marketed and sold, than any treatment we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our treatments.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from government and other third-party payors.

Government Regulation

In the United States, biological products, including gene therapy products, are subject to regulation under the Federal Food, Drug, and Cosmetic Act (FD&C Act) and the Public Health Service Act (PHS Act) and other federal, state, local and foreign statutes and regulations. Both the FD&C Act and the PHS Act and their corresponding regulations govern, among other things, the testing, manufacturing, safety, efficacy, labeling, packaging, storage, record keeping, distribution, reporting, advertising and other promotional practices involving biological products. Applications to the FDA are required before conducting clinical testing of biological products, and each clinical study protocol for a gene therapy product is reviewed by the FDA and, in some instances, the NIH, through its RAC. FDA approval also must be obtained before marketing of biological products. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources and we may not be able to obtain the required regulatory approvals.

Within the FDA, the CBER regulates gene therapy products. The CBER works closely with the NIH and its RAC, which makes recommendations to the NIH on gene therapy issues and engages in a public discussion of scientific, safety, ethical and societal issues related to proposed and ongoing gene therapy protocols. The FDA and the NIH have published guidance documents with respect to the development and submission of gene therapy protocols. The FDA also has published guidance documents related to, among other things, gene therapy products in general, their preclinical assessment, observing subjects involved in gene therapy studies for delayed adverse events, potency testing, and chemistry, manufacturing and control information in gene therapy INDs.

Ethical, social and legal concerns about gene therapy, genetic testing and genetic research could result in additional regulations restricting or prohibiting the processes we may use. Federal and state agencies, congressional committees and foreign governments have expressed interest in further regulating biotechnology. More restrictive regulations or claims that our products are unsafe or pose a hazard could prevent us from commercializing any products. New government requirements may be established that could delay or prevent regulatory approval of our product candidates under development. It is impossible to predict whether legislative changes will be enacted, regulations, policies or guidance changed, or interpretations by agencies or courts changed, or what the impact of such changes, if any, may be.

U.S. Biological Products Development Process

The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests and animal studies according to good laboratory practice (GLP) and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- submission to the FDA of an IND, which must become effective before human clinical studies may begin;
- performance of adequate and well-controlled human clinical studies according to the FDA's regulations on good clinical practice (GCP) and any additional requirements for the protection of human research subjects and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;
- submission to the FDA of a Biologics License Application (BLA) for marketing approval that includes substantive evidence of safety, purity, and potency from results of nonclinical testing and clinical studies;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with current Good Manufacturing Practices (cGMP), to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity and, if applicable, the FDA's current good tissue practice (GTP), for the use of human cellular and tissue products;
- potential FDA audit of the nonclinical and clinical study sites that generated the data in support of the BLA; and
- FDA review and approval, or licensure, of the BLA. Before testing any biological product candidate, including a gene therapy product, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLP.

Where a gene therapy study is conducted at, or sponsored by, institutions receiving NIH funding for recombinant DNA research, prior to the submission of an IND to the FDA, a protocol and related documentation is submitted to and the study is registered with the NIH Office of Biotechnology Activities (OBA) pursuant to the NIH Guidelines for Research Involving Recombinant DNA Molecules (NIH Guidelines). Compliance with the NIH Guidelines is mandatory for investigators at institutions receiving NIH funds for research involving recombinant DNA, however many companies and other institutions not otherwise subject to the NIH Guidelines voluntarily follow them. The NIH is responsible for convening the RAC, a federal advisory committee, which discusses protocols that raise novel or particularly important scientific, safety or ethical considerations at one of its quarterly public meetings. The OBA will notify the FDA of the RAC's decision regarding the necessity for full public review of a gene therapy protocol. RAC proceedings and reports are posted to the OBA web site and may be accessed by the public.

The clinical study sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. With gene therapy protocols, if the FDA allows

the IND to proceed, but the RAC decides that full public review of the protocol is warranted, the FDA will request at the completion of its IND review that sponsors delay initiation of the protocol until after completion of the RAC review process. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical studies due to safety concerns or non-compliance. If the FDA imposes a clinical hold, studies may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical studies to begin, or that, once begun, issues will not arise that suspend or terminate such studies.

Clinical studies involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the study sponsor's control. Clinical studies are conducted under protocols detailing, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical study will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical studies must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research subjects provide informed consent. Further, each clinical study must be reviewed and approved by an independent institutional review board (IRB) at or servicing each institution at which the clinical study will be conducted. An IRB is charged with protecting the welfare and rights of study participants and considers such items as whether the risks to individuals participating in the clinical studies are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical study subject or his or her legal representative and must monitor the clinical study until completed. Clinical studies also must be reviewed by an institutional biosafety committee (IBC) a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

- Phase I. The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.
- Phase II. The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.
- Phase III. Clinical studies are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling. Post-approval clinical studies, sometimes referred to as Phase IV clinical studies, may be conducted after initial marketing approval. These clinical studies are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up. The FDA recommends that sponsors observe subjects for potential gene therapy-related delayed adverse events for a 15-year period, including a minimum of five years of annual examinations followed by ten years of annual queries, either in person or by questionnaire, of study subjects.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical study investigators. Annual progress reports detailing the results of the clinical studies must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other

studies, tests in laboratory animals or in vitro testing that suggest a significant risk for human subjects, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase I, Phase II and Phase III clinical studies may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human gene therapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the study period, the number of patients the FDA will require to be enrolled in the studies in order to establish the safety, efficacy, purity and potency of human gene therapy products, or that the data generated in these studies will be acceptable to the FDA to support marketing approval. The NIH and the FDA have a publicly accessible database, the Genetic Modification Clinical Research Information System which includes information on gene transfer studies and serves as an electronic tool to facilitate the reporting and analysis of adverse events on these studies.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHS Act emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

U.S. Review and Approval Processes

After the completion of clinical studies of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human studies, information on the manufacture and composition of the product, proposed labeling and other relevant information. In addition, under the Pediatric Research Equity Act (PREA) a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the biological product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of pediatric data or full or partial waivers of pediatric requirements. Unless otherwise required by regulation, PREA does not apply to any biological product for an indication for which orphan designation has been granted. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things,

whether the proposed product is safe and potent, or effective, for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy (REMS) is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS; the FDA will not approve the BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. For a gene therapy product, the FDA also will not approve the product if the manufacturer is not in compliance with the GTP. These are FDA regulations that govern the methods used in, and the facilities and controls used for, the manufacture of human cells, tissues, and cellular and tissue based products (HCT/Ps) which are human cells or tissue intended for implantation, transplant, infusion, or transfer into a human recipient. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require tissue establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical studies were conducted in compliance with IND study requirements and GCP requirements. To assure cGMP, GTP and GCP compliance, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that usually describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a risk management plan, or otherwise limit the scope of any approval. In addition, the FDA may require post marketing clinical studies, sometimes referred to as Phase IV clinical studies, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

One of the performance goals agreed to by the FDA under the PDUFA is to review 90% of standard BLAs in 10 months of the 60-day filing date and 90% of priority BLAs in six months of the 60-day filing date, whereupon a review decision is to be made. The FDA does not always meet its PDUFA goal dates for standard and priority BLAs and its review goals are subject to change from time to time. The review process and the PDUFA goal date may be extended by three months if the FDA requests or the BLA sponsor otherwise provides additional information, or clarification regarding information already provided in the submission, constituting a major amendment to the BLA.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biological product intended to treat a rare disease or condition, which is defined under the FD&C Act as a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making a drug or biological product available in the United States for this type of disease or condition will be recovered from sales of the product. Orphan product designation must be requested before submitting a BLA. After the FDA grants orphan product designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product that has orphan designation subsequently receives the first FDA approval for the disease or condition for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug or biological product for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan exclusivity. Competitors, however, may receive approval of different products for the indication for which the orphan product has exclusivity or obtain approval for the same product but for a different indication for which the orphan product has exclusivity. Orphan product exclusivity also could block the approval of one of our products for seven years if a competitor obtains approval of the same biological product as defined by the FDA or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug or biological product designated as an orphan product receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan product exclusivity. Orphan drug status in the European Union has similar, but not identical, benefits.

Expedited Development and Review Programs

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new drugs and biological products that meet certain criteria. Specifically, new drugs and biological products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening condition and demonstrate the potential to address unmet medical needs for the condition. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. The sponsor of a new drug or biologic may request the FDA to designate the drug or biologic as a Fast Track product at any time during the clinical development of the product. Unique to a Fast Track product, the FDA may consider for review sections of the marketing application on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the application, the FDA agrees to accept sections of the application and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the application.

Any product submitted to the FDA for marketing, including under a Fast Track program, may be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. Under the Breakthrough Therapy program, products intended to treat a serious or life-threatening disease or condition may be eligible for the benefits of the Fast Track program when preliminary clinical evidence demonstrates that such product may have substantial improvement on one or more clinically significant endpoints over existing therapies. Additionally, FDA will seek to ensure the sponsor of a breakthrough therapy product receives timely advice and interactive communications to help the sponsor design and conduct a development program as efficiently as possible. Any product is eligible for priority review if it has the potential to provide safe and effective therapy where no satisfactory alternative therapy exists or a significant improvement in the treatment, diagnosis or prevention of a disease compared to marketed products. The FDA will attempt to direct additional resources to the evaluation of an application for a new drug or biological product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Drug or biological products studied for their safety and effectiveness in treating serious or

life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments may receive accelerated approval, which means that they may be approved on the basis of adequate and well-controlled clinical studies establishing that the product has an effect on a surrogate endpoint that is reasonably likely to predict a clinical benefit, or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform adequate and well-controlled post-marketing clinical studies. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. Fast Track designation, Breakthrough Therapy designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

Post-Approval Requirements

Maintaining substantial compliance with applicable federal, state, and local statutes and regulations requires the expenditure of substantial time and financial resources. Rigorous and extensive FDA regulation of biological products continues after approval, particularly with respect to cGMP. We will rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of any products that we may commercialize. Manufacturers of our products are required to comply with applicable requirements in the GMP regulations, including quality control and quality assurance and maintenance of records and documentation. Other post-approval requirements applicable to biological products, include reporting of cGMP deviations that may affect the identity, potency, purity and overall safety of a distributed product, record-keeping requirements, reporting of adverse effects, reporting updated safety and efficacy information, and complying with electronic record and signature requirements. After a BLA is approved, the product also may be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by the FDA, the manufacturer submits samples of each lot of product to the FDA together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. The FDA also may perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, the FDA conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

We also must comply with the FDA's advertising and promotion requirements, such as those related to direct-to-consumer advertising, the prohibition on promoting products for uses or in patient populations that are not described in the product's approved labeling (known as "off-label use"), industry-sponsored scientific and educational activities, and promotional activities involving the internet. Discovery of previously unknown problems or the failure to comply with the applicable regulatory requirements may result in restrictions on the marketing of a product or withdrawal of the product from the market as well as possible civil or criminal sanctions. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant or manufacturer to administrative or judicial civil or criminal sanctions and adverse publicity. FDA sanctions could include refusal to approve pending applications, withdrawal of an approval, clinical hold, warning or untitled letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, mandated corrective advertising or communications with doctors, debarment, restitution, disgorgement of profits, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

Biological product manufacturers and other entities involved in the manufacture and distribution of approved biological products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA,

including withdrawal of the product from the market. In addition, changes to the manufacturing process or facility generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

U.S. Patent Term Restoration and Marketing Exclusivity

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Amendments. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved biological product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The United States Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant BLA.

A biological product can obtain pediatric market exclusivity in the United States. Pediatric exclusivity, if granted, adds six months to existing exclusivity periods and patent terms. This six-month exclusivity, which runs from the end of other exclusivity protection or patent term, may be granted based on the voluntary completion of a pediatric study in accordance with an FDA-issued "Written Request" for such a study.

The Patient Protection and Affordable Care Act (Affordable Care Act) signed into law on March 23, 2010, includes a subtitle called the Biologics Price Competition and Innovation Act of 2009 which created an abbreviated approval pathway for biological products shown to be similar to, or interchangeable with, an FDA-licensed reference biological product. This amendment to the PHS Act attempts to minimize duplicative testing. Biosimilarity, which requires that there be no clinically meaningful differences between the biological product and the reference product in terms of safety, purity, and potency, can be shown through analytical studies, animal studies, and a clinical study or studies. Interchangeability requires that a product is biosimilar to the reference product and the product must demonstrate that it can be expected to produce the same clinical results as the reference product and, for products administered multiple times, the biologic and the reference biologic may be switched after one has been previously administered without increasing safety risks or risks of diminished efficacy relative to exclusive use of the reference biologic. However, complexities associated with the larger, and often more complex, structure of biological products, as well as the process by which such products are manufactured, pose significant hurdles to implementation that are still being worked out by the FDA.

A reference biologic is granted 12 years of exclusivity from the time of first licensure of the reference product. The first biologic product submitted under the abbreviated approval pathway that is determined to be interchangeable with the reference product has exclusivity against other biologics submitting under the abbreviated approval pathway for the lesser of (i) one year after the first commercial marketing, (ii) 18 months after approval if there is no legal challenge, (iii) 18 months after the resolution in the applicant's favor of a lawsuit challenging the biologics' patents if an application has been submitted, or (iv) 42 months after the application has been approved if a lawsuit is ongoing within the 42-month period.

Additional Regulation

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations. Equivalent laws have been adopted in other countries that impose similar obligations.

Other U.S. Healthcare Laws and Regulations

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and use of pharmaceutical products that are granted marketing approval. Arrangements with third-party payors, existing or potential customers and referral sources are subject to broadly applicable fraud and abuse and other healthcare laws and regulations, and these laws and regulations may constrain the business or financial arrangements and relationships through which manufacturers market, sell and distribute the products for which they obtain marketing approval. Such restrictions under applicable federal and state healthcare laws and regulations include the following:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons from knowingly and willfully soliciting, receiving, offering or paying remuneration, directly or indirectly, in cash or kind, in exchange for, or to induce, either the referral of an individual for, or the purchase, order or recommendation of, any good or service for which payment may be made under federal healthcare programs such as the Medicare and Medicaid programs. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers, on the one hand, and prescribers, purchasers and formulary managers on the other. The PPACA amends the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- the federal False Claims Act (FCA), which prohibits, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid or other third-party payors that are false or fraudulent. Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, also may implicate the FCA:
- federal criminal laws that prohibit executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- the federal Physician Payment Sunshine Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report annually to CMS information related to payments and other transfers of value to physicians, other healthcare providers and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members;
- the Health Insurance Portability and Accountability Act of 1996 (HIPAA) imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, which governs the conduct of certain electronic healthcare transactions and protects the security and privacy of protected health information; and
- state and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to: items or services reimbursed by any third-party payor, including

commercial insurers; state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government or otherwise restrict payments that may be made to healthcare providers and other potential referral sources; state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians and other healthcare providers or marketing expenditures; and state laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Violation of any of the laws described above or any other governmental laws and regulations may result in penalties, including civil and criminal penalties, damages, fines, the curtailment or restructuring of operations, the exclusion from participation in federal and state healthcare programs and imprisonment. Furthermore, efforts to ensure that business activities and business arrangements comply with applicable healthcare laws and regulations can be costly for manufacturers of branded prescription products.

Coverage and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of any products for which we may obtain regulatory approval. In the United States and markets in other countries, sales of any product candidates for which regulatory approval for commercial sale is obtained will depend in part on the availability of coverage and reimbursement from third-party payors. Third-party payors include government authorities, managed care providers, private health insurers and other organizations. The process for determining whether a payor will provide coverage for a drug product may be separate from the process for setting the reimbursement rate that the payor will pay for the drug product. Third-party payors may limit coverage to specific drug products on an approved list, or formulary, which might not include all of FDA-approved drugs for a particular indication. Moreover, a payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved.

Third-party payors are increasingly challenging the price and examining the medical necessity and cost-effectiveness of medical products and services, in addition to their safety and efficacy. New metrics frequently are used as the basis for reimbursement rates, such as average sales price, average manufacturer price and actual acquisition cost. In order to obtain coverage and reimbursement for any product that might be approved for sale, it may be necessary to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the products, in addition to the costs required to obtain regulatory approvals. If third-party payors do not consider a product to be cost-effective compared to other available therapies, they may not cover the product after approval as a benefit under their plans or, if they do, the level of payment may not be sufficient to allow a company to sell its products at a profit. Health Technology Assessment which is intended to take account of medical, social, economic and ethical issues when determining the suitability of a medicinal product for reimbursement has increasingly become an element of the pricing and reimbursement decisions of the competent authorities in European Union Member States.

The United States government, state legislatures and foreign governments have shown significant interest in implementing cost containment programs to limit the growth of government-paid health care costs, including price controls, restrictions on reimbursement and requirements for substitution of generic products for branded prescription drugs. By way of example, the PPACA contains provisions that may reduce the profitability of drug products, including, for example, increasing the minimum rebates owed by manufacturers under the Medicaid Drug Rebate Program, extending the rebate program to individuals enrolled in Medicaid managed care plans, addressing a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected and establishing annual fees based on pharmaceutical companies' share of sales to federal health care programs. Adoption of government controls and measures, and tightening of restrictive policies in jurisdictions with existing controls and measures, could limit payments for pharmaceuticals.

U.S. Foreign Corrupt Practices Act

The United States Foreign Corrupt Practices Act, to which we are subject, prohibits corporations and individuals from engaging in certain activities to obtain or retain business or to influence a person working in an official capacity. It is illegal to pay, offer to pay or authorize the payment of anything of value to any foreign government official, government staff member, political party or political candidate in an attempt to obtain or retain business or to otherwise influence a person working in an official capacity. Equivalent laws have been adopted in other countries that impose similar obligations.

Government Regulation Outside of the United States

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical studies and any commercial sales and distribution of our products. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

Whether or not we obtain FDA approval for a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical studies or marketing of the product in those countries. Many countries outside of the United States have a similar process that requires the submission of a clinical study application much like the IND prior to the commencement of human clinical studies. In the European Union, for example, a Clinical Trial Authorization (CTA) must be submitted to the competent regulatory authorities and the competent Ethics Committees in the European Union Member States in which the clinical trial takes place, much like the FDA and the IRB, respectively. Once the CTA is approved in accordance with the European Union and the European Union Member State requirements, the corresponding clinical study may proceed.

The requirements and process governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational biological product under European Union regulatory systems, we must submit a marketing authorization application. The grant of marketing authorization in the European Union for products containing viable human tissues or cells such as gene therapy medicinal products is governed by Regulation 1394/2007/EC on advanced therapy medicinal products, read in combination with Directive 2001/83/EC of the European Parliament and of the Council, commonly known as the Community code on medicinal products. Regulation 1394/2007/EC lays down specific rules concerning the authorization, supervision and pharmacovigilance of gene therapy medicinal products, somatic cell therapy medicinal products and tissue engineered products. Manufacturers of advanced therapy medicinal products must demonstrate the quality, safety and efficacy of their products to EMA which provides an opinion regarding the application for marketing authorization. The European Commission grants or refuses marketing authorization in light of the opinion delivered by EMA.

Innovative medicinal products are authorized in the European Union on the basis of a full marketing authorization application (as opposed to an application for marketing authorization that relies on data in the marketing authorization dossier for another, previously approved medicinal product). Applications for marketing authorization for innovative medicinal products must contain the results of pharmaceutical tests, pre-clinical tests and clinical trials conducted with the medicinal product for which marketing authorization is sought. Innovative medicinal products for which marketing authorization is granted are entitled to eight years of data exclusivity. During this period, applicants for approval of generics or biosimilars of these innovative products cannot rely on data contained in the marketing authorization dossier submitted for the innovative medicinal product to support their application. Innovative medicinal products for which marketing authorization is granted are also entitled to

ten years of market exclusivity. During these ten years' of market exclusivity, no generic or biosimilar medicinal product may be placed on the European Union market even if a marketing authorization for approval of a generic or biosimilar of the innovative product has been submitted to the EMA or to the competent regulatory authorities in the European Union Member States and marketing authorization has been granted. The ten years of market exclusivity will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. However, there is no guarantee that a product will be considered by the European Union's regulatory authorities to be an innovative medicinal product which is eligible for the relevant periods of data and market exclusivity.

Products authorized as "orphan medicinal products" in the European Union are entitled to benefits additional to those granted in relation to innovative medicinal products. In accordance with Article 3 of Regulation (EC) No. 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, a medicinal product may be designated as an orphan medicinal product if (1) it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition; (2) either (a) such condition affects no more than five in 10,000 persons in the European Union when the application is made, or (b) the product, without the incentives derived from orphan medicinal product status, would not generate sufficient return in the European Union to justify investment; and (3) there exists no satisfactory method of diagnosis, prevention or treatment of such condition authorized for marketing in the European Union, or if such a method exists, the product will be of significant benefit to those affected by the condition. Further guidance on such criteria is provided in Regulation (EC) No. 847/2000 of 27 April 2000 laying down the provisions for implementation of the criteria for designation of a medicinal product as an orphan medicinal product and definitions of the concepts "similar medicinal product" and "clinical superiority". Orphan medicinal products are eligible for financial incentives such as reduction of fees or fee waivers and are, upon grant of a marketing authorization, entitled to ten years of market exclusivity for the approved therapeutic indication. The application for orphan drug designation must be submitted before the application for marketing authorization. The applicant may receive a fee reduction for the marketing authorization application if the orphan drug designation has been granted, but not if the designation is still pending at the time the marketing authorization is submitted. Orphan drug designation does not convey any advantage in, or shorten the duration of, the regulatory review and approval process.

Products authorized in the European Union as orphan medicinal products are entitled to 10 years of data exclusivity. The products are, in parallel, entitled to 10 years of market exclusivity. The 10-year market exclusivity may be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan designation, for example, if the product is sufficiently profitable not to justify maintenance of market exclusivity. Additionally, marketing authorization may be granted to a similar product during the 10-year period of market exclusivity for the same therapeutic indication at any time if:

- The second applicant can establish in its application that its product, although similar to the orphan medicinal product already authorized, is safer, more effective or otherwise clinically superior;
- The holder of the marketing authorization for the original orphan medicinal product consents to a second orphan medicinal product application; or
- The holder of the marketing authorization for the original orphan medicinal product cannot supply enough orphan medicinal product.

For other countries outside of the European Union, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical studies are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

If we fail to comply with applicable foreign regulatory requirements, we may be subject to, among other things, fines, suspension or withdrawal of regulatory approvals, product recalls, seizure of products, operating restrictions and criminal prosecution.

Facilities

Our corporate headquarters are currently located in Rockville, Maryland. We occupy approximately 11,000 square feet of office space in this location under a lease that expires in September 2020, renewable for two additional three-year terms, and which includes a right of first refusal on an additional 19,000 square feet of office, laboratory and manufacturing space adjacent to our current premises which we exercised in August 2015. We currently anticipate occupying the additional space in the fourth quarter of 2015. In addition, we occupy 375 square feet of lab space in Philadelphia, Pennsylvania under a lease that expires at the end of 2015. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

Employees

As of June 30, 2015, we employed 18 full-time employees, including six in research and development and twelve in executive, general and administrative. We also employed one part-time employee in executive, general and administrative as of June 30, 2015. We have never had a work stoppage, and none of our employees is represented by a labor organization or under any collective bargaining arrangements. We consider our relationship with our employees to be good.

Legal Proceedings

We are not a party to any material legal proceedings at this time. From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this prospectus, we do not believe we are party to any claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

MANAGEMENT

Executive Officers, Key Employee, Directors and Key Advisor

Our executive officers, directors and key advisors and employees, and their ages and positions as of June 30, 2015, are set forth below:

Name	Age	Position
Kenneth T. Mills	40	Chief Executive Officer, President and Director
Vittal Vasista	47	Chief Financial Officer, Senior Vice President of Corporate Development and Treasurer
Stephen Yoo, M.D.	37	Chief Medical Officer
Sara Garon Berl, Esq.	39	Vice President, General Counsel and Secretary
James M. Wilson, M.D., Ph.D.	60	Chief Scientific Advisor
Donald J. Hayden, Jr. (1),(2)	59	Chairman of the Board of Directors
Luke M. Beshar (1),(2)	57	Director
Edgar G. Engleman, M.D. (3)	68	Director
Allan M. Fox	67	Director
A.N. “Jerry” Karabelas, Ph.D. (3)	62	Director
Camille Samuels (1),(2)	44	Director

(1) Member of Audit Committee.
(2) Member of Compensation Committee.
(3) Member of Nominating and Corporate Governance Committee.

Executive Officers and Key Employee

Kenneth T. Mills has been our President, Chief Executive Officer and Director since March 2009. Mr. Mills was with FoxKiser, most recently as a partner, from January 2007 to January 2015. Mr. Mills was previously the Chief Financial Officer and Vice President of Business Development at Meso Scale Diagnostics, a privately-held life sciences company from January 2004 to December 2006 and was part of the original management team that established the company’s operations and financing strategy. From March 1997 to December 2003, Mr. Mills was employed at IGEN International, a medical diagnostics company, where he served as Director of Business Development up through the company’s acquisition by Roche. Mr. Mills received an S.B. in Chemistry from the Massachusetts Institute of Technology. We believe that Mr. Mills’ qualifications to serve as a director of our company include his extensive experience as an executive in the gene therapy and biotechnology industries and his prior service as a senior-level executive in both early stage and mature biotechnology companies.

Vittal “Vit” Vasista has been our Chief Financial Officer and Senior Vice President of Corporate Development since August 2009. Prior to joining us, Mr. Vasista served as Principal at PRTM Management Consultants from October 2006 to July 2009, where he developed operational strategies for both private and public organizations, including the development of market entry strategies, innovative business models, and operational improvements. Earlier in his career, Mr. Vasista served as Director, Business Development at Meso Scale Diagnostics, a privately held life sciences company, from June 2002 to May 2006. Mr. Vasista received an M.B.A. from The Wharton School at the University of Pennsylvania, an M.S. in Mechanical Engineering from Stanford University, and an S.B. in Mechanical Engineering from the Massachusetts Institute of Technology.

Stephen Yoo, M.D. has been our Chief Medical Officer since October 2014. Prior to joining us, Dr. Yoo was Medical Science Director and Group Director of Clinical Development at AstraZeneca from January 2014 to October 2014. In these roles, he led the late-phase clinical project teams while providing strategic and operational

leadership to physicians and scientists. In previous roles at MedImmune, LLC, AstraZeneca's global biologics research and development arm, from April 2010 to May 2014, Dr. Yoo provided strategic clinical leadership for early-phase programs. Earlier in his career, Dr. Yoo served as Associate Director of Clinical Development at Abbott Laboratories from June 2008 to April 2010. Dr. Yoo holds an M.D. from the University of California, Los Angeles School of Medicine and a B.A. in Molecular and Cell Biology from the University of California, Berkeley.

Sara Garon Berl, Esq. has been our Vice President, General Counsel and Secretary since June 2015 and was previously our General Counsel and Vice President of Advocacy from September 2014 to June 2015, our Vice President, Legal Affairs and Advocacy Development from July 2014 until September 2014, and our Vice President, Legal Affairs from January 2013 to July 2014. Prior to joining us, Ms. Berl was a principal with FoxKiser LLP from April 2012 to December 2013. Ms. Berl was previously an associate at Covington & Burling from September 2001 to September 2005, where she focused on commercial litigation and arbitration in the areas of health law, intellectual property, securities, and deceptive trade practices. Ms. Berl received a J.D. from Stanford University and an S.B. in Mathematics from the Massachusetts Institute of Technology.

Key Advisor

James M. Wilson, M.D., Ph.D. has been our Chief Scientific Advisor since September 2014. Dr. Wilson is also Professor of Medicine at the University of Pennsylvania where he has served since 1993 and director of the Gene Therapy Program in the Department of Pathology and Laboratory Medicine at the University of Pennsylvania where he has served since 2006. Following his residency in Internal Medicine at the Harvard-affiliated Massachusetts General Hospital from 1985 to 1986 and a postdoctoral fellowship at the Massachusetts Institute of Technology from 1986 to 1988, he returned to the University of Michigan where he took his first faculty position and began his studies in gene therapy, where he served from 1988 to 1993. Dr. Wilson holds an M.D. and Ph.D. from the University of Michigan and a B.S. in Chemistry from Albion College.

Directors

Luke M. Beshar has been a Director since April 2015. Mr. Beshar was the Executive/Senior Vice President and Chief Financial Officer of NPS Pharmaceuticals, Inc., a global biopharmaceutical company from November 2007 to February 2015. He is a former Chief Financial Officer of various public and private companies and has more than 30 years of general and financial management experience. Mr. Beshar served as Executive Vice President and Chief Financial Officer of Cambrex Corporation from December 2002 to November 2007, a global life sciences company, and previously as Senior Vice President and Chief Financial Officer at Dendrite International, a leading provider of services to the life sciences industry. Mr. Beshar began his career with Arthur Andersen & Co. in 1980 and is a Certified Public Accountant. Mr. Beshar is a Director of Trillium Therapeutics, Inc. and Chair of its Audit Committee and a Director of Fluorinov Pharma Inc. Mr. Beshar holds a B.S. degree in Accounting and Finance from Michigan State University and is a graduate of The Executive Program at the Darden Graduate School of Business at the University of Virginia. Mr. Beshar has specific attributes that qualify him to serve as a member of our board of directors, including his experience in the biotechnology and medical industries, his financial and accounting expertise, as well as his prior service on public and private company boards.

Edgar G. Engleman, M.D. has been a Director since May 2015. Dr. Engleman is a founding member and Managing Partner of Vivo Capital, LLC (formerly Vivo Ventures) and since 1990 has served as professor of Pathology and Medicine at Stanford University School of Medicine, where he oversees the Stanford Blood Center as well as his own immunology research group. An editor of numerous scientific journals and the inventor of multiple patented technologies, Dr. Engleman has authored more than 250 publications in medical and scientific journals and has trained more than 200 graduate students and postdoctoral fellows. Dr. Engleman has co-founded a number of biopharmaceutical companies including Cetus Immune Corporation (acquired by Chiron Corporation), Genelabs Technologies, Inc., (acquired by GlaxoSmithKline plc), Dendreon Corporation, Medeor Therapeutics, Inc. and Bolt Biotherapeutics, Inc. He is the lead inventor of the technology underlying Provenge, Dendreon's cancer vaccine, which was approved in 2010 to treat asymptomatic or minimally symptomatic

metastatic hormone refractory prostate cancer. Dr. Engleman currently serves on the boards of several private biotechnology companies, including Eiger BioPharma, Inc., Synapse Biomedical, Inc., Bolt Biotherapeutics, Inc. and Semnur Pharmaceuticals, Inc., and one public company, Capnia Inc. He received his M.D. from Columbia University School of Medicine and his B.A. from Harvard University. Dr. Engleman has specific attributes that qualify him to serve as a member of our board of directors, including his extensive knowledge of the healthcare industry, his medical expertise and his service on other public and private boards of directors.

Allan M. Fox has been a Director since July 2008. Mr. Fox is the founding partner of FoxKiser, a nationally recognized firm committed to the strategic development of transformative innovations from biomedical research, which was formed in September 1986. Mr. Fox specializes in identifying business opportunities and improving competitive market positions. He has participated in the formation and development of numerous ventures in the public and private sectors. Before forming FoxKiser, Mr. Fox co-led the establishment of the Washington office of the law firm of Kaye Scholer. While in the public sector, Mr. Fox served as Chief of Staff and Chief Legislative Assistant to U.S. Senator Jacob K. Javits of New York. He also served as Chief Counsel to the United States Senate Health and Scientific Research Subcommittee, chaired by Senator Edward M. Kennedy. He is the National Board Chair of the Alliance for Aging Research. Mr. Fox was a Fellow in Law, Science and Medicine at Yale Law School where he received an LL.M. degree. Mr. Fox also holds a J.D. and B.A. from Temple University. Mr. Fox has specific attributes that qualify him to serve as a member of our board of directors, including his experience in the biotechnology sector and FDA consulting, as well as his prior service on private company boards.

Donald J. Hayden, Jr. has been a Director and Chairman of our board of directors since February 2013. From 1991 to 2005, Mr. Hayden held several executive positions with Bristol-Myers Squibb Company, most recently serving as Executive Vice President and President, Americas. Mr. Hayden is currently a member and chairman of the board of directors of Insmed Incorporated, Vitae Pharmaceuticals Inc., Alvine Pharmaceuticals, Inc., and Nora Therapeutics Inc. He is also lead independent director at Amicus Therapeutics, Inc., a member of the board of directors at Otsuka America Pharmaceutical, Inc., and serves as a senior advisor to Prospect Venture Partners, a leading life sciences venture capital firm. Mr. Hayden served as a director of Dimension Therapeutics, Inc. from October 2013 to July 2015. Mr. Hayden holds a B.A. from Harvard University and an M.B.A. from Indiana University. Mr. Hayden has specific attributes that qualify him to serve as a member of our board of directors, including his experience in the biotechnology and pharmaceutical industries, as well as his prior service on public and private company boards and his executive-level service at a number of public and private companies.

A.N. "Jerry" Karabelas, Ph.D. has been a Director since May 2015. Since December 2001, Mr. Karabelas has been a managing member at Care Capital II, LLC and Care Capital III, LLC (Care Capital), a provider of capital for entrepreneurial private and public companies developing pharmaceuticals. Prior to his work at Care Capital, from July 2000 to September 2001, Mr. Karabelas was Chairman at Novartis BioVentures, which is owned by Novartis AG (Novartis), a provider of capital for life sciences companies across the biotech, medical devices and diagnostics industries, prior to which Mr. Karabelas was the Chief Executive Officer of Novartis Pharma AG, which is owned by Novartis. In connection with his work at Care Capital, Mr. Karabelas has served on numerous boards of directors of pharmaceutical and therapeutics companies, including Renovo, plc, Vanda Pharmaceuticals, Inc. and NitroMed, Inc. Since June 2013, Mr. Karabelas has served as Chairman of Polyphor AG. Mr. Karabelas also served as a member of the boards of directors of SkyePharma, plc from May 2001 to May 2009 and Human Genome Sciences. Mr. Karabelas received a B.S. from the University of New Hampshire and a Ph.D. from the Massachusetts College of Pharmacy. Mr. Karabelas has specific attributes that qualify him to serve as a member of our board of directors, including his extensive experience in working with publicly held pharmaceuticals companies, advising developing life sciences, therapeutics and pharmaceuticals companies and his executive leadership, managerial and business experience.

Camille Samuels has been a Director since January 2015. Ms. Samuels has been a Partner at Venrock, a venture capital firm, since May 2014. Prior to Venrock, Ms. Samuels spent over a decade as a Managing Director at Versant Ventures, a life sciences venture capital firm, which she joined in 2000 and for which she provided

services through March 2014. Ms. Samuels currently serves on the board of Kythera Biopharmaceuticals, Inc., Spirox Corporation, and Unity Biosciences, Inc. She previously served as a board member or a board observer on other healthcare companies including Achaogen, Inc. (AKAO), Fluidigm Sciences Inc., Genomic Health, Inc., Novacardia, Inc. (acquired by Merck & Co., Inc.), ParAllele BioScience, Inc. (acquired by Affymetrix Inc.), and Syrrx Inc. (acquired by Takeda Pharmaceutical Co.). Prior to her venture career, Ms. Samuels held business development and strategic marketing roles at Tularik Inc. (acquired by Amgen Inc.) and Genzyme Corporation (acquired by Sanofi-Aventis SA). She also worked as a management consultant to consumer, healthcare and biotech companies at LEK Consulting. Ms. Samuels holds a B.A. in Biology from Duke University and an M.B.A. from Harvard Business School. Ms. Samuels has specific attributes that qualify her to serve as a member of our board of directors, including her experience in venture capital investing and in the biotechnology sector, as well as her prior service on public and private company boards and audit committees.

Board of Directors

Our business and affairs are managed under the direction of our board of directors, which is currently composed of seven members. Our current directors were elected pursuant to an amended and restated voting agreement among certain of our preferred and common stock holders. This agreement will terminate upon the closing of this offering, at which time there will be no further contractual obligations regarding the election of our directors.

Independent Directors

We have applied to list our common stock on the NASDAQ Global Market. Under NASDAQ rules, independent directors must comprise a majority of a listed company's board of directors within 12 months from the date of listing. In addition, NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent within 12 months from the date of listing. Audit committee members must also satisfy additional independence criteria, including those set forth in Rule 10A-3 under the Securities Exchange Act of 1934, as amended (the Securities Exchange Act), and compensation committee members must also satisfy additional independence criteria, including those set forth in Rule 10C-1 of the Securities Exchange Act.

In June 2015, our board of directors undertook a review of its composition and that of its committees, as well as the independence of each director who will serve following the consummation of this offering. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations, including family relationships, our board of directors has determined that each of Luke M. Beshar, Edgar G. Engleman, M.D., A.N. "Jerry" Karabelas, Ph.D. and Camille Samuels qualify as independent directors in accordance with the rules of NASDAQ. Our board of directors currently expects that Donald Hayden, Jr. will qualify as an independent director in accordance with the rules of NASDAQ commencing during the fourth quarter of 2016. The independent members of our board of directors will hold separate regularly scheduled executive session meetings at which only independent directors are present.

Classified Board

Immediately after this offering, our board of directors will be divided into three classes with staggered three-year terms. At each annual meeting of stockholders, the successors to directors whose terms then expire will be elected to serve from the time of election and qualification until the third annual meeting following election. Our directors will be divided among the three classes as follows:

- The Class I directors will be Edgar G. Engleman, M.D., Allan M. Fox and Camille Samuels, and their terms will expire at the annual meeting of stockholders to be held in 2016;
- The Class II directors will be Donald J. Hayden Jr. and A.N. "Jerry" Karabelas, Ph.D., and their terms will expire at the annual meeting of stockholders to be held in 2017; and
- The Class III directors will be Luke M. Beshar and Kenneth T. Mills, and their terms will expire at the annual meeting of stockholders to be held in 2018.

Each director's term will continue until the election and qualification of his successor, or his earlier death, resignation, retirement, disqualification or other removal. Any increase or decrease in the number of directors will be distributed among the three classes so that, as nearly as reasonably possible, each class will consist of one-third of our directors.

The authorized number of directors may be changed only by resolution of the board of directors. This classification of the board of directors into three classes with staggered three-year terms may have the effect of delaying or preventing changes in our control or management.

Our directors may be removed only for cause and by the affirmative vote of the holders of two-thirds of our outstanding voting stock.

Board Leadership Structure

Our board of directors is currently led by its chairman, Donald J. Hayden, Jr. Our board of directors recognizes that it is important to determine an optimal board leadership structure to ensure the oversight of management as the company continues to grow. We separate the roles of chief executive officer and chairman of the board in recognition of the differences between the two roles. The chief executive officer is responsible for setting the strategic direction for the company and the day-to-day leadership and performance of the company, while the chairman of the board of directors provides guidance to the chief executive officer and presides over meetings of the full board of directors. We believe that this separation of responsibilities provides a balanced approach to managing the board of directors and overseeing the company.

Our board of directors has concluded that our current leadership structure is appropriate at this time. However, our board of directors will continue to periodically review our leadership structure and may make such changes in the future as it deems appropriate.

Board Oversight of Risk

Our board of directors has responsibility for the oversight of the company's risk management processes and, either as a whole or through its committees, regularly discusses with management our major risk exposures, their potential impact on our business and the steps we take to manage them. The risk oversight process includes our board receiving regular reports from board committees and members of senior management to enable our board to understand the company's risk identification, risk management and risk mitigation strategies with respect to areas of potential material risk, including operations, finance, legal, regulatory, strategic and reputational risk.

The audit committee of our board of directors reviews information regarding liquidity and operations, and oversees our management of financial risks. Periodically, the audit committee reviews our policies with respect to risk assessment, risk management, loss prevention and regulatory compliance. Oversight by the audit committee includes direct communication with our external auditors, and discussions with management regarding significant risk exposures and the actions management has taken to limit, monitor or control such exposures. The compensation committee of our board of directors is responsible for assessing whether any of our compensation policies or programs has the potential to encourage excessive risk-taking. The nominating and corporate governance committee of our board of directors manages risks associated with the independence of the board, corporate disclosure practices, and potential conflicts of interest. While each committee is responsible for evaluating certain risks and overseeing the management of such risks, the entire board is regularly informed through committee reports about such risks. Matters of significant strategic risk are considered by our board as a whole.

Code of Business Conduct

Our board of directors adopted a code of business conduct that applies to each of our directors, officers and employees, including our principal executive officer, principal financial officer, principal accounting officer or controller and persons performing similar functions. The code addresses various topics, including:

- compliance with applicable laws, rules and regulations;
- conflicts of interest;
- public disclosure of information;
- insider trading;
- corporate opportunities;
- competition and fair dealing;
- gifts;
- discrimination, harassment and retaliation;
- health and safety;
- record-keeping;
- confidentiality;
- protection and proper use of company assets;
- payments to government personnel; and
- the reporting of illegal and unethical behavior.

Prior to the completion of this offering, the code of business conduct will be posted on the Investor Relations section of our website, which is located at www.regenxbio.com. Any waiver of the code of business conduct for an executive officer or director may be granted only by our board of directors or a committee thereof and must be timely disclosed as required by applicable law. We intend to disclose future amendments to certain provisions of our code of business conduct, or waivers of those provisions, applicable to any principal executive officer, principal financial officer, principal accounting officer or controller, or persons performing similar functions on our website, www.regenxbio.com.

We have implemented whistleblower procedures that establish formal protocols for receiving and handling complaints from employees. Any concerns regarding accounting or audit matters reported under these procedures will be communicated promptly to the audit committee.

Board Committees

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee. Prior to the completion of this offering, the composition of these committees, subject to applicable phase-in rules, will meet the criteria for independence under, and the functioning of these committees will comply with, the applicable requirements of the rules of NASDAQ and SEC rules and regulations. We intend to comply with future requirements as they become applicable to us.

Each committee operates under a charter that has been approved by our board of directors. Prior to the completion of this offering, copies of each committee's charter will be posted on the Investor Relations section of our website, which is located at www.regenxbio.com. Each committee has the composition and responsibilities described below. Our board of directors may from time to time establish other committees.

Audit Committee

In June 2015, our board of directors adopted a revised charter for the audit committee of the board, which is currently comprised of Luke M. Beshar, Donald J. Hayden, Jr. and Camille Samuels, each of whom is a non-employee member of the board of directors. Luke M. Beshar serves as the chair of the audit committee. The audit committee's main function is to oversee our accounting and financial reporting processes, internal systems of control, independent registered public accounting firm relationships and the audits of our financial statements. Pursuant to the audit committee charter, the functions of the committee include, among other things:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from such firm;
- reviewing and discussing with management and the registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting and our disclosure controls and procedures;
- meeting independently with our independent registered public accounting firm and management;
- furnishing the audit committee report required by SEC rules;
- reviewing and approving or ratifying any related party transactions; and
- overseeing our risk assessment and risk management policies.

All members of our audit committee meet the requirements for financial literacy under the applicable rules and regulations of the SEC and NASDAQ. Our board of directors has determined that Luke M. Beshar is an "audit committee financial expert" as defined by applicable SEC rules and has the requisite financial sophistication as defined under the applicable NASDAQ rules and regulations.

Our board of directors has determined that each of Luke M. Beshar and Camille Samuels satisfies the independence requirements for audit committee members under the listing standards of NASDAQ and Rule 10A-3 of the Securities Exchange Act. Our board of directors has determined that Donald J. Hayden, Jr. is not currently an independent director under NASDAQ listing standards. However, we are permitted to phase-in our compliance with the independent audit committee requirements set forth in the rules of NASDAQ which would require the audit committee be comprised of all independent directors within one year of listing. We expect that, within one year of our listing on NASDAQ, Donald J. Hayden, Jr. will have resigned from our audit committee and an independent director for audit committee purposes (as determined under the listing standards of NASDAQ and Securities Exchange Act rules) will have been added to our audit committee.

Compensation Committee

In June 2015, our board of directors established a compensation committee, which is currently comprised of Luke M. Beshar, Donald J. Hayden, Jr. and Camille Samuels. Donald J. Hayden, Jr. serves as the chair of the compensation committee. Our compensation committee reviews and recommends policies relating to compensation and benefits of our officers and employees. Pursuant to the compensation committee charter, the functions of this committee include:

- evaluating the performance of our chief executive officer and determining the chief executive officer's salary and contingent compensation based on his or her performance and other relevant criteria;
- identifying the corporate and individual objectives governing the chief executive officer's compensation;

- approving the compensation of our other executive officers;
- making recommendations to our board with respect to director compensation;
- reviewing and approving the terms of material agreements between us and our executive officers;
- overseeing and administering our equity incentive plans and employee benefit plans;
- reviewing and approving policies and procedures relating to the perquisites and expense accounts of our executive officers;
- preparing the annual compensation committee report required by SEC rules; and
- conducting a review of executive officer succession planning, as necessary, reporting its findings and recommendations to our board of directors, and working with the Board in evaluating potential successors to executive officer positions.

In accordance with NASDAQ listing standards, our board of directors has granted our compensation committee the authority and responsibility required under Rules 10C-1(b)(2), (3) and (4) of the Securities Exchange Act, relating to the authority to retain or obtain the advice of compensation consultants, legal counsel and other compensation advisers, the authority to fund such advisers, and the responsibility to consider the independence factors specified under Rules 10C-1(b)(4)(i) through (vi) and any additional factors the compensation committee deems relevant.

Our board of directors has determined that each of Luke M. Beshar and Camille Samuels satisfies the independence requirements for compensation committee members under the listing standards of NASDAQ and Rule 10C-1 of the Securities Exchange Act. Our board of directors has determined that Donald J. Hayden, Jr. is not currently an independent director under NASDAQ listing standards. However, we are permitted to phase-in our compliance with the independent compensation committee requirements set forth in the rules of NASDAQ and the Securities Exchange Act, which would require the compensation committee to be compromised of all independent members within one year of listing. We expect that, within one year of our listing on NASDAQ, Donald J. Hayden, Jr. will have resigned from our compensation committee. At such time, we may appoint an independent director (as determined under the listing standards of NASDAQ and Securities Exchange Act rules) to our compensation committee or have two directors serve on the committee. Our board of directors has determined that each of Luke M. Beshar, Donald J. Hayden, Jr. and Camille Samuels is a “non-employee director” as defined in Rule 16b-3 promulgated under the Securities Exchange Act and is an “outside director” as that term is defined in Section 162(m) of the Internal Revenue Code.

Nominating and Corporate Governance Committee

In June 2015, our board of directors established a nominating and corporate governance committee of the board, which is currently comprised of Edgar G. Engleman, M.D. and A.N. “Jerry” Karabelas, Ph.D. A.N. “Jerry” Karabelas, Ph.D. serves as the chair of the nominating and corporate governance committee. Pursuant to the nominating and corporate governance committee charter, the functions of this committee include, among other things:

- identifying, evaluating, and making recommendations to our board of directors and our stockholders concerning nominees for election to our board, to each of the board’s committees and as committee chairs;
- annually reviewing the performance and effectiveness of our board and developing and overseeing a performance evaluation process;
- annually evaluating the performance of management, the board and each board committee against their duties and responsibilities relating to corporate governance;

- annually evaluating adequacy of our corporate governance structure, policies, and procedures; and
- providing reports to our board regarding the committee's nominations for election to the board and its committees.

Disclosure Committee and Committee Charter

We have a disclosure committee and disclosure committee charter. Our disclosure committee is comprised of all of our directors, our Chief Executive Officer, Chief Financial Officer, Chief Medical Officer and General Counsel. The purpose of the committee is to provide assistance to the Chief Executive Officer and the Chief Financial Officer in fulfilling their responsibilities regarding the identification and disclosure of material information about us, and the accuracy, completeness and timeliness of our financial reports.

Compensation Committee Interlocks and Insider Participation

None of the members of our compensation committee will be or will have in the past served as an officer or employee of our company. None of our executive officers will serve, or in the past year have served, as a member of the board of directors or compensation committee of any entity that has one or more executive officers serving on our board of directors or compensation committee.

Limitations on Liability and Indemnification Matters

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to our directors, officers or controlling persons, we have been informed that in the opinion of the SEC such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

DIRECTOR COMPENSATION

During our fiscal year ended December 31, 2014, we paid cash fees and granted options to purchase shares of our common stock to the Chairman of our board of directors in return for his services as a director. Kenneth T. Mills, our president and chief executive officer and a member of our board of directors, did not receive any compensation from us during our fiscal year ended December 31, 2014 for his service as a director and is not included in the table below.

Name	Fees Earned or Paid In Cash	Option Awards(1)	Total
Benjamin Auspitz(2)	$ —	$ —	$ —
Luke M. Beshar(3)	$ —	$ —	$ —
Edgar G. Engleman, M.D.(4)	$ —	$ —	$ —
Allan M. Fox	$ —	$ —	$ —
Michael Gelman(5)	$ —	$ —	$ —
Donald J. Hayden, Jr.	$ 40,000	$ 180,591	$220,591
Jerry Karabelas, Ph.D.(6)	$ —	$ —	$ —
John Daniel Kiser(7)	$ —	$ —	$ —
Camille Samuels(8)	$ —	$ —	$ —

(1) Reflects the aggregate grant date fair value of options granted during the fiscal year calculated in accordance with FASB ASC Topic 718. See Note 2 to our financial statements for the years ended December 31, 2014 and 2013 included elsewhere in this prospectus for a discussion of the assumptions made by us in determining the grant date fair value of our equity awards.
(2) Mr. Auspitz resigned from our board of directors in May 2015.
(3) Mr. Beshar joined our board of directors in April 2015.
(4) Dr. Engleman joined our board of directors in May 2015.
(5) Mr. Gelman joined our board of directors in February 2015 and resigned from our board of directors in April 2015.
(6) Dr. Karabelas joined our board of directors in June 2015.
(7) Mr. Kiser resigned from our board of directors in April 2015.
(8) Ms. Samuels joined our board of directors in February 2015.

As of December 31, 2014, the following non-employee directors held outstanding options to purchase shares of our common stock: Mr. Hayden (354,100 shares); and as of May 31, 2015, the following non-employee directors held outstanding options to purchase shares of our common stock: Mr. Hayden (466,100 shares), Mr. Beshar (80,000 shares), Dr. Karabelas (40,000 shares). Dr. Engleman, Mr. Fox and Ms. Samuels were not issued options as a result of their or their respective affiliates' ownership of our capital stock.

Non-Employee Director Compensation

Our board of directors, upon the recommendation of our compensation committee, adopted a compensation program for non-employee directors in August 2015. Pursuant to the program, each member of our board of directors who is not our employee will receive the following cash compensation for board services, as applicable:

- $35,000 per year for service as a board of directors member;
- $30,000 per year for service as chairman of the board of directors;
- $15,000 per year for service as chairman of the audit committee;
- $7,500 per year for service as a member of the audit committee;
- $10,000 per year for service as chairman of the compensation committee;
- $5,000 per year for service as a member of the compensation committee;

- $8,000 per year for service as chairman of the nominating and corporate governance committee; and
- $4,000 per year for service as a member of the nominating and corporate governance committee

Non-employee members of our board of directors will also receive automatic grants of non-statutory stock options under our 2015 Equity Incentive Plan. Each non-employee director joining our board of directors will automatically be granted a non-statutory stock option to purchase 25,000 shares of our common stock with an exercise price equal to the fair market value of our common stock on the grant date. Each of these options will vest in equal monthly installments over the 36 months following the date of the grant, and each provides for full acceleration in the event of a change of control. Upon consummation of this offering, Dr. Engleman, Mr. Fox and Ms. Samuels will be granted an initial option grant as set forth above.

In addition, on the date of each annual meeting of our stockholders, each non-employee director will automatically be granted a non-statutory stock option to purchase 12,500 shares of our common stock with an exercise price equal to the fair market value of our common stock on the grant date. A non-employee director who receives an initial award will not receive the additional annual award in the same calendar year. Automatic annual grants vest in equal monthly installments over the 12 months following the date of the grant, and each provides for full acceleration in the event of a change of control. Upon consummation of this offering, Messrs. Beshar and Hayden and Dr. Karabelas will be granted their respective annual option grant in a pro-rated amount to reflect their service from the month of this offering through the date of our annual meeting of stockholders in 2016.

We will also continue to reimburse our non-employee directors for their reasonable out-of-pocket expenses incurred in attending board and committee meetings.

Pursuant to the letter agreement he entered into with us on February 6, 2013, Mr. Hayden, the Chairman of our board of directors, agreed to serve as a member of our board of directors. In consideration of such services, we agreed to pay Mr. Hayden an annual fee of $40,000. Pursuant to his letter agreement, we issued Mr. Hayden an option to purchase 6,420,000 Class B Preferred Units of our predecessor limited liability company. In connection with our conversion to a C-corporation in September 2014, Mr. Hayden's Class B Preferred Units were cancelled and Mr. Hayden received an option to purchase 354,100 shares of our common stock. We intend to terminate the letter agreement with Mr. Hayden upon completion of this offering, and Mr. Hayden will be compensated in accordance with the provisions of our compensation program for non-employee directors.

EXECUTIVE COMPENSATION

Summary Compensation Table

The following table provides information concerning the compensation paid to our President and Chief Executive Officer and our next two most highly compensated executive officers during the year ended December 31, 2014. We refer to these individuals as our named executive officers.

Name and Principal Position	Year	Salary ($)	Bonus ($)	Option Awards ($)(1)	All Other Compensation ($)	Total ($)
Kenneth T. Mills	2014	$ 500,000	$ 250,000	$ 361,182	$ 35,805(3)	$ 1,146,998
President and Chief Executive Officer	2013	$ 500,000	$ 200,000	—	$ 406,883	$ 1,106,883
Stephen Yoo, M.D.	2014	$ 65,625(2)	$ 20,000	$ 121,471	$ 4,296(3)	$ 211,392
Chief Medical Officer						
Vittal K. Vasista	2014	$ 300,000	$ 120,000	$ 216,669	$ 29,212(3)	$ 665,881
Chief Financial Officer	2013	$ 295,000	$ 60,000	—	$ 28,526(3)	$ 383,526

(1) Reflects the aggregate grant date fair value of options granted during the fiscal year calculated in accordance with FASB ASC Topic 718. See Note 9 to our audited financial statements for the years ended December 31, 2014 and 2013, each included elsewhere in this prospectus for a discussion of the assumptions made by us in determining the grant date fair value of our equity awards.

(2) Officer's employment with us commenced on October 13, 2014. The amount reported represents the pro rata portion of the officer's annual salary from commencement of employment through December 31, 2014.

(3) Represents payment of healthcare premiums.

Narrative Explanation of Certain Aspects of the Summary Compensation Table

Pursuant to employment agreements entered into with us, as amended from time to time, each of our named executive officers is eligible to receive a base salary and an annual discretionary bonus payable in cash, stock or a combination and based on the achievement of individual and corporate objectives.

The base salary and target annual performance bonus for each of our named executive officers for our fiscal year ended December 31, 2014, is listed in the table below:

Name	2014 Base Salary ($)	2014 Target Performance Bonus (%)
Kenneth T. Mills	$ 500,000	40%
Stephen Yoo, M.D.	$ 315,000	30%
Vittal K. Vasista	$ 300,000	30%

Objectives for the named executive officers' target bonuses for our fiscal year ended December 31, 2014 included both subjective and objective goals determined in the discretion of our board of directors. Subject to the completion of this offering, Mr. Mills' target bonus will be increased to 50% of his base salary and Dr. Yoo's and Mr. Vasista's target bonus will be increased to 35% of their respective base salaries for the portion of our fiscal year after the effective date of this offering and thereafter.

On February 1, 2015, the annual base salary of Mr. Vasista was increased to $315,000. Subject to completion of this offering, Dr. Yoo's annual base salary will be increased to $340,000.

Each of our named executive officers is eligible to receive certain benefits if his employment is terminated under certain circumstances, as described under "Employment Agreements" below.

Equity Compensation

Since our conversion to a C-corporation, we have offered stock options to our employees, including our named executive officers, as the long-term incentive component of our compensation program. We typically grant equity awards to new hires upon their commencing employment with us. Stock options allow employees to purchase shares of our common stock at a price per share equal to the fair market value of our common stock on the date of grant and may or may not be intended to qualify as "incentive stock options" for U.S. federal income tax purposes. Awards to newly hired employees generally vest with respect to 25% of the total number of option shares on the first anniversary of the vesting commencement date and in equal monthly installments over the following 36 months.

As described under "Outstanding Equity Awards as of December 31, 2014" below, certain equity awards granted to our named executive officers are subject to accelerated vesting in the event such officer is subject to an involuntary termination or if we experience a change in control.

Outstanding Equity Awards as of December 31, 2014

The following table sets forth information regarding each outstanding and unexercised option held by each of our named executive officers as of December 31, 2014. The number of shares subject to each award and, where applicable, the exercise price per share, reflects all changes as a result of our capitalization adjustments.

The vesting schedule applicable to each outstanding award is described in the footnotes to the table below.

		Option Awards				
Name	Vesting Commencement Date	Number of Securities Underlying Unexercised Options Exercisable	Number of Securities Underlying Unexercised Options Unexercisable	Equity Incentive Plan Awards: Number of Securities Underlying Unexercised, Unearned Options	Option Exercise Price ($)	Option Expiration Date
Kenneth T. Mills	9/17/2014	120,394(1)	354,096(2)	233,710(3)	$ 0.85	9/23/2024
Stephen Yoo, M.D.	10/13/2014	23,600(1)	141,600(4)	82,700(3)	0.85	11/3/2024
Vittal K. Vasista	9/17/2014	72,235(1)	212,448(5)	140,217(3)	0.85	9/23/2024

(1) The option vested with respect to these shares on the vesting commencement date.

(2) Subject to the optionee providing continuous service to our company, the option vests with respect to 88,524 shares on the one year anniversary of the vesting commencement date and with respect to an additional 7,377 shares following each month of service following such date.

(3) The vesting of the option with respect to these shares (the Contingent Shares) was conditioned on our completion of a financing in which we raised gross proceeds of not less than $5,000,000 on or before January 1, 2016 (a Qualified Financing), which was satisfied upon the consummation of our Series C Preferred Stock financing in February 2015. As such, effective as of the closing of our Series C Preferred Stock financing, the option vested with respect to 25% of the Additional Shares with respect to the options held by Messrs. Mills and Vasista, and 15% of the Contingent Shares with respect to the option held by Dr. Yoo, as of the vesting commencement date. Subject to the optionee providing continuous service to our company, the option vests with respect to 25% of the remaining Contingent Shares on the one year anniversary of the vesting commencement date and with respect to an additional 1/48th of such remaining Contingent Shares following each month of service following such date.

(4) Subject to the optionee providing continuous service to our company, the option vests with respect to 35,400 shares on the one year anniversary of the vesting commencement date and with respect to an additional 2,950 shares following each month of service following such date.

(5) Subject to the optionee providing continuous service to our company, the option vests with respect to 53,112 shares on the one year anniversary of the vesting commencement date. Further, subject to achievement of

certain business goals, including completion of our initial public offering, our hiring of a new chief financial officer and achievement of two other business goals by December 31, 2015, then another 14,833 shares will vest on achievement of each of the four business goals for a total of 59,533 shares that would have otherwise vested between January 2018 and September 2018. The remaining shares shall vest in equal monthly installments following each month of service following such date.

On May 19, 2015, our board of directors approved the grant of options to purchase common stock to Messrs. Mills and Vasista and Dr. Yoo. Mr. Mills was granted an option to purchase 275,000 shares of our common stock; Mr. Vasista was issued an option to purchase 30,000 shares of our common stock; and Dr. Yoo was issued an option to purchase 70,000 shares of our common stock. Each of the options has a vesting commencement date of May 19, 2015, a 10 year term and vest 25% on completion of one year of service following the vesting commencement date and in 36 equal monthly installments thereafter.

Employment Agreements

In connection with this offering, the compensation committee retained an independent compensation consultant, Radford, to provide the committee with comparative information on executive compensation at peer group companies as well as advice on terms of employment for our named executive officers. Subject to completion of this offering, and based on consultations with Radford, we have entered into new employment agreements with our named executive officers. Pursuant to the employment agreements, if we terminate the employment of our Chief Executive Officer and our other named executive officers without cause or if such officer voluntarily resigns for good reason, then each will be eligible to receive, contingent on his timely executing and not revoking a general release of all claims he may have against us and on his returning all of our property in his possession, continued payment of base salary for (i) 12 months for Mr. Mills and (ii) nine months for Dr. Yoo and Mr. Vasista. If a terminated named executive officer obtains employment during the salary continuation period, then we will cease to be obligated to pay the terminated named executive officer any further payments. In addition, we will pay the terminated named executive officer a lump sum equal to the COBRA premiums for the same period of time.

Further, if we terminate the employment of our Chief Executive Officer and our other named executive officers without cause or if such officer voluntarily resigns for good reason immediately prior to or during the 18 months following a change in control, as such term is defined in our 2015 Plan, then each will be eligible to receive, contingent on his timely executing and not revoking a general release of all claims he may have against us and on his returning all of our property in his possession, continued payment of base salary and target annual bonus for (i) 18 months for Mr. Mills and (ii) 12 months for Dr. Yoo and Mr. Vasista. In addition, we will pay the named executive officer a lump sum equal to the COBRA premiums for the same period of time. All outstanding unvested options that were outstanding as of the date of a change in control will vest if we or our successor terminates the employment of our Chief Executive Officer or other named executive officers without cause or if such officer voluntarily resigns for good reason during the remaining vesting period.

“Cause” means, with respect to Messrs. Mills and Vasista and Dr. Yoo:

- the conviction of, or the entering a plea of guilty or no contest (or pleading or accepting deferred adjudication or receiving unadjudicated probation) to or for, any felony or any crime involving moral turpitude;
- the commission of a material breach of any of the covenants, terms and provisions of the employment agreement or the proprietary information and inventions agreement;
- the commission of an act of fraud, embezzlement, misappropriation, willful misconduct or breach of fiduciary duty against us or other similar conduct materially harmful or potentially materially harmful to our best interest, as determined by our Board, in its reasonable sole discretion; or
- the failure to perform assigned duties or responsibilities, provided we provide the executive written notice and he fails to cure the failure within 10 days of receiving such notice.

"Good Reason" means an officer's resignation within 12 months after one of the following conditions comes into existence without such officer's consent, provided the officer gives us written notice of the condition within 90 days after it first comes into existence and we fail to remedy such condition within 30 days after receipt of such written notice:

- a significant reduction in the officer's duties or responsibilities or removal from officer's position, unless he is assigned comparable duties or responsibilities or employed in a different position, respectively;
- a significant reduction (30% or more) in base salary;
- a significant reduction in the type or level of employee benefits to which officer is entitled that results in a significant reduction in officer's overall benefits package (other than a reduction applicable to all employees) as determined in Board's sole discretion; or
- a relocation of the officer's principal workplace by more than 35 miles.

In connection with their employment, our named executive officers entered into our standard form of proprietary information and inventions agreement. The proprietary information agreement provides that our officers are, generally, prohibited for one year after termination of employment from, directly or indirectly, soliciting our employees or customers, or competing against us.

Retirement Benefits

We have established a 401(k) tax-deferred savings plan, which permits participants, including our named executive officers, to make contributions by salary deduction pursuant to Section 401(k) of the Internal Revenue Code. We are responsible for administrative costs of the 401(k) plan. We may, at our discretion, make matching contributions to the 401(k) plan.

Employee Benefits and Perquisites

Our named executive officers are eligible to participate in our health and welfare plans to the same extent as all full-time employees would be eligible generally, including reimbursement of certain medical expenses incurred by such named executive officer and, if applicable, his or her eligible dependents. We pay 100% of the premium cost for our group health plan for all of our employees including the named executive officers.

We do not generally provide our named executive officers with perquisites or other personal benefits (other than occasional payment of relocation expenses and severance benefits, as described above).

Equity Plans

2015 Equity Incentive Plan

General. Our board of directors adopted our 2015 Equity Incentive Plan (2015 Plan) in July 2015, and we expect our stockholders to approve the 2015 Plan prior to the completion of this offering. The 2015 Plan became effective immediately on adoption although no awards will be made under it until the effective date of the registration statement of which this prospectus is a part. Our 2015 Plan replaced our 2014 Plan (described below). However, awards outstanding under our 2014 Plan will continue to be governed by their existing terms.

Share Reserve. shares of our common stock were reserved for issuance under our 2015 Plan, which amount equals the sum of shares plus up to shares remaining available for issuance under, or issued pursuant to or subject to awards granted under our 2014 Plan. The number of shares reserved for issuance under the 2015 Plan will be increased automatically on the first business day of each of our fiscal years, commencing in 2016, by a number equal to the smallest of:

- 4% of the shares of common stock outstanding on the last business day of the prior fiscal year; or

- the number of shares determined by our board of directors.

In general, to the extent that any awards under the 2015 Plan are forfeited, terminate, expire or lapse without the issuance of shares, or if we repurchase the shares subject to awards granted under the 2015 Plan, those shares will again become available for issuance under the 2015 Plan, as will shares applied to pay the exercise or purchase price of an award or to satisfy tax withholding obligations related to any award.

Administration. The compensation committee of our board of directors administers the 2015 Plan. The compensation committee has complete discretion to make all decisions relating to the 2015 Plan and outstanding awards, including repricing outstanding options and modifying outstanding awards in other ways.

Eligibility. Employees, non-employee directors, consultants and advisors are eligible to participate in our 2015 Plan.

Types of Awards. Our 2015 Plan provides for the following types of awards:

- incentive and nonstatutory stock options;
- stock appreciation rights;
- restricted shares;
- stock units; and
- performance cash awards.

Options and Stock Appreciation Rights. The exercise price for options granted under the 2015 Plan may not be less than 100% of the fair market value of our common stock on the grant date; however, the exercise price for an incentive stock option granted to a holder of more than 10% of our stock may not be less than 110% of such fair market value on the grant date in accordance with Section 422(c)(5) of the Internal Revenue Code. Optionees may pay the exercise price in cash or, with the consent of the compensation committee:

- with shares of common stock that the optionee already owns;
- by an immediate sale of shares through a broker approved by us;
- by instructing us to withhold a number of shares having an aggregate fair market value that does not exceed the exercise price; or
- by other methods permitted by applicable law.

An optionee who exercises a stock appreciation right receives the increase in value of our common stock over the base price. The base price for stock appreciation rights may not be less than 100% of the fair market value of our common stock on the grant date. The settlement value of a stock appreciation right may be paid in cash, shares of our common stock or a combination.

Options and stock appreciation rights vest as determined by the compensation committee. In general, they will vest over a four-year period following the date of grant. Options and stock appreciation rights expire at the time determined by the compensation committee but in no event more than ten years after they are granted. These awards generally expire earlier if the participant's service terminates earlier. No participant may be granted stock options or stock appreciation rights under our 2015 Plan covering more than shares in any calendar year, except that a new employee may receive stock options or stock appreciation rights covering up to additional shares in the calendar year in which employment commences.

Restricted Shares and Stock Units. Restricted shares and RSUs may be awarded under the 2015 Plan in return for any lawful consideration, and participants who receive restricted shares or stock units generally are not required to pay cash for their awards. In general, these awards will be subject to vesting. Vesting may be based on length of service, the attainment of performance-based milestones or a combination of both, as determined by

the compensation committee. No participant may be granted restricted share awards or stock units with performance-based vesting covering more than shares during any calendar year, except that a new employee may receive restricted shares or stock units covering up to additional shares in the calendar year in which employment commences. Settlement of vested stock units may be made in the form of cash, shares of common stock or a combination.

Performance Cash Awards. Performance cash awards may be granted under the 2015 Plan that qualify as performance-based compensation that is not subject to the income tax deductibility limitations imposed by Section 162(m) of the Internal Revenue Code, if the award is approved by our compensation committee and the grant or vesting of the award is tied solely to the attainment of performance goals during a designated performance period. No participant may be paid more than $1.0 million in cash in any calendar year pursuant to a performance cash award granted under the 2015 Plan.

Performance goals for the grant or vesting of performance awards under the 2015 Plan may be based on any one, or combination, of the following:

- Earnings (before or after taxes)
- Earnings per share
- Earnings before interest, taxes and depreciation
- Earnings before interest, taxes, depreciation and amortization
- Total stockholder return
- Return on equity or average stockholders' equity
- Return on assets, investment or capital employed
- Operating income
- Gross margin
- Operating margin
- Net operating income
- Net operating income after tax
- Return on operating revenue
- Objective corporate or individual strategic goals
- Sales or revenue (using a measure thereof that complies with Section 162(m))
- Expense or cost reduction
- Working capital
- Economic value added (or an equivalent metric)
- Market share
- Cash measures including cash flow and cash balance
- Operating cash flow
- Cash flow per share
- Share price
- Debt reduction
- Customer satisfaction
- Stockholders' equity
- Contract awards or backlog
- Objective individual performance goals

To the extent a performance award is not intended to comply with Section 162(m) of the Internal Revenue Code, the compensation committee may select other measures of performance.

Corporate Transactions. In the event we are a party to a merger, consolidation or certain change in control transactions, outstanding awards granted under the 2015 Plan, and all shares acquired under the 2015 Plan, will be subject to the terms of the definitive transaction agreement (or, if there is no such agreement, as determined by our compensation committee). Such treatment may include any of the following with respect to each outstanding award:

- the continuation, assumption or substitution of an award by a surviving entity or its parent;
- the cancellation of the vested portion of an award (and any portion that becomes, or would become, vested as of, or following, the effective time of the transaction) in exchange for a payment equal to the

excess, if any, of the value that the holder of each share of our common stock receives in the transaction over (if applicable) the exercise price otherwise payable in connection with the stock award; or

- the assignment of any reacquisition or repurchase rights held by us in respect of an award of restricted shares to the surviving entity or its parent (with proportionate adjustments made to the price per share to be paid upon exercise of such rights).

The compensation committee is not required to treat all awards, or portions thereof, in the same manner.

The vesting of an outstanding award may be accelerated upon the occurrence of a change in control, whether or not the award is to be assumed or replaced in the transaction, or in connection with a termination of service following a change in control transaction.

A change in control includes:

- any person acquiring beneficial ownership of more than 50% of our total voting power;
- the sale or other disposition of all or substantially all of our assets;
- our merger or consolidation after which our voting securities represent 50% or less of the total voting power of the surviving or acquiring entity; or
- a majority of our board of directors being replaced, over a 12-month period, by persons whose appointment or election is not endorsed by a majority of our board of directors.

Changes in Capitalization. In the event of certain changes in our capital structure without our receipt of consideration, such as a stock split, reverse stock split or dividend paid in common stock, proportionate adjustments will automatically be made to:

- the maximum number and kind of shares available for issuance under the 2015 Plan, including the maximum number and kind of shares that may be issued upon the exercise of incentive stock options;
- the maximum number and kind of shares covered by, and exercise price, base price or purchase price, if any, applicable to each outstanding stock award;
- the maximum number and kind of shares by which the share reserve may increase automatically each year; and
- the maximum number and kind of shares subject to stock awards that may be granted to a participant in a fiscal year (as established under the 2015 Plan pursuant to Section 162(m) of the Internal Revenue Code).

In the event that there is a declaration of an extraordinary dividend payable in a form other than our common stock in an amount that has a material effect on the price of our common stock, a recapitalization, a spin-off or a similar occurrence, the compensation committee may make such adjustments to any of the foregoing as it deems appropriate, in its sole discretion.

Amendments or Termination. Our board of directors may amend or terminate the 2015 Plan at any time. If our board of directors amends the 2015 Plan, it does not need stockholder approval of the amendment unless required by applicable law, regulation or rules. The 2015 Plan will terminate automatically 10 years after the later of the date when our board of directors adopted the 2015 Plan or approved the latest share increase that was also approved by our stockholders.

2014 Stock Plan

Our 2014 Stock Plan (the 2014 Plan) was adopted by our board of directors and approved by our stockholders in September 2014. Our 2014 Plan was subsequently amended on January 8, 2014. No further awards will be made under our 2014 Plan. Awards outstanding under our 2014 Plan continue to be governed by their existing terms.

Share Reserve. As of June 30, 2015, options to purchase 3,063,200 shares of our common stock were outstanding under the 2014 Plan.

Administration. The compensation committee of our board of directors administers the 2014 Plan. The committee has the complete discretion to make all decisions relating to the plan and outstanding awards, including repricing outstanding options and modifying outstanding awards in other ways.

Eligibility. Employees, non-employee directors and consultants were eligible to participate in our 2014 Plan; however, only employees are eligible for the grant of incentive stock options.

Types of Awards. The 2014 Plan provides for the following types of awards granted with respect to shares of our common stock:

- incentive and nonstatutory stock options;
- direct award or sale of shares of our common stock; and
- restricted stock units.

Terms of Awards. Subject to the terms of the 2014 Plan, the plan administrator determines the terms of all awards.

- The exercise price for options granted under the 2014 Plan may not be less than 100% of the fair market value of our common stock on the grant date; however, the exercise price for an incentive stock option granted to a holder of more than 10% of our stock may not be less than 110% of such fair market value on the grant date. Options are generally transferable only by beneficiary designation, a will or the laws of descent and distribution; however, the administrator may permit the transfer of stock options by gift or pursuant to a domestic relations order. The term of options granted under the 2014 Plan may not exceed ten years and will generally expire sooner if the optionee's service terminates. Options vest at the times determined by the administrator.
- Restricted stock units and restricted shares may be awarded under the 2014 Plan in return for any lawful consideration, including consideration for services rendered to us. Shares may also be sold under the 2014 Plan. Participants who receive restricted stock units generally are not required to pay cash for their awards. Shares awarded or sold under the 2014 Plan may be fully vested at grant or subject to special forfeiture conditions or rights of repurchase, as determined by the administrator.
- Participants may pay the exercise price for options, or the purchase price for shares (if applicable) in cash or check, or at the discretion of the plan administrator, by tendering shares of common stock already owned; through withholding by the company of shares otherwise issuable; by tender of a promissory note; through a cashless exercise program established with a securities brokerage firm; through any other lawful consideration; or any combination of the above. Settlement of vested stock units may be made in the form of cash, shares of common stock or a combination

Corporate Transactions. In the event that we are a party to a merger, consolidation, or sale of all or substantially all of our assets, all outstanding options and other awards shall be treated in the manner described in the definitive transaction agreement or, if the transaction does not entail a definitive agreement to which we are a party, as determined by the administrator in its sole discretion. Such treatment may include, without limitation:

- the continuation, assumption or substitution of an award by the surviving entity or its parent;
- the cancellation of any portion of an option not exercised without payment of any consideration;
- the cancellation of the vested portion of outstanding options or share awards in exchange for a payment per share equal to the excess, if any, of (a) the consideration payable in such transaction to a holder of shares of common stock over (b) the per share exercise or purchase price of the award, if any; or
- cancellation of options without the payment of any consideration.

The administrator may, in its discretion, accelerate the vesting of any or all portions of outstanding awards. It is not obligated to treat all awards in the same manner.

Changes in Capitalization. All share numbers described in this summary of the 2014 Plan will automatically adjust in the event of a stock split, a stock dividend, or a reverse stock split. In addition, the number of shares subject to awards, and the exercise or purchase applicable to such awards, shall be proportionately adjusted in the event of such change in capitalization. In the event that there is a declaration of an extraordinary dividend payable in a form other than our common stock in an amount that has a material effect on the price of our common stock, a recapitalization, a spin-off or a similar occurrence, the administrator may make such adjustments to any of the foregoing as it deems appropriate, in its sole discretion.

2015 Employee Stock Purchase Plan

General. Our 2015 Employee Stock Purchase Plan (the 2015 ESPP) was adopted by our board of directors in June, and we expect our stockholders to approve the 2015 Plan prior to the completion of this offering. The 2015 ESPP will become effective as of the effective date of the registration statement of which this prospectus is a part. Our 2015 ESPP is intended to qualify under Section 423 of the Internal Revenue Code.

Share Reserve. We have reserved shares of our common stock for issuance under the 2015 ESPP. The number of shares reserved for issuance under the 2015 ESPP will automatically be increased on the first business day of each of our fiscal years, commencing in 2016, by a number equal to the least of:

- one percent of the shares of common stock outstanding on the last business day of the prior fiscal year; or
- the number of shares determined by our board of directors.

The number of shares reserved under the 2015 ESPP will automatically be adjusted in the event of a stock split, stock dividend or a reverse stock split (including an adjustment to the per-purchase period share limit).

Administration. The compensation committee of our board of directors will administer the 2015 ESPP.

Eligibility. All of our employees are eligible to participate if we employ them for more than 20 hours per week and for five months or more per calendar year. Eligible employees may begin participating in the 2015 ESPP at the start of any offering period.

Offering Periods. Each offering period will last a number of months determined by the compensation committee, not to exceed 27 months. A new offering period will begin periodically, as determined by the compensation committee. Offering periods may overlap or may be consecutive. Unless otherwise determined by the compensation committee, two offering periods of six months' duration will begin in each year on January 1st and July 1st.

Amount of Contributions. Our 2015 ESPP permits each eligible employee to purchase common stock through payroll deductions. Each employee's payroll deductions may not exceed 15% of the employee's cash compensation. Each participant may purchase up to the number of shares determined by our board of directors on any purchase date, not to exceed shares. The value of the shares purchased may not exceed $25,000 for each calendar year in which the offering period was outstanding. Participants may withdraw their contributions at any time before stock is purchased.

Purchase Price. The price of each share of common stock purchased under our 2015 ESPP will not be less than 85% of the lower of the fair market value per share of common stock on the first day of the applicable offering period (or, in the case of the first offering period, the price at which one share of common stock is offered to the public in this offering) or the fair market value per share of common stock on the purchase date.

Other Provisions. Employees may end their participation in the 2015 ESPP at any time. Participation ends automatically upon termination of employment with us. If we experience a change in control, our 2015 ESPP will end and shares will be purchased with the payroll deductions accumulated to date by participating employees. Our board of directors or our compensation committee may amend or terminate the 2015 ESPP at any time.

CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS

The following is a description of transactions since January 1, 2012 to which we have been a party, in which the amount involved exceeded or will exceed $120,000, and in which any of our directors, executive officers or beneficial owners of more than five percent of our common stock, or an affiliate or immediate family member thereof, had or will have a direct or indirect material interest, other than compensation, termination and change-in-control arrangements, which are described under "Executive Compensation."

Conversion from Limited Liability Company to Corporation

In September 2014, we converted from a Delaware limited liability company named ReGenX Biosciences, LLC (formerly known as ReGenX, LLC) (the LLC) to REGENXBIO Inc., a Delaware corporation (the Conversion). The Conversion was effected pursuant to a plan of conversion whereby (i) each 50 units of Class A Units of the LLC were converted into one share of our common stock, (ii) each 50 units of our Series A Preferred Units of the LLC were converted into one share of Series A Preferred Stock and (iii) each 50 units of Series B Preferred Units of the LLC were converted into one share of Series B Preferred Stock. Additionally, we terminated all outstanding Class B Units of the LLC and the LLC's equity incentive plan. As part of the Conversion, the members of the LLC became stockholders of ours in exactly the same ownership proportions as immediately prior to the Conversion. Effective upon the Conversion, our stockholders entered into an investors' rights agreement, voting agreement and right of first refusal and co-sale agreement which contained provisions similar to those set forth in the LLC's limited liability company agreement immediately prior to the Conversion. As a result of the Conversion, holders of previously issued preferred units were given the right to convert their units to common stock. Additionally, dividends on the newly issued preferred shares were no longer compounded annually as they were with respect to the previously issued preferred units, which decreased the liquidation and redemption values of the securities.

Series D Financing

In May 2015, we entered into a stock purchase agreement (the Series D Purchase Agreement) with new and existing investors, including certain of our existing stockholders at the time who were represented by members of our board of directors, including entities affiliated with Venrock Partners (Venrock Partners) and Beacon Bioventures Fund III Limited Partnership (Beacon Bioventures), to raise approximately $70.5 million from the sale of 7,366,849 shares of our Series D convertible preferred stock, $0.0001 par value per share (the Series D Preferred Stock), at a purchase price of $9.5699 per share (the Series D Financing).

Series C Financing

In January 2015, we entered into a stock purchase agreement (the Series C Purchase Agreement) with new and existing investors, including FoxKiser and Beacon Bioventures, which were stockholders at the time who were represented by members of our board of directors, to raise approximately $30.0 million, including the conversion of approximately $3.8 million in outstanding convertible notes held by FoxKiser, from the sale of 4,631,774 shares of our Series C convertible preferred stock, $0.0001 par value per share (the Series C Preferred Stock), at a purchase price of $6.477 per share (the Series C Financing).

Series B Financing

In October 2013, we entered into a unit purchase agreement with new and existing investors, including certain of our existing stockholders at the time who were represented by members of our board of directors, including FoxKiser and Beacon Bioventures, to raise approximately $7.9 million, including the conversion of approximately $5.9 million in outstanding convertible notes held by FoxKiser, from the sale of 95,314,803 (pre-Conversion units) Series B Preferred Units, at a purchase price of $0.082798 per unit. These units were converted into 1,906,295 shares of Series B Preferred Stock in the Conversion.

The following table summarizes the issuances and purchases of our preferred stock and common stock in the Conversion, the Series D Financing, the Series C Financing and the Series B Financing by our directors, officers or the beneficial holders of more than five percent of our capital stock (excluding any shares purchased in this offering) or entities affiliated with them:

Name of Stockholder	REGENXBIO Director	Series A Preferred Stock	Series B Preferred Stock	Series C Preferred Stock	Series D Preferred Stock	Common Stock	Aggregate Purchase Price(1)
Entities Affiliated with Allan M. Fox	Allan M. Fox	1,444,970	853,915(1)	478,463(1)	—	443,700	$ 10,684,132.48(2)
Beacon Bioventures Fund III Limited Partnership	Benjamin Auspitz(3)	—	483,103	236,982	365,731	—	$ 7,034,939.64
Brookside Capital Partners Fund, L.P.	—	—	—	1,080,748	679,213	—	$ 13,500,005.29
Deerfield Private Design Fund III, L.P.	—	—	—	771,963	397,079	—	$ 8,800,001.68
GFO II, LLC	Michael Gelman(4)	—	—	771,963	—	—	$ 5,000,004.36
GlaxoSmithKline LLC	—	—	—	—	—	1,085,824	$ 1,094,118.00(5)
Entities Affiliated with John Daniel Kiser	John Daniel Kiser(6)	948,157	569,277(1)	318,976(1)	—	443,700	$ 7,872,754.98(2)
Entities Affiliated with Venrock Partners	Camille Samuels(7)	—	—	771,963	1,044,944	—	$ 15,000,013.95
Entities Affiliated with Vivo Ventures	Edgar G. Engleman, M.D.(8)	—	—	—	940,449	—	$ 9,000,002.89

(1) Includes shares issued upon the conversion of certain convertible promissory notes then outstanding, for which the converted principal and accrued interest are included in the aggregate purchase price.
(2) Includes $750,000 contributed through in-kind contributions and allocated to entities affiliated with Messrs. Fox and Kiser.
(3) Mr. Auspitz is affiliated with Beacon Bioventures Fund III Limited Partnership, but resigned from our board of directors in May 2015. We have entered into a letter agreement (the Voting Rights Waiver) with Beacon Bioventures pursuant to which Beacon Bioventures agreed to waive all voting rights that it may have in respect of any voting securities issued by us that exceed, in the aggregate, 4.99% of the total voting rights exercisable by our outstanding voting securities.
(4) Mr. Gelman is affiliated with GFO II, LLC (which subsequently transferred its shares to RegenX GRAT U/A/D May 15, 2015) and resigned from our board of directors in April 2015.
(5) Aggregate purchase price based on value of shares of Class A Units issued to GSK upon execution of license agreement.
(6) Mr. Kiser resigned from our board of directors in April 2015.
(7) Ms. Samuels is affiliated with Venrock Partners and is the current director appointed by Venrock Partners.
(8) Dr. Engleman is affiliated with Vivo Capital and is the current director appointed by entities affiliate with Vivo Capital.

Amended and Restated Investors' Rights Agreement

In connection with the closing of the Series D Financing described above, we entered into an amended and restated investors' rights agreement (the Investors' Rights Agreement) with our significant stockholders, including entities affiliated with FoxKiser (which were subsequently transferred to trusts affiliated with Allan M. Fox and John Daniel Kiser), Holdings, Brookside Capital Partners, Venrock Partners, Beacon Bioventures, Deerfield Management and Vivo Capital. See "Principal Stockholders" for additional information regarding the shares held by these entities. Pursuant to this agreement, we granted such stockholders certain registration rights with respect to shares of our common stock and a right of first offer with respect to future issuances of our securities. The sections other than with regard to registration rights of the Investors' Rights Agreement will terminate pursuant to its terms upon the consummation of this offering. For more information regarding this agreement, see "Description of Capital Stock—Registration Rights."

Amended and Restated Voting Agreement

In connection with the closing of the Series D Financing, we entered into an amended and restated voting agreement, along with certain holders of our common stock and convertible preferred stock, including FoxKiser (which were subsequently transferred to trusts affiliated with Allan M. Fox and John Daniel Kiser), Holdings, Brookside Capital Partners, Venrock Partners, Beacon Bioventures, Deerfield Management and Vivo Capital. Under the terms of the voting agreement, the parties have agreed, subject to certain conditions, to vote their shares so as to elect as directors the nominees designated by certain of our investors, including Holdings, which designated Luke M. Beshar, Allan M. Fox, Donald J. Hayden, Jr. and A.N. "Jerry" Karabelas, Ph.D., Venrock Partners, which designated Camille Samuels, and Vivo Capital, which designated Edgar G. Engleman, M.D. In addition, the parties to the voting agreement have agreed to vote their shares so as to elect to our board of directors our Chief Executive Officer, who is currently

Kenneth T. Mills and additional directors nominated by the board of directors and elected by the holders of our common stock and preferred stock. The voting agreement will terminate immediately prior to the completion of this offering.

Amended and Restated Right of First Refusal and Co-Sale Agreement

We are party to a right of first refusal and co-sale agreement (the First Refusal Agreement) with certain holders of our common stock and our convertible preferred stock, including FoxKiser (which were subsequently transferred to trusts affiliated with Allan M. Fox and John Daniel Kiser), Holdings, Brookside Capital Partners, Venrock Partners, Beacon Bioventures, Deerfield Management and Vivo Capital. Allan M. Fox, one of our directors, is a partner of FoxKiser and affiliated with Holdings, Camille Samuels, one of our directors, is a general partner at Venrock Partners and Edgar G. Engleman, M.D., one of our directors, is a partner at Vivo Capital. Pursuant to this agreement, the holders of convertible preferred stock have a right of first refusal and co-sale in respect of certain sales of securities by our founders and management team. Upon the closing of this offering, the right of first refusal and co-sale agreement will terminate.

Dimension Therapeutics, Inc.

In October 2013, we entered into an exclusive license agreement with Dimension Therapeutics, Inc. (Dimension) as part of the formation of Dimension and the licensing of certain portions of our intellectual property portfolio (the Dimension Transaction). As part of the Dimension Transaction, pursuant to the exclusive license agreement, as amended, we exclusively license our NAV Vectors to Dimension for the development and commercialization of products to treat hemophilia A, hemophilia B, ornithine transcarbamylase deficiency and glycogen storage disease type Ia. Under the terms of the agreement, we received 10,000 shares of common stock of Dimension, an annual maintenance fee in the low tens of thousands per disease indication licensed, single digit royalty percentages on net sales of licensed products and Dimension will pay any milestone fees owed by us to GSK or sublicense fees owed by us to Penn or GSK as a result of Dimension's activities under the license agreement. See "Business—License Agreements and Commercial Licenses—Dimension Therapeutics, Inc." for further information regarding our license agreement with Dimension.

In connection with the Dimension Transaction and the formation of Dimension, Allan M. Fox, John Daniel Kiser (who was our director at the time of the Dimension Transaction), Donald J. Hayden, Jr. and Kenneth T. Mills purchased an aggregate of 6,954,536 shares of Dimension's common stock for an aggregate purchase price of $695.45 in October 2013 (the Dimension Purchase), along with the purchase of shares by other investors in Dimension. At the time of the Dimension Purchase, our directors and officers named in the preceding sentence were each greater than five percent beneficial owners of Dimension's outstanding capital stock and held in the aggregate greater than 10% of Dimension's outstanding capital stock. Additionally, at the time of the Dimension Purchase, Messrs. Fox and Hayden were on Dimension's board of directors. As of the date of this prospectus, to our knowledge, none of the Messrs. Fox, Kiser, Hayden or Mills are greater than five percent beneficial owners of Dimension's outstanding capital stock, Mr. Fox is no longer on Dimension's board of directors and Mr. Hayden resigned as a director of Dimension effective as of July 10, 2015.

FoxKiser Service Agreements

In 2012, we entered into a Management Services & Support Agreement with FoxKiser, one of our principle stockholders prior to a stock transfer to affiliated underlying owners and an affiliate of Allan M. Fox, one of our directors, which replaced a prior Services Agreement entered into in February 2009 (the 2012 Services Agreement). Pursuant to the 2012 Services Agreement, we incurred a monthly fixed fee, plus a support fee at the discretion of FoxKiser, for office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance and other services provided to us. In September 2013, we entered into a promissory note agreement with FoxKiser (the 2013 Note) to allow FoxKiser to settle the entire amount accrued under the 2012 Services Agreement at that date of $5.9 million in shares upon

the next round of preferred financing. In October 2012, in connection with the Series B Financing, FoxKiser exercised its share settlement option and converted the $5.9 million outstanding under the 2013 Note into 71,159,630 Series B Preferred Units (which were subsequently converted into 1,423,192 shares of Series B Preferred Stock in the Conversion). All amounts due under the 2013 Note were converted in full in connection with the Series B Financing and the 2013 Note is no longer outstanding.

In September 2014, we entered into an Amended and Restated Management Services & Support Agreement with FoxKiser, which replaced the 2012 Services Agreement (the 2014 Services Agreement). Pursuant to the 2014 Services Agreement, we incurred a monthly fixed fee, plus a support fee at the discretion of FoxKiser, for office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance and other services provided to us. In July 2014, we entered into a promissory note agreement with FoxKiser (the 2014 Note) to allow FoxKiser to settle the entire amount accrued under the 2014 Services Agreement at that date, plus any future accruals under the agreement up to a maximum of $2.0 million, in shares upon the next round of preferred financing. In July and September 2014, we received $1.8 million and $0.6 million, respectively, in promissory notes from FoxKiser, which could be settled in shares upon the next round of preferred financing. In January 2015, in connection with our Series C Financing, FoxKiser exercised its share settlement option and outstanding principal and interest of $3.8 million under the 2014 Note and the July 2014 and September 2014 promissory notes were converted into 585,577 shares of Series C Preferred Stock. In January 2015, we and FoxKiser agreed to mutually terminate the 2014 Services Agreement, which was agreed to as part of the Series C Financing, and we paid the remaining amount due through the termination date in full in cash. All amounts due under the July 2014 and September 2014 promissory notes were converted in full in connection with the Series C Financing and are no longer outstanding.

GlaxoSmithKline LLC

For information regarding our relationship with GSK, please see “License Agreements and Commercial Licenses—Platform Licenses—GlaxoSmithKline LLC” located elsewhere in this prospectus.

Indemnification Agreements

Prior to the consummation of this offering, we expect to enter into separate indemnification agreements with our directors and executive officers. These agreements, among other things, will provide for indemnification of our directors and executive officers for certain expenses, judgments, fines and settlement amounts, among others, incurred by this person in any action or proceeding arising out of this person’s services as a director or executive officer in any capacity with respect to any employee benefit plan or as a director, partner, trustee or agent of another entity at our request. We believe that our indemnification agreements, along with the provisions of our restated certificate of incorporation and amended and restated bylaws will be necessary to attract and retain qualified persons as directors and executive officers.

Employment Agreements

We have entered into offer letters with each of our named executive officers. For more information regarding these agreements, see the section of this prospectus entitled “Executive Compensation—Narrative Explanation of Certain Aspects of the Summary Compensation Table.”

Stock Option Grants to Executive Officers and Directors

We have granted stock options to our executive officers and certain of our directors as more fully described in the section entitled “Director Compensation” and “Executive Compensation.”

Policies and Procedures for Related Party Transactions

We have adopted a related party transaction policy under which our directors and executive officers, including their immediate family members and affiliates, are not permitted to enter into a related party transaction with us without the prior consent of our audit committee, or other independent committee of our board of directors in the case it is inappropriate for our audit committee to review such transaction due to a conflict of interest. Any request for us to enter into a transaction with an executive officer, director, or any of such persons' immediate family members or affiliates, in which the amount involved exceeds $120,000 must first be presented to our audit committee for review, consideration and approval. All of our directors and executive officers are required to report to our audit committee any such related party transaction. In approving or rejecting the proposed agreement, our audit committee shall consider the relevant facts and circumstances available and deemed relevant to the audit committee, including, but not limited to the risks, costs, and benefits to us, the terms of the transaction, the availability of other sources for comparable services or products, and, if applicable, the impact on a director's independence. Our audit committee shall approve only those agreements that, in light of known circumstances, are not inconsistent with our best interests, as our audit committee determines in the good faith exercise of its discretion.

All of the transactions described above were entered into prior to the adoption of our related party transaction policy and were approved by our board of directors. We believe that we have executed all of the transactions set forth above on terms no less favorable to us than we could have obtained from unaffiliated third-parties. It is our intention to ensure that all future transactions between us and our officers, directors, and principal stockholders and their affiliates are approved by a majority of our board of directors, including a majority of the independent and disinterested members of our board of directors, and are on terms no less favorable to us than those that we could obtain from unaffiliated third-parties.

PRINCIPAL STOCKHOLDERS

The following table provides information concerning beneficial ownership of our capital stock as of August 1, 2015, and as adjusted to reflect the sale of the common stock being sold in this offering, by:

- each stockholder, or group of affiliated stockholders, that owns five percent or greater of our outstanding capital stock;
- each of our named executive officers;
- each of our directors; and
- all of our directors and executive officers as a group.

The following table lists the number of shares and percentage of shares beneficially owned based on 19,060,858 shares of our common stock outstanding as of August 1, 2015. This number reflects:

- 2,762,813 shares of common stock; and
- the conversion of 16,298,045 shares of our convertible preferred stock into 16,298,045 shares of common stock upon the closing of this offering.

This number excludes:

- 3,053,050 shares of common stock issuable upon the exercise of options outstanding as of August 1, 2015 under the 2014 Stock Plan at a weighted average exercise price of $1.86 per share;
- 927,100 shares of common stock reserved for issuance under our 2014 Stock Plan; and
- shares of common stock reserved for issuance under our 2015 Equity Incentive Plan, which became effective in June 2015 but with respect to which no awards will be granted prior to the effective date of the registration statement of which this prospectus is a part, and shares of common stock reserved for issuance under our 2015 Employee Stock Purchase Plan which becomes effective on the effective date of the registration statement of which this prospectus is a part, subject in each case to automatic annual adjustment in accordance with the terms of the plan.

The table also lists the applicable percentage beneficial ownership based on shares of common stock outstanding upon completion of this offering, assuming no exercise of the underwriters' option to purchase up to an aggregate of shares of our common stock.

Beneficial ownership is determined in accordance with the rules of the SEC, and generally includes voting power and/or investment power with respect to the securities held. Shares of common stock subject to options currently exercisable or exercisable within 60 days of August 1, 2015, are deemed outstanding and beneficially owned by the person holding such options for purposes of computing the number of shares and percentage beneficially owned by such person, but are not deemed outstanding for purposes of computing the percentage beneficially owned by any other person. Except as indicated in the footnotes to this table, and subject to applicable community property laws, the persons or entities named have sole voting and investment power with respect to all shares of our common stock shown as beneficially owned by them.

Unless otherwise indicated, the principal address of each of the stockholders below is c/o REGENXBIO Inc., 9712 Medical Center Drive, Suite 100, Rockville, MD 20850.

Name and Address of Beneficial Owner	Shares Beneficially Owned Prior to the Offering		Shares Beneficially Owned After the Offering	
	Number	Percentage	Number	Percentage
5% or Greater Stockholders				
Entities Affiliated with Allan M. Fox(1) 750 17th St., NW, Suite 1100 Washington, DC 20006	3,221,048	16.9%		
Entities Affiliated with John Daniel Kiser(2) 750 17th St., NW, Suite 1100 Washington, DC 20006	2,280,110	12.0%		
Entities Affiliated with Venrock Partners(3) 3340 Hillview Avenue Palo Alto, CA 94304	1,816,907	9.5%		
Brookside Capital Partners Fund, L.P.(4) John Hancock Tower 200 Clarendon Street Boston, MA 02116	1,759,961	9.2%		
Deerfield Private Design Fund III, L.P.(5) 780 Third Avenue 37th floor New York, NY 10017	1,169,042	6.1%		
Beacon Bioventures Fund III Limited Partnership(6) 82 Devonshire Street, MZ EPC 13A Boston, MA 02109	1,085,816	5.7%		
GlaxoSmithKline LLC(7) 2301 Renaissance Blvd. Mail Code RN0220 King of Prussia, PA 19406	1,085,824	5.7%		
Directors and Named Executive Officers				
Kenneth T. Mills(8)	326,605	1.7%		
Vittal Vasista(9)	277,131	1.4%		
Stephen Yoo, M.D.(10)	36,005	*		
Donald J. Hayden, Jr.(11)	194,177	1.0%		
Luke Beshar	—	*		
Edgar G. Engleman, M.D.(12)	940,449	4.9%		
Allan M. Fox(1)	3,221,048	16.9%		
A.N. “Jerry” Karabelas, Ph.D.	—	*		
Camille Samuels(13)	1,816,907	9.5%		
All current directors and executive officers as a group (9 persons)(14)	6,812,322	35.7%		

* Less than one percent of the outstanding shares of common stock.

(1) Consists of 443,700 shares of common stock held by FoxKiser Holdings, LLC (Holdings), 722,485 shares of common stock issuable upon conversion of preferred stock held by The Allan M. Fox Trust (U/A/D April 21, 2015) (the Fox Trust) and 2,054,863 shares of common stock issuable upon conversion of preferred stock held by The Allan M. Fox Revocable Trust. Mr. Fox holds shared dispositive power over the

shares held by Holdings described in the foregoing sentence with John Daniel Kiser, with Mr. Fox having a 60% voting interest in Holdings. Mr. Kiser is the trustee of the Fox Trust and holds sole dispositive voting power over such trust. Mr. Fox otherwise holds sole dispositive power over the shares held by the other entities described.

(2) Consists of 443,700 shares of common stock held by Holdings, 948,157 shares of common stock issuable upon conversion of preferred stock held by The Kiser 2012 Gift Trust (the Kiser Gift Trust) and 888,253 shares of common stock issuable upon conversion of preferred stock held by the John Daniel Kiser Revocable Trust U/A/D July 27, 2011. Mr. Kiser holds shared dispositive power over the shares held by Holdings described in the foregoing sentence with Mr. Fox, with Mr. Kiser having a 40% voting interest in Holdings. Mr. Fox is the trustee of the Kiser Gift Trust and holds sole dispositive voting power over such trust. Mr. Kiser holds sole dispositive power over the shares held by the other entities described.

(3) Consists of 838,956 shares of common stock issuable upon conversion of preferred stock held by Venrock Associates VII, L.P. (VA VII), 696,311 shares of common stock issuable upon conversion of preferred stock held by Venrock Healthcare Capital Partners II, L.P. (VHCP II), 212,143 shares of common stock issuable upon conversion of preferred stock held by VHCP CO-Investment Holdings II, LLC (VHCP Co. II) and 69,497 shares of common stock issuable upon conversion of preferred stock held by Venrock Partners VII, L.P. (VP VII). Venrock Management VII, LLC (VM VI) is the sole general partner of VA VII. Venrock Partners Management VII, LLC (VPM VII) is the sole general partner of VP VII. VHCP Management II, LLC (VHCPM II) is the sole general partner of each of VHCP II and VHCP Co. II. VM VII, VPM VII and VHCPM II expressly disclaim beneficial ownership over all shares held by VA VII, VP VII, VHCP II and VHCP Co. II, except to the extent of their indirect pecuniary interest therein. Anders D. Hove and Bryan E. Roberts are members of VA VII, VP VII and VHCPM II and disclaim beneficial ownership over all shares held by VA VII, VP VII, VHCP II and VHCP Co. II, except to the extent of their indirect pecuniary interests therein.

(4) Consists of 1,759,961 shares of common stock issuable upon conversion of preferred stock. Brookside Capital Management, LLC, the sole general partner of Brookside Capital Investors, L.P., which is the sole general partner of Brookside Capital Partners Fund, L.P., has voting and dispositive power with respect to the shares.

(5) Consists of 1,169,042 shares of common stock issuable upon conversion of preferred stock. The shares directly held by Deerfield Private Design Fund III, L.P. are indirectly beneficially owned by Deerfield Mgmt III, L.P., its general partner, Deerfield Management Company, L.P., its investment manager, and James E. Flynn. As the sole member of the respective general partners of Deerfield Mgmt III, L.P. and Deerfield Management Company, L.P., Mr. Flynn is the sole natural person possessing beneficial ownership over such shares.

(6) Consists of 1,085,816 shares of common stock issuable upon conversion of preferred stock. Beacon Bioventures Advisors Fund III Limited Partnership (Advisors Fund) is the general partner of Beacon Bioventures Fund III Limited Partnership (Beacon Fund). Advisors Fund is solely managed by Impresa Management LLC (Impresa), its general partner and investment manager. Impresa is owned by the shareholders and certain employees of FMR LLC. Impresa is managed on a day-to-day basis by its President, Paul L. Mucci, and as such Mr. Mucci may be deemed to share voting and dispositive power with respect to all shares held by Beacon Fund. Each of the individuals and entities listed above expressly disclaims beneficial ownership of the securities listed above except to the extent of any pecuniary interest therein. We have entered into a letter agreement (the Voting Rights Waiver) with Beacon Fund pursuant to which Beacon Fund agreed to waive all voting rights that it may have in respect of any voting securities issued by us that exceed, in the aggregate, 4.99% of the total voting rights exercisable by our outstanding voting securities.

(7) GlaxoSmithKline plc has sole voting and dispositive power over the shares held by GlaxoSmithKline LLC.

(8) Consists of 15,440 shares of common stock issuable upon conversion of preferred stock and includes options to purchase 311,165 shares of common stock that may be exercised within 60 days of August 1, 2015.

(9) Consists of 115,440 shares of common stock and common stock issuable upon conversion of preferred stock and includes options to purchase 161,691 shares of common stock that may be exercised within 60 days of August 1, 2015.

(10) Includes options to purchase 36,005 shares of common stock that may be exercised within 60 days of August 1, 2015.
(11) Consists of 38,599 shares of common stock issuable upon conversion of preferred stock and includes options to purchase 155,578 shares of common stock that may be exercised within 60 days of August 1, 2015.
(12) Dr. Engleman is affiliated with Vivo Capital Fund VIII, L.P. (Vivo Fund VIII) and Vivo Capital Surplus Fund VIII, L.P. (Vivo Surplus VIII). Vivo Fund VIII's ownership consists of 826,341 shares of common stock issuable upon conversion of preferred stock and Vivo Surplus VIII's ownership consists of 114,108 shares of common stock issuable upon conversion of preferred stock. Vivo Capital VIII, LLC, the sole general partner of both Vivo Fund VIII and Vivo Surplus VIII, has shared voting power and shared investment power over such securities, may be deemed to beneficially own such shares, and disclaims beneficial ownership of the shares except to the extent of its pecuniary interests therein. Dr. Engleman disclaims beneficial ownership of the shares held by Vivo Fund VIII and Vivo Surplus Fund, except to the extent of his pecuniary interest therein.
(13) Ms. Samuels is affiliated with Venrock Partners. Ms. Samuels disclaims beneficial ownership of the shares held by the entities affiliated with Venrock Partners referenced in footnote 3 above, except to the extent of her pecuniary interest therein.
(14) Consists of 6,147,883 shares of common stock and common stock issuable upon conversion of preferred stock and includes options to purchase 664,439 shares of common stock that may be exercised within 60 days of August 1, 2015. Mr. Fox holds shared dispositive power over certain shares as described in footnote 1 above and these shares are only counted once for the purpose of this calculation.

DESCRIPTION OF CAPITAL STOCK

General

Following the closing of this offering, our authorized capital stock will consist of 100,000,000 shares of common stock, par value $0.0001 per share, and 10,000,000 shares of preferred stock, par value $0.0001 per share. The following description summarizes some of the terms of our restated certificate of incorporation and amended and restated bylaws. This description does not purport to be complete and is qualified in its entirety by the provisions of our certificate of incorporation and bylaws, copies of which have been filed as exhibits to the registration statement of which this prospectus is a part.

Common Stock

As of June 30, 2015, there were 19,050,708 shares of our common stock outstanding, held of record by 55 stockholders, assuming conversion of all outstanding shares of our preferred stock into shares of common stock immediately prior to the closing of this offering.

Voting Rights

Each holder of common stock is entitled to one vote for each share of common stock held on all matters submitted to a vote of the stockholders, including the election of directors. Our restated certificate of incorporation and bylaws do not provide for cumulative voting rights. Because of this, the holders of a majority of the shares of common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they should so choose.

Dividends

Subject to preferences that may be applicable to any then outstanding preferred stock, the holders of our outstanding shares of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally available funds. At present, we have no plans to issue dividends. See the section titled “Dividend Policy” above.

Liquidation

In the event of our liquidation, dissolution or winding up, holders of common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

Other Rights and Preferences

Holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

Fully Paid and Nonassessable

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

Preferred Stock

Upon the closing of this offering, we will have no shares of our preferred stock outstanding. Outstanding shares of Series A Preferred Stock will be converted into 2,393,127 shares of common stock, outstanding shares of Series B Preferred Stock will be converted into 1,906,295 shares of common stock, outstanding shares of Series C Preferred Stock will be converted into 4,631,774 shares of common stock, and outstanding shares of Series D Preferred Stock will be converted into 7,366,849 shares of common stock immediately prior to the closing of this offering.

Under the terms of our restated certificate of incorporation, to be effective at the completion of this offering, our board of directors will be authorized to issue preferred stock in one or more series, to establish the number of shares to be included in each such series and to fix the designation, powers, preferences and rights of such shares and any qualifications, limitations or restrictions thereof. The issuance of preferred stock may have the effect of delaying, deferring or preventing a change in control of our company without further action by the stockholders and may adversely affect the voting and other rights of the holders of common stock. The issuance of preferred stock with voting and conversion rights may adversely affect the voting power of the holders of common stock, including the loss of voting control to others. At present, we have no plans to issue any preferred stock.

Options

As of June 30, 2015, options to purchase 3,063,200 shares of our common stock were outstanding under our 2014 Plan at a weighted-average exercise price of $1.86 per share, of which 404,287 were vested and exercisable as of that date.

Registration Rights

Demand Registration Rights

Pursuant to the Investors' Rights Agreement, at any time after the earlier of (i) May 15, 2020 or (ii) six months after the effective date of this offering, the holders of at least 50% of the registrable shares of our common stock issued or issuable upon conversion of our preferred stock can request that we file up to two registration statements with an anticipated aggregate offering price of at least $30.0 million in each instance registering all or a portion of their registrable shares. As of June 30, 2015, the holders of 16,298,045 shares of our common stock, including shares issuable upon the automatic conversion of our preferred stock, have demand registration rights. Under specified circumstances, we also have the right to defer filing of a requested registration statement for a period of not more than 90 days, which right may not be exercised more than once during any period of 12 consecutive months. These registration rights are subject to additional conditions and limitations, including the right of the underwriters to limit the number of shares included in any such registration under certain circumstances.

Form S-3 Registration Rights

Pursuant to the Investors' Rights Agreement, if we are eligible to file a registration statement on Form S-3, the holders of at least 30% of the registrable shares of common stock issued or issuable upon the conversion of preferred stock have the right to demand that we file additional registration statements, including a shelf registration statement, for such holders on Form S-3. Such right is limited to two such demands within any 12 month period.

Piggyback Registration Rights

Pursuant to the Investors' Rights Agreement, whenever we propose to file a registration statement under the Securities Act, other than with respect to a registration related to employee benefit or similar plans, a registration

on any form which does not include substantially the same information as would be required to be included in this registration statement, or a registration in which the only common stock being registered is common stock issuable upon conversion of debt securities which are also being registered, the holders of registrable shares of common stock issued or issuable upon conversion of our convertible preferred stock are entitled to notice of the registration and have the right to include their registrable shares in such registration. As of June 30, 2015, the holders of 16,298,045 shares of our common stock, including shares issuable upon the automatic conversion of our Preferred Stock, will be entitled to notice of this registration and will be entitled to include their shares of common stock in the registration statement but we anticipate that such right will be waived prior to this offering. The underwriters of any underwritten offering will have the right to limit the number of shares having registration rights to be included in the registration statement.

Expenses of Registration

We are required to pay all expenses relating to any demand, Form S-3 or piggyback registration, other than underwriting discounts and commissions, subject to certain limited exceptions. We will not pay for any expenses of any demand registration if the request is subsequently withdrawn by the holders of a majority of the shares requested to be included in such a registration statement, subject to limited exceptions.

Expiration of Registration Rights

The registration rights described above will expire for each holder upon the earlier of (i) five years after this offering is completed and (ii) the closing of a deemed liquidation event as defined in our restated certificate of incorporation.

Other Stockholder Rights

The First Refusal Agreement provides certain rights of first refusal and co-sale rights to certain of our stockholders. The First Refusal Agreement will terminate upon the completion of this offering.

Anti-Takeover Effects of Delaware Law and Our Restated Certificate of Incorporation and Amended and Restated Bylaws

Delaware law, our restated certificate of incorporation and our amended and restated bylaws contain provisions that could make the following transactions more difficult: an acquisition of us by means of a tender offer; an acquisition of us by means of a proxy contest or otherwise; or the removal of our incumbent officers and directors. It is possible that these provisions could make it more difficult to accomplish or could deter transactions that stockholders may otherwise consider to be in their best interest or in our best interests, including transactions which provide for payment of a premium over the market price for our shares.

These provisions, summarized below, are intended to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors. We believe that the benefits of the increased protection of our potential ability to negotiate with the proponent of an unfriendly or unsolicited proposal to acquire or restructure us outweigh the disadvantages of discouraging these proposals because negotiation of these proposals could result in an improvement of their terms.

Undesignated Preferred Stock

The ability of our board of directors, without action by the stockholders, to issue up to 10,000,000 shares of undesignated preferred stock with voting or other rights or preferences as designated by our board of directors could impede the success of any attempt to change control of us. The existence of authorized but unissued shares of preferred stock may have the effect of deferring hostile takeovers or delaying changes in control or management of our company.

Stockholder Action by Written Consent; Stockholder Meetings

Our restated certificate of incorporation and amended and restated bylaws eliminate the right of stockholders to act by written consent without a meeting. As a result, a holder controlling a majority of our capital stock would not be able to amend our bylaws or remove directors without holding a meeting of our stockholders called in accordance with our bylaws. Our amended and restated bylaws provide that a special meeting of stockholders may be called only by our chairman of the board, chief executive officer or president, or by a resolution adopted by a majority of our board of directors. These provisions might delay the ability of our stockholders to force consideration of a proposal or for stockholders controlling a majority of our capital stock to take any action, including the removal of directors.

Requirements for Advance Notification of Stockholder Nominations and Proposals

Our amended and restated bylaws establish advance notice procedures with respect to stockholder proposals to be brought before a stockholder meeting and the nomination of candidates for election as directors, other than nominations made by or at the direction of the board of directors or a committee of the board of directors. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Staggered Board

Our board of directors is divided into three classes. The directors in each class will serve for a three-year term, one class being elected each year by our stockholders. For more information on the classified board, see “Management—Board of Directors—Classified Board.” This system of electing and removing directors may discourage a third-party from making a tender offer or otherwise attempting to obtain control of us, because it generally makes it more difficult for stockholders to replace a majority of the directors.

Removal of Directors

Our restated certificate of incorporation provides that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of holders of at least two-thirds of the total voting power of all of our outstanding voting stock then entitled to vote in the election of directors.

Board of Directors Vacancies

Our restated certificate of incorporation and amended and restated bylaws authorize our board of directors to fill vacant directorships. In addition, the number of directors constituting our board of directors is set only by resolution adopted by a majority vote of our entire board of directors. These provisions will prevent a stockholder from increasing the size of our board of directors and gaining control of our board of directors by filling the resulting vacancies with its own nominees.

Stockholders Not Entitled to Cumulative Voting

Our restated certificate of incorporation does not permit stockholders to cumulate their votes in the election of directors. Accordingly, the holders of a majority of the outstanding shares of our common stock entitled to vote in any election of directors can elect all of the directors standing for election, if they choose, other than any directors that holders of our preferred stock may be entitled to elect.

Amendment of Charter Provisions

The amendment of any of the above provisions, except for the provision making it possible for our board of directors to issue preferred stock, would require approval by holders of at least two-thirds of the total voting power of all of our outstanding voting stock.

The provisions of Delaware law, our restated certificate of incorporation and our amended and restated bylaws could have the effect of discouraging others from attempting hostile takeovers and, as a consequence, they may also inhibit temporary fluctuations in the market price of our common stock that often result from actual or rumored hostile takeover attempts. These provisions may also have the effect of preventing changes in the composition of our board and management. It is possible that these provisions could make it more difficult to accomplish transactions that stockholders may otherwise deem to be in their best interests.

Choice of Forum

Upon the completion of this offering, our restated certificate of incorporation will provide that the Court of Chancery of the State of Delaware will be the exclusive forum for any derivative action or proceeding brought on our behalf; any action asserting a breach of fiduciary duty; any action asserting a claim against us arising pursuant to the Delaware General Corporation Law, our restated certificate of incorporation or amended and restated bylaws; or any action asserting a claim against us that is governed by the internal affairs doctrine. The enforceability of similar choice of forum provisions in other companies' certificates of incorporation has been challenged in legal proceedings, and it is possible that a court could find these types of provisions to be inapplicable or unenforceable.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be Computershare Trust Company, N.A. The transfer agent and registrar's address is 250 Royall Street, Canton, Massachusetts 02021.

NASDAQ Global Market

We have applied to list our common stock on the NASDAQ Global Market under the symbol "RGNX."

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no market for our common stock. Future sales of substantial amounts of our common stock in the public market could adversely affect market prices prevailing from time to time. Furthermore, because only a limited number of shares will be available for sale shortly after this offering due to existing contractual and legal restrictions on resale as described below, there may be sales of substantial amounts of our common stock in the public market after the restrictions lapse. This may adversely affect the prevailing market price and our ability to raise equity capital in the future.

Upon completion of this offering, we will have shares of common stock outstanding assuming no exercise of the underwriters' option to purchase additional shares, the conversion of all outstanding shares of preferred stock and no exercise of outstanding options or warrants after June 30, 2015. Of these shares, the shares (shares if the underwriters exercise their option to purchase additional shares in full) sold in this offering will be freely transferable without restriction or registration under the Securities Act, except for any shares purchased by one of our existing "affiliates," as that term is defined in Rule 144 under the Securities Act. The remaining shares of common stock existing are "restricted shares" as defined in Rule 144. Restricted shares may be sold in the public market only if registered or if they qualify for an exemption from registration under Rules 144 or 701 of the Securities Act. As a result of the contractual 180-day lock-up period described below and the provisions of Rules 144 and 701, these shares will be available for sale in the public market as follows:

- no restricted shares will be eligible for sale in the public market immediately upon completion of this offering; and
- 19,050,708 shares will be eligible for sale in the public market beginning 180 days from the date of this prospectus (subject, in some cases, to volume limitations), upon the expiration of the 180-day lock-up and/or market standoff agreements entered into prior to our initial public offering and the lapse of our right of repurchase with respect to any unvested shares, if applicable.

Lock-up Agreements

We and all directors and officers and the holders of 19,060,858 shares of our outstanding stock, who collectively own 100.0% of our common stock, based on 19,060,858 shares outstanding as of August 1, 2015, have agreed that, without the prior written consent of Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated on behalf of the underwriters, we and they will not, during the period ending 180 days after the date of this prospectus:

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock;
- file any registration statement with the Securities and Exchange Commission relating to the offering of any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock; or
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock.

whether any such transaction described above is to be settled by delivery of common stock or such other securities, in cash or otherwise. In addition, we and each such person agrees that, without the prior written consent of Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated on behalf of the underwriters, we or such other person will not, during such 180-day period, make any demand for, or exercise any right with respect to, the registration of any shares of common stock or any security convertible into or exercisable or exchangeable for common stock.

The lock-up restrictions and specified exceptions are described in more detail under "Underwriters."

Rule 144

In general, a person who has beneficially owned our restricted common shares for at least six months would be entitled to sell their securities provided that (1) such person is not deemed to have been one of our affiliates at the time of, or at any time during the 90 days preceding, a sale, and (2) we are subject to the Securities Exchange Act of 1934 periodic reporting requirements for at least 90 days before the sale. In addition, under Rule 144, any person who is not an affiliate of ours and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available. Persons who have beneficially owned restricted common shares for at least six months but who are our affiliates at the time of, or any time during the 90 days preceding, a sale, would be subject to additional restrictions, by which such person would be entitled to sell within any three-month period only a number of securities that does not exceed the greater of either of the following:

- one percent of the number of common shares then outstanding, which will equal approximately shares immediately after this offering assuming no exercise of the underwriters' option to purchase additional shares, based on the number of common shares outstanding as of June 30, 2015; or
- the average weekly trading volume of our common shares during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale;

provided, in each case, that we are subject to the Securities Exchange Act of 1934 periodic reporting requirements for at least 90 days before the sale. Such sales both by affiliates and by non-affiliates must also comply with the manner of sale, current public information and notice provisions of Rule 144.

Rule 701

Rule 701, as currently in effect, permits resales of shares in reliance upon Rule 144 but without compliance with certain restrictions, including the holding period requirement, of Rule 144. Any employee, officer or director of or consultant to us who purchased shares under a written compensatory plan or contract may be entitled to rely on the resale provisions of Rule 701. Rule 701 permits affiliates to sell their Rule 701 shares under Rule 144 without complying with the holding period requirements of Rule 144. Rule 701 further provides that non-affiliates may sell such shares in reliance on Rule 144 without having to comply with the holding period, public information, volume limitation or notice provisions of Rule 144. All holders of Rule 701 shares are required to wait until 90 days after the date of this prospectus before selling such shares. All Rule 701 shares are, however, subject to lock-up agreements and will only become eligible for sale upon the expiration of these lock-up agreements.

Registration Rights

Upon completion of this offering, the holders of 16,298,045 shares of our common stock have the right to have their shares registered under the Securities Act. See the "Description of Capital Stock—Registration Rights" section of this prospectus. All such shares are covered by lock-up agreements. Following the expiration of the lock-up period, registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act immediately upon the effectiveness of the registration, except for shares purchased by our affiliates.

Form S-8 Registration Statements

Prior to the expiration of the lock-up period, we intend to file one or more registration statements on Form S-8 under the Securities Act to register the shares of our common stock that are issuable pursuant to our 2014 Stock Plan, 2015 Equity Incentive Plan and 2015 Employee Stock Purchase Plan. See the "Management —Stock-based Compensation Plans" section of this prospectus. Subject to the lock-up agreements described above and any applicable vesting restrictions, shares registered under these registration statements will be available for resale in the public market immediately upon the effectiveness of these registration statements, except with respect to Rule 144 volume limitations that apply to our affiliates.

MATERIAL UNITED STATES FEDERAL INCOME TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following is a discussion of the material United States federal income tax considerations with respect to the ownership and disposition of shares of common stock applicable to non-U.S. holders who acquire such shares in this offering, hold such shares as a capital asset within the meaning of Section 1221 of the Internal Revenue Code of 1986, as amended (the Code) (generally, property held for investment) and do not own and have not owned, actually or constructively, more than five percent of our common stock. For purposes of this discussion, a "non-U.S. holder" means a beneficial owner of our common stock (other than an entity or arrangement that is treated as a partnership for U.S. federal income tax purposes) that is not, for U.S. federal income tax purposes, any of the following:

- a citizen or resident of the United States;
- a corporation (or other entity treated as a corporation for U.S. federal income tax purposes) created or organized in the United States or under the laws of the United States, any state thereof or the District of Columbia, or any other corporation treated as such;
- an estate, the income of which is includable in gross income for U.S. federal income tax purposes regardless of its source; or
- a trust if (i) a court within the United States is able to exercise primary supervision over the administration of the trust and one or more "U.S. persons," as defined under the Code, have the authority to control all substantial decisions of the trust or (ii) such trust has made a valid election to be treated as a U.S. person for U.S. federal income tax purposes.

This discussion is based on current provisions of the Code, Treasury regulations promulgated thereunder, judicial opinions, published positions of the Internal Revenue Service and other applicable authorities, all of which are subject to change (possibly with retroactive effect). This discussion does not address all aspects of U.S. federal income taxation that may be important to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances, nor does it address any aspects of the unearned income Medicare contribution tax pursuant to the Health Care and Education Reconciliation Act of 2010, any U.S. federal estate and gift taxes, any U.S. alternative minimum taxes or any state, local or non-U.S. taxes. This discussion may not apply, in whole or in part, to particular non-U.S. holders in light of their individual circumstances or to holders subject to special treatment under the United States federal income tax laws (such as insurance companies, tax-exempt organizations, financial institutions, brokers or dealers in securities, "controlled foreign corporations," "passive foreign investment companies," non-U.S. holders that hold our common stock as part of a straddle, hedge, conversion transaction or other integrated investment and certain U.S. expatriates).

If a partnership (or other entity or arrangement treated as a partnership for U.S. federal income tax purposes) holds our common stock, the tax treatment of a partner therein will generally depend on the status of the partner and the activities of the partnership. Partners of a partnership holding our common stock should consult their tax advisor as to the particular U.S. federal income tax consequences applicable to them.

THIS SUMMARY IS FOR GENERAL INFORMATION ONLY AND IS NOT INTENDED TO CONSTITUTE A COMPLETE DESCRIPTION OF ALL TAX CONSEQUENCES FOR NON-U.S. HOLDERS RELATING TO THE OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK. PROSPECTIVE HOLDERS OF OUR COMMON STOCK SHOULD CONSULT WITH THEIR TAX ADVISORS REGARDING THE TAX CONSEQUENCES TO THEM (INCLUDING THE APPLICATION AND EFFECT OF ANY STATE, LOCAL, NON-U.S. INCOME AND OTHER TAX LAWS) OF THE OWNERSHIP AND DISPOSITION OF OUR COMMON STOCK.

Dividends

In general, the gross amount of any distribution we make to a non-U.S. holder with respect to its shares of common stock will be subject to U.S. withholding tax at a rate of 30% to the extent the distribution constitutes a dividend for U.S. federal income tax purposes, unless the non-U.S. holder is eligible for a reduced rate of withholding tax under an applicable tax treaty and the non-U.S. holder provides proper certification of its eligibility for such reduced rate (generally an applicable IRS Form W-8). A distribution will constitute a dividend for U.S. federal income tax purposes to the extent of our current or accumulated earnings and profits as determined for U.S. federal income tax purposes. To the extent any distribution does not constitute a dividend, it will be treated first as reducing the adjusted basis in the non-U.S. holder's shares of common stock and then, to the extent it exceeds the adjusted basis in the non-U.S. holder's shares of common stock, as gain from the sale or exchange of such stock. Any such gain will be subject to the treatment described below under "—Gain on Sale or Other Disposition of Common Stock."

Dividends we pay to a non-U.S. holder that are effectively connected with its conduct of a trade or business within the United States (and, if required by an applicable tax treaty, are attributable to a U.S. permanent establishment of such non-U.S. holder) will not be subject to U.S. withholding tax, as described above, if the non-U.S. holder complies with applicable certification and disclosure requirements (including a properly executed IRS Form W-8ECI). Instead, such dividends generally will be subject to U.S. federal income tax on a net income basis, at regular U.S. federal income tax rates. Dividends received by a non-U.S. corporation that are effectively connected with its conduct of trade or business within the United States may be subject to an additional branch profits tax at a rate of 30% (or such lower rate as may be specified by an applicable tax treaty).

Gain on Sale or Other Disposition of Common Stock

Subject to the discussions below, a non-U.S. holder generally will not be subject to U.S. federal income tax on any gain realized upon the sale or other disposition of the non-U.S. holder's shares of common stock unless:

- the gain is effectively connected with a trade or business carried on by the non-U.S. holder within the United States (and, if required by an applicable tax treaty, is attributable to a U.S. permanent establishment of such non-U.S. holder);
- the non-U.S. holder is an individual and is present in the United States for 183 days or more in the taxable year of disposition and certain other conditions are met; or
- we are or have been a U.S. real property holding corporation for U.S. federal income tax purposes at any time within the shorter of the five-year period preceding such disposition or such non-U.S. holder's holding period of our common stock, and the non-U.S. holder has held, at any time during said period, more than five percent of the class of our stock being sold.

Gain that is effectively connected with the conduct of a trade or business in the United States (or so treated) generally will be subject to U.S. federal income tax on a net income tax basis, at regular U.S. federal income tax rates. If the non-U.S. holder is a non-U.S. corporation, the branch profits tax described above also may apply to such effectively connected gain. An individual non-U.S. holder who is subject to U.S. federal income tax because the non-U.S. holder was present in the United States for 183 days or more during the year of sale or other disposition of our common stock will be subject to a flat 30% tax on the gain derived from such sale or other disposition, which may be offset by U.S. source capital losses. We believe that we are not and we do not anticipate becoming a U.S. real property holding corporation for U.S. federal income tax purposes.

Withholdable Payments to Foreign Financial Institutions and Other Non-U.S. Entities

The Foreign Account Tax Compliance Act (FATCA), will impose a U.S. federal withholding tax of 30% on certain payments to foreign financial institutions, investment funds and certain other non-U.S. persons that fail to comply with certain information reporting and certification requirements pertaining to their direct and indirect U.S. securityholders and/or U.S. accountholders. Such payments would include our dividends and the gross proceeds from the sale or other disposition of our common stock. Under applicable Treasury Regulations, this withholding will apply to payments of dividends on our common stock and to payments of gross proceeds from a sale or other disposition of our common stock made on or after January 1, 2017. If FATCA withholding is imposed, a beneficial owner that is not a foreign financial institution generally may obtain a refund of any amounts withheld by filing a U.S. federal income tax return (which may entail significant administrative burden). Prospective investors are encouraged to consult with their own tax advisors regarding the possible implications of this legislation on their investment in our common stock.

Backup Withholding, Information Reporting and Other Reporting Requirements

We must report annually to the Internal Revenue Service and to each non-U.S. holder the amount of dividends paid to, and the tax withheld with respect to, each non-U.S. holder. These reporting requirements apply regardless of whether withholding was reduced or eliminated by an applicable tax treaty. Copies of this information reporting may also be made available under the provisions of a specific tax treaty or agreement with the tax authorities in the country in which the non-U.S. holder resides or is established.

A non-U.S. holder will generally be subject to backup withholding for dividends on our common stock paid to such holder unless such holder certifies under penalties of perjury (generally by providing an applicable IRS Form W-8) that, among other things, it is a non-U.S. holder (and the payor does not have actual knowledge or reason to know that such holder is a U.S. person) or otherwise establishes an exemption.

Information reporting and backup withholding generally are not required with respect to the amount of any proceeds from the sale or other disposition of our common stock by a non-U.S. holder outside the United States through an office outside the United States of a non-U.S. broker that does not have certain specified connections to the United States. However, if a non-U.S. holder sells or otherwise disposes of its shares of common stock through a U.S. broker or the United States offices of a non-U.S. broker, the broker will generally be required to report the amount of proceeds paid to the non-U.S. holder to the Internal Revenue Service and also backup withhold on that amount unless such non-U.S. holder provides appropriate certification to the broker of its status as a non-U.S. person (and the payor does not have actual knowledge or reason to know that such holder is a U.S. person) or otherwise establishes an exemption. Information reporting will also apply if a non-U.S. holder sells its shares of common stock through a non-U.S. broker deriving more than a specified percentage of its income from U.S. sources or having certain other connections to the United States, unless such broker has documentary evidence in its records that such non-U.S. holder is a non-U.S. person (and the payor does not have actual knowledge or reason to know that such holder is a U.S. person) and certain other conditions are met, or such non-U.S. holder otherwise establishes an exemption.

Backup withholding is not an additional income tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder generally can be credited against the non-U.S. holder’s U.S. federal income tax liability, if any, or refunded, provided that the required information is furnished to the Internal Revenue Service in a timely manner. Non-U.S. holders should consult their tax advisors regarding the application of the information reporting and backup withholding rules to them.

UNDERWRITING

Under the terms and subject to the conditions in an underwriting agreement dated the date of this prospectus, the underwriters named below, for whom Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated are acting as representatives, have severally agreed to purchase, and we have agreed to sell to them, severally, the number of shares indicated below:

Name	Number of Shares
Morgan Stanley & Co. LLC	
Merrill Lynch, Pierce, Fenner & Smith Incorporated	
Piper Jaffray & Co.	
Chardan Capital Markets, LLC	
Total:	

The underwriters and the representatives are collectively referred to as the “underwriters” and the “representatives,” respectively. The underwriters are offering the shares of common stock subject to their acceptance of the shares from us and subject to prior sale. The underwriting agreement provides that the obligations of the several underwriters to pay for and accept delivery of the shares of common stock offered by this prospectus are subject to the approval of certain legal matters by their counsel and to certain other conditions. The underwriters are obligated to take and pay for all of the shares of common stock offered by this prospectus if any such shares are taken. However, the underwriters are not required to take or pay for the shares covered by the underwriters’ option to purchase additional shares described below.

The underwriters initially propose to offer part of the shares of common stock directly to the public at the offering price listed on the cover page of this prospectus and part to certain dealers. After the initial offering of the shares of common stock, the offering price and other selling terms may from time to time be varied by the representatives.

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to additional shares of common stock at the public offering price listed on the cover page of this prospectus, less underwriting discounts and commissions. To the extent the option is exercised, each underwriter will become obligated, subject to certain conditions, to purchase about the same percentage of the additional shares of common stock as the number listed next to the underwriter’s name in the preceding table bears to the total number of shares of common stock listed next to the names of all underwriters in the preceding table.

The following table shows the per share and total public offering price, underwriting discounts and commissions, and proceeds before expenses to us. These amounts are shown assuming both no exercise and full exercise of the underwriters’ option to purchase up to an additional shares of common stock.

	Per Share	Total	
		No Exercise	Full Exercise
Public offering price	$	$	$
Underwriting discounts and commissions to be paid by us	$	$	$
Proceeds, before expenses, to us	$	$	$

The estimated offering expenses payable by us, exclusive of the underwriting discounts and commissions, are approximately $. We have agreed to reimburse the underwriters for expense relating to clearance of this offering with the Financial Industry Regulatory Authority up to $.

The underwriters have informed us that they do not intend sales to discretionary accounts to exceed five percent of the total number of shares of common stock offered by them.

We have applied to have our common stock approved for quotation on the NASDAQ Global Market under the trading symbol “RGNX”.

We and all directors and officers and the holders of all shares of our outstanding common stock have agreed that, without the prior written consent of Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated on behalf of the underwriters, we and they will not, during the period ending 180 days after the date of this prospectus (the restricted period):

- offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock;
- file any registration statement with the Securities and Exchange Commission relating to the offering of any shares of common stock or any securities convertible into or exercisable or exchangeable for common stock; or
- enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock.

whether any such transaction described above is to be settled by delivery of common stock or such other securities, in cash or otherwise. In addition, we and each such person agrees that, without the prior written consent of Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated on behalf of the underwriters, we or such other person will not, during the restricted period, make any demand for, or exercise any right with respect to, the registration of any shares of common stock or any security convertible into or exercisable or exchangeable for common stock.

The restrictions described in the immediately preceding paragraph relating to our directors and officers and our shareholders do not apply to:

- transfers or dispositions of shares of common stock or any security convertible into or exercisable or exchangeable for common stock as a bona fide gift including to a charitable organization;
- transfers or distributions of shares of common stock or any security convertible into or exercisable or exchangeable for common stock to limited partners, general partners, managers, directors, officers, employees, members, stockholders or trust beneficiaries or to any controlled investment fund or other entity, including transfers or distributions of shares to a fund managed by the same manager or managing member or general partner or management company or by an entity controlling, controlled by, or under common control with such manager or managing member or general partner or managing company;
- transfers or dispositions of shares of common stock or any security convertible into common stock by will or other testamentary document or by intestacy;
- transfers or dispositions of shares of common stock or any security convertible into or exercisable or exchangeable for common stock to any trust for the direct or indirect benefit of immediate family members in a transaction not involving a disposition for value;
- transfers or dispositions of common stock acquired in this offering or acquired in open market transactions after this offering;
- the exercise of options to purchase shares of common stock granted under a stock incentive plan or stock purchase plan described in this prospectus or the exercise of warrants to purchase shares of common stock (or any security convertible into or exercisable or exchangeable for common stock) described in this prospectus and outstanding as of the date of this prospectus, provided that the underlying common stock continues to be subject to the restrictions set forth above;

- the exercise of options to purchase shares of common stock granted under a stock incentive plan or stock purchase plan described in this prospectus pursuant to an arrangement whereby we withhold shares issuable pursuant to such option in payment of the exercise price, provided that the underlying common stock continues to be subject to the restrictions set forth above;
- the transfer of common stock or any security convertible into or exchangeable for common stock that occurs by operation of law pursuant to a qualified domestic order or in connection with a divorce settlement or other court order;
- transfers or dispositions of common stock or any security convertible into or exercisable or exchangeable for common stock to us pursuant to any contractual arrangement that provides for the repurchase of common stock or such other securities by us or to us in connection with the termination of employment with us, provided that the repurchase price for any such shares or securities shall not exceed the original purchase price (subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization) paid to us for such shares or securities;
- transfers by an investment company registered under the Investment Company Act of 1940, as amended, pursuant to a merger or reorganization with or into another such investment company that shares the same investment adviser;
- the establishment of a trading plan that satisfies the requirements of Rule 10b5-1 under the Exchange Act for the transfer of shares of common stock, provided that (i) such plan does not provide for the transfer of common stock during the restricted period and (ii) to the extent a public announcement or filing under the Exchange Act, if any, is required or voluntarily made regarding the establishment of such plan, such announcement or filing shall include a statement to the effect that no transfer of common stock may be made under such plan during the restricted period;
- the transfer to us of common stock upon a vesting event or upon the exercise of options or warrants to purchase common stock, in each case on a “cashless” or “net exercise” basis or to cover tax withholding obligations in connection with such vesting or exercise, provided that no filing under Section 16(a) of the Exchange Act, reporting a reduction in beneficial ownership of common stock, shall be required or shall be voluntarily made; and
- transfers in connection with a bona fide third party tender offer, merger, consolidation or other similar transaction that is approved by our board of directors, made to all holders of our common stock involving a change of control occurring after the closing of this offering, provided that in the event that the tender offer, merger, consolidation or other such transaction is not completed, the common stock shall remain subject to the restrictions in the immediately preceding paragraph;

provided further that (i) in the case of any transfer or distribution as described in the first, second, third, fourth, eighth or tenth bullet point above, the recipient shall agree to be subject to the restrictions described in the immediately preceding paragraph and (ii) in the case of any transfer or distribution described in the first, second, fourth, fifth, seventh, eighth, ninth or tenth bullet point above, no filing under Section 16(a) of the Exchange Act, reporting a reduction in beneficial ownership of shares of common stock, shall be required or shall be voluntarily made during the restricted period.

In addition, the restrictions described in the paragraph above relating to us do not apply to:

- the shares to be sold in this offering;
- our issuance of shares of common stock or securities convertible into or exercisable for shares of common stock upon the exercise of an option or warrant or the conversion of a security outstanding on the date of the purchase agreement and disclosed in this prospectus;

- our issuance of shares of common stock or other securities convertible into or exercisable for shares of common stock pursuant to our equity incentive plans described in this prospectus, provided that, prior to the issuance of any such shares of common stock or other securities where the shares of common stock or other securities vest within the restricted period, we shall cause each recipient of such grant or issuance to execute a lock-up agreement; and
- the entry into an agreement providing for the issuance of shares of common stock or any security convertible into or exercisable for shares of common stock in connection with joint ventures, commercial relationships or other strategic transactions, and the issuance of any such securities pursuant to any such agreement, provided that the aggregate number of shares of common stock that we may sell or issue or agree to sell or issue, or that may be issuable upon conversion or exercise of all other securities that we may sell or issue or agree to sell or issue, pursuant to this exception shall not exceed 5% of the total number of shares of common stock issued and outstanding immediately following the completion of this offering, and provided further, that each recipient of shares or other securities issued pursuant to this exception shall be subject to the restrictions described in the paragraph above.

Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated, in their sole discretion, may release the common stock and other securities subject to the lock-up agreements described above in whole or in part at any time with or without notice. When determining whether or not to release common stock and other securities from lock-up agreements, Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated will consider, among other factors, the holder's reasons for requesting the release, the number of shares of common stock and other securities for which the release is being requested and market conditions at the time. In addition, in the event that any of our executive officers or directors or persons that held at least 1% of our common stock as of the date of this prospectus is granted an early release from the lock-up restrictions with respect to shares of our common stock or any security convertible into or exercisable or exchangeable for our common stock having a fair market value in excess of $5.0 million in the aggregate (whether in one or multiple releases), then each signatory to the Investors Rights Agreement (each, an IRA Signatory) automatically will be granted an equivalent early release from its obligations under the lock-up agreement on a pro-rata basis (a Pro-Rata Release). Such Pro-Rata Release shall not be applicable in the event of any underwritten primary or secondary public offering or sale of our common stock during the period ending 180 days after the date of this prospectus; provided, however, that each IRA Signatory is given an opportunity to participate on a pro-rata basis in such underwritten primary or secondary public offering with and otherwise pursuant to the same terms and conditions as any other IRA Signatory. Notwithstanding any other provisions of the lock-up agreement, if Morgan Stanley & Co. LLC and Merrill Lynch, Pierce, Fenner & Smith Incorporated in their sole judgment determine that a holder or holders of shares of common stock should be granted an early release from a lock-up agreement due to circumstances of an emergency or hardship, then the IRA Signatories shall not have any right to a Pro-Rata Release if the aggregate number of shares of common stock released due to an emergency or hardship does not exceed 50,000 shares.

In order to facilitate the offering of the common stock, the underwriters may engage in transactions that stabilize, maintain or otherwise affect the price of the common stock. Specifically, the underwriters may sell more shares than they are obligated to purchase under the underwriting agreement, creating a short position. A short sale is covered if the short position is no greater than the number of shares available for purchase by the underwriters under the option. The underwriters can close out a covered short sale by exercising the option or purchasing shares in the open market. In determining the source of shares to close out a covered short sale, the underwriters will consider, among other things, the open market price of shares compared to the price available under the option. The underwriters may also sell shares in excess of the option, creating a naked short position. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the common stock in the open market after pricing that could adversely affect investors who purchase in this offering. As an additional means of facilitating this offering, the underwriters may bid for, and purchase, shares of common stock in the open market to stabilize the price of the common stock. These activities may raise

or maintain the market price of the common stock above independent market levels or prevent or retard a decline in the market price of the common stock. The underwriters are not required to engage in these activities and may end any of these activities at any time.

We and the underwriters have agreed to indemnify each other against certain liabilities, including liabilities under the Securities Act.

A prospectus in electronic format may be made available on websites maintained by one or more underwriters, or selling group members, if any, participating in this offering. The representatives may agree to allocate a number of shares of common stock to underwriters for sale to their online brokerage account holders. Internet distributions will be allocated by the representatives to underwriters that may make Internet distributions on the same basis as other allocations.

The underwriters and their respective affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging. financing and brokerage activities. Certain of the underwriters and their respective affiliates have, from time to time, performed, and may in the future perform, various financial advisory and investment banking services for us, for which they received or will receive customary fees and expenses.

In addition, in the ordinary course of their various business activities, the underwriters and their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers and may at any time hold long and short positions in such securities and instruments. Such investment and securities activities may involve our securities and instruments. The underwriters and their respective affiliates may also make investment recommendations or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long or short positions in such securities and instruments.

Pricing of the Offering

Prior to this offering, there has been no public market for our common stock. The initial public offering price was determined by negotiations between us and the representatives. Among the factors considered in determining the initial public offering price were our future prospects and those of our industry in general, our sales, earnings and certain other financial and operating information in recent periods, and the price-earnings ratios, price-sales ratios, market prices of securities, and certain financial and operating information of companies engaged in activities similar to ours.

Selling Restrictions

European Economic Area

In relation to each Member State of the European Economic Area which has implemented the Prospectus Directive (each, a Relevant Member State) an offer to the public of any shares of our common stock may not be made in that Relevant Member State, except that an offer to the public in that Relevant Member State of any shares of our common stock may be made at any time under the following exemptions under the Prospectus Directive, if they have been implemented in that Relevant Member State:

(a) to any legal entity which is a qualified investor as defined in the Prospectus Directive;

(b) to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the representatives for any such offer; or

(c) in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of shares of our common stock shall result in a requirement for the publication by us or any underwriter of a prospectus pursuant to Article 3 of the Prospectus Directive.

For the purposes of this provision, the expression an "offer to the public" in relation to any shares of our common stock in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and any shares of our common stock to be offered so as to enable an investor to decide to purchase any shares of our common stock, as the same may be varied in that Member State by any measure implementing the Prospectus Directive in that Member State, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

United Kingdom

Each underwriter has represented and agreed that:

(a) it has only communicated or caused to be communicated and will only communicate or cause to be communicated an invitation or inducement to engage in investment activity (within the meaning of Section 21 of the Financial Services and Markets Act 2000 (FSMA) received by it in connection with the issue or sale of the shares of our common stock in circumstances in which Section 21(1) of the FSMA does not apply to us; and

(b) it has complied and will comply with all applicable provisions of the FSMA with respect to anything done by it in relation to the shares of our common stock in, from or otherwise involving the United Kingdom.

INDUSTRY AND MARKET DATA

This prospectus includes industry and market data that we obtained from periodic industry publications, third-party studies and surveys, filings of public companies in our industry and internal company surveys. These sources include government and industry sources. Industry publications and surveys generally state that the information contained therein has been obtained from sources believed to be reliable. Although we believe the industry and market data to be reliable as of the date of this prospectus, this information could prove to be inaccurate. Industry and market data could be wrong because of the method by which sources obtained their data and because information cannot always be verified with complete certainty due to the limits on the availability and reliability of raw data, the voluntary nature of the data gathering process and other limitations and uncertainties. In addition, we do not know all of the assumptions regarding general economic conditions or growth that were used in preparing the forecasts from the sources relied upon or cited herein.

LEGAL MATTERS

The validity of the common stock being offered will be passed upon for us by Gunderson Dettmer Stough Villeneuve Franklin & Hachigian, LLP, Boston, Massachusetts. Certain partners and employees of Gunderson Dettmer Stough Villeneuve Franklin & Hachigian, LLP are the beneficial owners of 15,440 shares of common stock issuable upon conversion of our preferred stock. Certain legal matters in connection with this offering will be passed upon for the underwriters by Davis Polk & Wardwell LLP, New York, New York.

EXPERTS

The financial statements as of December 31, 2014 and 2013 and for each of the two years in the period ended December 31, 2014 included in this prospectus have been so included in reliance on the report (which contains an emphasis of a matter paragraph relating to the significance of related party transactions) of PricewaterhouseCoopers LLP, an independent registered public accounting firm, given on the authority of said firm as experts in auditing and accounting.

CHANGE IN INDEPENDENT ACCOUNTANT

In January 2015, our board of directors decided to change independent accounting firms from Baker Tilly Virchow Krause, LLP (Baker Tilly) to PricewaterhouseCoopers LLP (PwC).

The reports of Baker Tilly on our financial statements for each of the two fiscal years prior to the change did not contain any adverse opinion or disclaimer of opinion, nor were such reports qualified or modified as to uncertainty, audit scope or accounting principles. We had no disagreements with Baker Tilly on any matter of accounting principles or practices, financial statement disclosure or auditing scope or procedure, which disagreements, if not resolved to its satisfaction, would have caused Baker Tilly to make reference in connection with its opinion to the subject matter of the disagreement during its audits for each of the two fiscal years prior to its dismissal. During the two most recent fiscal years preceding the change from Baker Tilly, there were no “reportable events” as such term is defined in Item 304(a)(1)(v) of Regulation S-K.

During the two years ended December 31, 2014, neither we, nor anyone acting on our behalf, consulted with PwC on matters that involved the application of accounting principles to a specified transaction, either completed or proposed, the type of audit opinion that might be rendered on our financial statements, and neither a written report nor oral advice was provided to us by PwC that was an important factor considered by us in reaching a decision as to the accounting, auditing or financial reporting issue or any other matter that was the subject of a disagreement as that term is used in Item 304 (a)(1)(iv) of Regulation S-K and the related instructions to Item 304 of Regulation S-K or a reportable event as that term is used in Item 304(a)(1)(v) and the related instructions to Item 304 of Regulation S-K.

We have provided Baker Tilly with a copy of the foregoing disclosure and have requested that Baker Tilly furnish us with a letter addressed to the SEC stating whether or not Baker Tilly agrees with the above statements and, if not, stating the respects in which it does not agree. A copy of the letter from Baker Tilly is filed as an exhibit to the registration statement of which this prospectus is a part.

WHERE YOU CAN FIND ADDITIONAL INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the common stock we are offering. This prospectus, which constitutes a part of the registration statement, does not contain all of the information set forth in the registration statement, some items of which are contained in exhibits to the registration statement as permitted by the rules and regulations of the SEC. For further information with respect to us and our common stock, we refer you to the registration statement, including the exhibits and the financial statements and notes filed as a part of the registration statement. Statements contained in this prospectus concerning the contents of any contract or any other document are not necessarily complete. If a contract or document has been filed as an exhibit to the registration statement, please see the copy of the contract or document that has been filed. Each statement in this prospectus relating to a contract or document filed as an exhibit is qualified in all respects by the filed exhibit. The exhibits to the registration statement should be reviewed for the complete contents of these contracts and documents.

A copy of the registration statement, including the exhibits and the financial statements and notes filed as a part of the registration statement, may be inspected without charge at the SEC's public reference facilities at 100 F Street, N.E., Washington, D.C. 20549, and copies of all or any part of the registration statement may be obtained from the SEC upon the payment of fees prescribed by it. You may call the SEC at 1-800-SEC-0330 for more information on the operation of the public reference facilities. The SEC maintains a website at www.sec.gov that contains reports, proxy and information statements and other information regarding companies, such as REGENXBIO, that file electronically with it.

Upon the completion of this offering, we will be subject to the information reporting requirements of the Securities Act and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for inspection and copying at the public reference room and website of the SEC referred to above. We also maintain a website at www.regenxbio.com, at which you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on, or that can be accessed through, our website is not incorporated by reference into this prospectus, and you should not consider information contained on our website to be part of this prospectus or in deciding whether to purchase shares of our common stock.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Index
December 31, 2013 and 2014, and June 30, 2015 (unaudited)

Report of Independent Registered Public Accounting Firm

To the stockholders and Board of Directors of
REGENXBIO Inc.:

In our opinion, the accompanying balance sheets of REGENXBIO Inc. (the Company) and the related statements of operations, statement of convertible preferred stock and preferred units and stockholders' and members' deficit, and statements of cash flows present fairly, in all material respects, the financial position of the Company at December 31, 2014 and 2013, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2014 in conformity with accounting principles generally accepted in the United States of America. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits. We conducted our audits of these statements in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

As discussed in Note 11 to the financial statements, the Company has significant related party transactions.

/s/ PricewaterhouseCoopers LLP

McLean, VA
July 1, 2015

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Balance Sheets

(In thousands, except per share data)	December 31, 2013	December 31, 2014	June 30, 2015 (unaudited)	Pro Forma June 30, 2015 (unaudited)
Assets				
Current assets				
Cash and cash equivalents	$ 1,119	$ 1,121	$ 85,215	$ 85,215
Accounts receivable				
Trade receivables	50	805	704	704
Related party receivables	924	750	—	—
Unbilled receivables	114	327	—	—
Prepaid expenses	—	28	1,170	1,170
Total current assets	2,207	3,031	87,089	87,089
Property and equipment, net	—	—	312	312
Cost method investments	303	303	303	303
Deferred issuance costs	—	157	1,056	1,056
Other assets	—	—	40	40
Total assets	$ 2,510	$ 3,491	$ 88,800	$ 88,800
Liabilities, Convertible Preferred Stock and Preferred Units, and Stockholders' and Members' Equity (Deficit)				
Current liabilities				
Accounts payable	$ 301	$ 334	$ 925	$ 925
Accrued expenses	194	1,115	3,062	3,062
Due to related party under services agreement	655	1,423	—	—
Related party promissory notes	—	2,403	—	—
Other related party payables	3,503	3,761	1,919	1,919
Advance payments	—	153	132	132
Total current liabilities	4,653	9,189	6,038	6,038
Deferred rent	—	—	134	134
Total liabilities	4,653	9,189	6,172	6,172
Commitments and contingencies (Note 5)				
Convertible preferred stock and preferred units				
Series A preferred units; no par value; 119,656 units authorized, issued, and outstanding at December 31, 2013, and no units authorized, issued, and outstanding at December 31, 2014, June 30, 2015 (unaudited), or pro forma (unaudited)	3,779	—	—	—
Series B preferred units; no par value; 95,315 units authorized, issued, and outstanding at December 31, 2013, and no units authorized, issued, and outstanding at December 31, 2014, June 30, 2015 (unaudited), or pro forma (unaudited)	7,999	—	—	—
Series A convertible preferred stock; $0.0001 par value; no shares authorized, issued, and outstanding at December 31, 2013, and 2,393 shares authorized, issued, and outstanding at December 31, 2014 (aggregate liquidation preference of $3,963) and June 30, 2015 (aggregate liquidation preference of $3,000) (unaudited), and no shares authorized, issued, and outstanding pro forma (unaudited)	—	3,963	3,000	—
Series B convertible preferred stock; $0.0001 par value; no shares authorized, issued, and outstanding at December 31, 2013, and 1,906 shares authorized, issued, and outstanding at December 31, 2014 (aggregate liquidation preference of $8,630) and June 30, 2015 (aggregate liquidation preference of $7,892) (unaudited), and no shares authorized, issued, and outstanding pro forma (unaudited)	—	8,630	7,892	—
Series C convertible preferred stock; $0.0001 par value; no shares authorized, issued, and outstanding at December 31, 2013 or December 31, 2014, and 4,632 shares authorized, issued, and outstanding at June 30, 2015 (aggregate liquidation preference of $30,000) (unaudited), and no shares authorized, issued, and outstanding pro forma (unaudited)	—	—	30,000	—
Series D convertible preferred stock; $0.0001 par value; no shares authorized, issued, and outstanding at December 31, 2013 or December 31, 2014, and 7,367 shares authorized, issued, and outstanding at June 30, 2015 (aggregate liquidation preference of $70,500) (unaudited), and no shares authorized, issued and outstanding pro forma (unaudited)	—	—	70,500	—
Total convertible preferred stock and preferred units	11,778	12,593	111,392	—
Stockholders' and members' equity (deficit)				
Class A units; no par value; 132,148 units authorized, issued, and outstanding at December 31, 2013, and no units authorized, issued, and outstanding at December 31, 2014, June 30, 2015 (unaudited), or pro forma (unaudited)	10,885	—	—	—
Common stock; $0.0001 par value; no shares authorized, issued, and outstanding at December 31, 2013, and 9,500, 23,100, and 100,000 shares authorized and 2,645, 2,753, and 19,051 shares issued and outstanding at December 31, 2014, June 30, 2015 (unaudited), and pro forma (unaudited), respectively	—	—	—	2
Additional paid-in capital	—	10,518	10,346	121,736
Accumulated deficit	(24,806)	(28,809)	(39,110)	(39,110)
Total stockholders' and members' equity (deficit)	(13,921)	(18,291)	(28,764)	82,628
Total liabilities, convertible preferred stock and preferred units, and stockholders' and members' equity (deficit)	$ 2,510	$ 3,491	$ 88,800	$ 88,800

The accompanying notes are an integral part of these financial statements.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Statements of Operations

(In thousands, except per share data)	Years Ended December 31, 2013	Years Ended December 31, 2014	Six Months Ended June 30, 2014	Six Months Ended June 30, 2015
			(unaudited)	
Revenues				
License revenue	$ 1,055	$ 4,355	$3,705	$ 570
License revenue from related party	2,700	220	—	1,000
Reagent sales	368	326	291	148
Grant revenue	1,964	1,219	490	289
Total revenues	6,087	6,120	4,486	2,007
Expenses				
Costs of revenues				
Licensing costs to related parties	151	885	741	314
Costs of reagent sales (including amounts to related parties)	173	122	102	49
Research and development (including amounts to related parties)	5,051	4,961	1,787	6,803
General and administrative (including amounts to related parties)	5,474	3,851	1,660	5,113
Foreign currency transaction losses (gains)	14	30	(14)	38
Other operating income	—	(47)	(24)	(21)
Total operating expenses	10,863	9,802	4,252	12,296
Income (loss) from operations	(4,776)	(3,682)	234	(10,289)
Other Income (Expense)				
Investment income	—	—	—	8
Interest expense	(611)	(321)	(111)	(20)
Total other income (expense)	(611)	(321)	(111)	(12)
Net income (loss)	(5,387)	(4,003)	123	(10,301)
Accretion and dividends on convertible preferred stock and preferred units	(422)	(815)	(467)	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders and members	$ (5,809)	$(4,818)	$ (344)	$(11,289)
Basic and diluted net loss per common share	$ (2.50)	$ (1.82)	$ (0.13)	$ (4.21)
Weighted-average basic and diluted common shares	2,320	2,643	2,643	2,679
Pro forma basic and diluted net loss per common share (unaudited) (Note 2)		$ (0.58)		$ (0.78)
Pro forma weighted-average basic and diluted common shares (unaudited) (Note 2)		6,943		13,149

The accompanying notes are an integral part of these financial statements.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Statement of Convertible Preferred Stock and Preferred Units, and Stockholders' and Members' Equity (Deficit)
(In thousands)

	Series A Preferred Units		Series B Preferred Units		Series A Convertible Preferred Stock		Series B Convertible Preferred Stock		Series C Convertible Preferred Stock		Series D Convertible Preferred Stock		Total Convertible Preferred Stock and Preferred Units	Class A Units		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' and Members' Equity (Deficit)
	Units	Amount	Units	Amount	Shares	Amount	Shares	Amount	Shares	Amount	Shares	Amount		Units	Amount	Shares	Amount			
Balances at December 31, 2012	119,656	$ 3,499	—	$ —	—	$ —	—	$ —	—	$ —	—	$ —	$ 3,499	112,672	$ 10,597	—	$ —	$ —	$ (18,709)	$ (8,112)
Issuance of Series B preferred units, net of transaction costs of $35	—	—	24,155	1,965	—	—	—	—	—	—	—	—	1,965	19,476	288	—	—	—	(288)	—
Issuance of Series B preferred units for the conversion of outstanding related party debt	—	—	71,160	5,892	—	—	—	—	—	—	—	—	5,892	—	—	—	—	—	—	—
Accretion of preferred units	—	280	—	142	—	—	—	—	—	—	—	—	422	—	—	—	—	—	(422)	(422)
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(5,387)	(5,387)
Balances at December 31, 2013	119,656	3,779	95,315	7,999	—	—	—	—	—	—	—	—	11,778	132,148	10,885	—	—	—	(24,806)	(13,921)
Conversion from LLC to C corporation	(119,656)	(3,779)	(95,315)	(7,999)	2,393	3,779	1,906	7,999	—	—	—	—	—	(132,148)	(10,885)	2,643	—	10,885	—	—
Accretion of convertible preferred stock	—	—	—	—	—	184	—	631	—	—	—	—	815	—	—	—	—	(815)	—	(815)
Discount on related party promissory notes	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	128	—	128
Exercise of stock options	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	2	—	1	—	1
Stock-based compensation expense	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	319	—	319
Net loss	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(4,003)	(4,003)
Balances at December 31, 2014	—	—	—	—	2,393	3,963	1,906	8,630	—	—	—	—	12,593	—	—	2,645	—	10,518	(28,809)	(18,291)
Issuance of Series C convertible preferred stock, net of transaction costs of $187 (unaudited)	—	—	—	—	—	—	—	—	4,047	26,021	—	—	26,021	—	—	—	—	—	—	—
Issuance of Series C convertible preferred stock, for the conversion of outstanding related party debt (unaudited)	—	—	—	—	—	—	—	—	585	3,792	—	—	3,792	—	—	—	—	—	—	—
Issuance of Series D convertible preferred stock, net of transaction costs of $2,502 (unaudited)	—	—	—	—	—	—	—	—	—	—	7,367	67,998	67,998	—	—	—	—	—	—	—
Loss (gain) on extinguishment of convertible preferred stock (unaudited)	—	—	—	—	—	1,317	—	(2,076)	—	—	—	—	(759)	—	—	—	—	759	—	759
Accretion (decretion) of convertible preferred stock (unaudited)	—	—	—	—	—	(2,280)	—	1,338	—	187	—	2,502	1,747	—	—	—	—	(1,747)	—	(1,747)
Discount on related party promissory notes (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	13	—	13
Exercise of stock options (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	108	—	92	—	92
Stock-based compensation expense (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	711	—	711
Net loss (unaudited)	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	—	(10,301)	(10,301)
Balances at June 30, 2015 (unaudited)	—	—	—	—	2,393	$ 3,000	1,906	$ 7,892	4,632	$30,000	7,367	$70,500	$ 111,392	—	$ —	2,753	$ —	$ 10,346	$ (39,110)	$ (28,764)

The accompanying notes are an integral part of these financial statements.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Statements of Cash Flows

(In thousands)	Years Ended December 31, 2013	Years Ended December 31, 2014	Six Months Ended June 30, 2014 (unaudited)	Six Months Ended June 30, 2015 (unaudited)
Cash Flows From Operating Activities				
Net income (loss)	$(5,387)	$(4,003)	$ 123	$(10,301)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities				
Non-cash consideration received for licenses granted	(303)	—	—	—
Unrealized foreign currency transaction losses (gains)	14	44	—	(7)
Stock-based compensation expense	—	319	—	711
Imputed interest on related party promissory notes	—	128	—	13
Other non-cash adjustments	—	3	—	—
Depreciation and amortization	—	—	—	15
(Increase) decrease in				
Trade receivables	(36)	(799)	(668)	108
Related party receivables	(924)	174	924	750
Unbilled receivables	109	(213)	(235)	327
Prepaid expenses	—	(28)	(70)	(1,142)
Other assets	—	—	—	(40)
Increase (decrease) in				
Accounts payable	257	33	17	585
Accrued expenses	107	764	401	1,067
Due to related party under services agreement	3,192	768	1,400	—
Other related party payables	(41)	258	(1,051)	(1,876)
Advance payments	—	153	176	(21)
Deferred rent	—	—	—	134
Net cash provided by (used in) operating activities	(3,012)	(2,399)	1,017	(9,677)
Cash Flows From Investing Activities				
Purchases of property and equipment	—	—	—	(315)
Net cash used in investing activities	—	—	—	(315)
Cash Flows From Financing Activities				
Issuance of Series B preferred units, net of transaction costs	1,965	—	—	—
Proceeds from related party promissory notes	—	2,400	—	—
Proceeds from exercise of stock options	—	1	—	92
Issuance of Series C convertible preferred stock, net of transaction costs	—	—	—	26,021
Issuance of Series D convertible preferred stock, net of transaction costs	—	—	—	67,998
Issuance costs for planned initial public offering	—	—	—	(25)
Net cash provided by financing activities	1,965	2,401	—	94,086
Net increase (decrease) in cash and cash equivalents	(1,047)	2	1,017	84,094
Cash and Cash Equivalents				
Beginning of period	2,166	1,119	1,119	1,121
End of period	$ 1,119	$ 1,121	$ 2,136	$ 85,215
Supplemental Cash Flow Information				
Cash paid for interest	$ —	$ 164	$ —	$ 7
Supplemental Disclosures of Non-Cash Investing and Financing Activities				
Conversion of accrued service fees to related party into Series B preferred units	$ 5,892	$ —	$ —	$ —
Non-cash consideration received for licenses granted	$ 303	$ —	$ —	$ —
Deferred issuance costs for Series C convertible preferred stock in accrued expenses	$ —	$ 157	$ —	$ —
Conversion of accrued service fees to related party into Series C convertible preferred stock	$ —	$ —	$ —	$ 2,403
Conversion of related party promissory notes into Series C convertible preferred stock	$ —	$ —	$ —	$ 1,389
Purchases of property and equipment included in accounts payable and accrued expenses	$ —	$ —	$ —	$ 12
Deferred issuance costs for planned initial public offering in accrued expenses	$ —	$ —	$ —	$ 1,031

The accompanying notes are an integral part of these financial statements.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
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1. Nature of Business

REGENXBIO Inc. (the "Company") was formed on July 16, 2008 in the state of Delaware as ReGenX, LLC, and on December 22, 2009, changed its name to ReGenX Biosciences, LLC. On September 16, 2014, the Company converted from a limited liability company ("LLC") to a C-corporation, and changed its name to REGENXBIO Inc. The Company uses its proprietary NAV® Technology platform and collaborates with clinical advisors to advance the development of gene therapy treatments for a range of severe diseases with unmet needs.

Liquidity and Risks

As of December 31, 2014, the Company generated an accumulated deficit of $28,809 since inception and will require substantial additional capital to fund its research and development. As of June 30, 2015 (unaudited), the accumulated deficit was $39,110. In January 2015, the Company issued 4,632 shares of Series C convertible preferred stock ("Series C Preferred Stock") (Note 6) for an aggregate gross proceeds of $30,000, including $26,208 in cash proceeds and $3,792 in share-settled debt (Note 4) to a related party. In May 2015, the Company issued 7,367 shares of Series D convertible preferred stock ("Series D Preferred Stock") (Note 12) for gross cash proceeds of $70,500. The Company believes these proceeds, together with cash and cash equivalents of $1,121 and $85,215 at December 31, 2014 and June 30, 2015 (unaudited), respectively, are sufficient cash resources to allow the Company to fund its current operations for at least the next twelve months from June 30, 2015. As the Company continues to incur losses, transition to profitability is dependent upon the successful development, approval, and commercialization of its product candidates and achieving a level of revenues adequate to support the Company's cost structure. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital. Management intends to fund future operations through additional private or public debt or equity offerings, and may seek additional capital through arrangements with strategic partners or from other sources.

The Company is seeking to complete an initial public offering ("IPO") of its common stock. Upon the closing of a qualified public offering, the Company's outstanding convertible preferred stock will automatically convert into shares of common stock (Note 6).

In the event the Company does not complete an IPO, the Company expects to seek additional funding through private financings, debt financing, collaboration agreements, or government grants. The Company may not be able to obtain financing on acceptable terms, or at all, and the Company may not be able to enter into collaboration arrangements or obtain government grants. The terms of any financing may adversely affect the holdings or the rights of the Company's stockholders. If the Company is unable to obtain funding, the Company could be forced to delay, reduce, or eliminate its research and development programs, product candidate expansion, or commercialization efforts, which could adversely affect its business prospects. Although management continues to pursue these plans, there is no assurance that the Company will be successful in obtaining sufficient funding on terms acceptable to the Company to fund continuing operations, if at all.

The Company is subject to risks common to companies in the biotechnology industry, including, but not limited to, development by the Company or its competitors of technological innovations, risks of failure of clinical studies, dependence on key personnel, protection of proprietary technology, compliance with government regulations, and ability to transition from preclinical manufacturing to commercial production of products.

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2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America ("GAAP"). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification ("ASC") and Accounting Standards Update ("ASU") of the Financial Accounting Standards Board ("FASB").

Unaudited Interim Financial Information

The accompanying balance sheet as of June 30, 2015, the statements of operations and statements of cash flows for the six months ended June 30, 2014 and 2015, and the statement of convertible preferred stock and preferred units, and stockholders' and members' equity (deficit) for the six months ended June 30, 2015 are unaudited. The interim unaudited financial statements have been prepared on the same basis as the annual audited financial statements; and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of June 30, 2015, and the results of its operations and its cash flows for the six months ended June 30, 2014 and 2015. The financial data and other information disclosed in these notes related to the six months ended June 30, 2014 and 2015 are unaudited. The results for the six months ended June 30, 2015 are not necessarily indicative of results to be expected for the year ending December 31, 2015, any other interim periods, or any future year or period.

Unaudited Pro Forma Information

The unaudited pro forma balance sheet as of June 30, 2015 assumes the automatic conversion of Series A convertible preferred stock ("Series A Preferred Stock") (2,393 shares), Series B convertible preferred stock ("Series B Preferred Stock") (1,906 shares), Series C Preferred Stock (4,632 shares), and Series D Preferred Stock (7,367 shares) into 16,298 shares of common stock upon the completion of an IPO of the Company's common stock.

Unaudited pro forma net loss per share applicable to common stockholders for the year ended December 31, 2014 and the six months ended June 30, 2015 is computed using the weighted-average number of common shares outstanding after giving pro forma effect to the automatic conversion of all outstanding shares of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock into common stock as if the conversion had occurred at the beginning of the period presented, or the date of original issuance, if later. Pro forma net loss applicable to common stockholders excludes the accretion/decretion and dividends on convertible preferred stock and the net gain on extinguishment of convertible preferred stock.

Foreign Currency Transactions

Transaction gains (losses) that arise from exchange rate fluctuations on transactions denominated in a currency other than the U.S. dollar are included in the statements of operations as incurred. For the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), the Company incurred foreign currency transaction gains (losses) of ($14), ($30), $14, and ($38), respectively. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had ($14), ($44), and $7, respectively, of unrealized foreign currency gains (losses), which are included in trade accounts receivable on the balance sheets.

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Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the reported amounts in the financial statements and accompanying notes. Actual results could materially differ from those estimates. Management considers many factors in selecting appropriate financial accounting policies and controls, and in developing the estimates and assumptions that are used in the preparation of these financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational changes, sensitivity and volatility associated with the assumptions used in developing estimates, and whether historical trends are expected to be representative of future trends. The estimation process often may yield a range of potentially reasonable estimates of the ultimate future outcomes and management must select an amount that falls within that range of reasonable estimates. This process may result in actual results differing materially from those estimated amounts used in the preparation of the financial statements. Estimates are used in the following areas, among others: stock-based compensation expense, accrued research and development expenses, and the fair value of financial instruments.

Segment and Geographical Information

Operating segments are identified as components of an enterprise about which separate discrete financial information is available for evaluation by the chief operating decision maker (“CODM”), or decision making group, in making decisions on how to allocate resources and assess performance. The Company’s CODM, the Chief Executive Officer, views its operations and manages its business as one operating segment.

For the year ended December 31, 2013, 86 percent and 13 percent of the Company’s revenue was generated from customers located in the United States and Europe, respectively. For the year ended December 31, 2014, 60 percent and 40 percent of the Company’s revenue was generated from customers located in the United States and Europe, respectively. For the six months ended June 30, 2014 (unaudited), 66 percent and 33 percent of the Company’s revenue was generated from customers located in the United States and Europe, respectively. For the six months ended June 30, 2015 (unaudited), 81 percent and 17 percent of the Company’s revenue was generated from customers located in the United States and Europe, respectively. All of the Company’s assets currently reside in the United States.

Cash and Cash Equivalents

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and money market mutual funds.

Concentrations of Credit Risk and Off-balance Sheet Risk

Cash and cash equivalents and accounts receivable are financial instruments that are potentially subject to concentrations of credit risk. The Company’s cash and cash equivalents are deposited in accounts at two financial institutions, and amounts may exceed federally insured limits. The Company believes it is not exposed to significant credit risk due to the financial strength of the depository institutions in which the cash is held. Management believes that it is not exposed to significant credit risk related to accounts receivable due to the credit quality and history of collections from its significant customers. The Company has no financial instruments with off-balance sheet risk of loss.

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The following table summarizes those customers who represented at least 10 percent of revenue or accounts receivable for the periods presented:

	Revenue				Accounts Receivable		
	Years Ended December 31,		Six Months Ended June 30,		December 31,		June 30,
	2013	2014	2014	2015	2013	2014	2015
			(unaudited)				(unaudited)
Customer A	*	33%	45%	15%	*	*	*
Customer B	11%	14%	*	12%	*	46%	76%
Customer C(1)	44%	*	*	50%	85%	40%	*
Customer D	*	*	*	*	*	11%	*
Customer E	21%	*	*	*	*	*	*
Customer F	*	*	*	*	*	*	14%
Customer G	*	*	11%	*	*	*	*
Customer H	*	*	13%	*	*	*	*
Customer I	*	*	11%	*	*	*	*

* Represented less than 10%
(1) Represents a related party

Accounts Receivable

Trade accounts receivable consist of amounts due to the Company resulting from the Company's licensing arrangements, reagent sales, and grant programs. Related party accounts receivable consists of amounts due from related parties (Note 11). Unbilled receivables consist of estimated costs incurred under the Company's grant programs which have not yet been submitted to the grantor for reimbursement. Receivables are stated net of an allowance for doubtful accounts, if deemed necessary based on the Company's evaluation of collectability using specific identification of account balances and historical information regarding write-offs. Account balances are charged off against the allowance when the potential for recovery is considered remote. The Company has not recorded an allowance for doubtful accounts as of December 31, 2013 or 2014, or June 30, 2015 (unaudited).

Deferred Issuance Costs

Deferred issuance costs, which consist of direct and incremental fees relating to the issuance of equity securities are capitalized. As of December 31, 2014, the Company capitalized $157 of deferred issuance costs related to Series C Preferred Stock (Note 6), which were offset against the proceeds from the issuance of the Series C Preferred Stock in January 2015. As of June 30, 2015 (unaudited), the Company capitalized $1,056 of deferred issuance costs related to the planned IPO, which will be offset against the proceeds from the potential offering. As of December 31, 2013, no amounts were deferred.

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Property and Equipment

Property and equipment is stated at cost. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred. Upon disposal, the related cost and accumulated depreciation is removed from the accounts and any resulting gain or loss is included in the results of operations. Upon retirement or sale, the cost of the disposed asset and the related accumulated depreciation are removed from the accounts and any resulting gain or loss is recognized. Depreciation and amortization is calculated using the straight-line method over the estimated useful lives of the assets, which are as follows:

Computer equipment and software	3 years
Lab equipment	5 years
Furniture and fixtures	5 years
Leasehold improvements	Shorter of lease term or estimated useful life

Impairment of Long-lived Assets

The Company reviews long-lived assets when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparison of the book values of the assets to estimated future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book value of the assets exceed their fair value, which is measured based on the projected discounted future net cash flows arising from the assets. As of December 31, 2013 and 2014, the Company had recorded no long-lived assets. No impairment losses have been recorded during the six months ended June 30, 2015 (unaudited).

Cost Method Investments

Cost method investments consist of holdings in certain corporations and are stated at cost. The Company accounts for its investments in other entities using the cost method if its ownership interest is below 20 percent and the Company does not have significant influence over the operations of the entity. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), cost method investments in Audentes Therapeutics, Inc. (“Audentes”) and Dimension Therapeutics, Inc. (“Dimension”) had a carrying value of $303. See Notes 7 and 11 for further information regarding the Company’s investments in Audentes and Dimension.

Declines in the fair value of cost method investments below their carrying value that are deemed to be other-than-temporary are reflected in the statements of operations as realized losses. In estimating other-than-temporary impairment losses, management considers, among other things, (i) the length of time and the extent to which the fair value has been less than cost, (ii) the financial condition and near-term prospects of the issuer, and (iii) the intent and ability of the Company to retain its investments in the issuer for a period of time sufficient to allow for the anticipated recovery in fair value. The Company has not identified any events or changes in circumstances that would have an adverse effect on the fair value of its cost method investments. Accordingly, no other-than-temporary impairment losses were recorded for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2015 (unaudited).

The Company applies the variable interest model under FASB ASC Topic 810, *Consolidation* (“ASC 810”), to any entity in which the Company holds an equity investment or to which the Company has granted a commercial license. If the entity is within the scope of the model, and meets the definition of a variable interest entity (“VIE”), the Company considers whether it must consolidate the VIE or if further disclosures regarding the Company’s involvement with the VIE are necessary. If the Company is determined to be the primary beneficiary of the VIE, the Company will consolidate the VIE. This analysis is performed at the initial investment in the entity or the inception of the commercial license agreement, or upon any reconsideration event.

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The Company considers a legal entity a VIE if (i) its investors do not have sufficient equity at risk for the legal entity to finance its activities without additional subordinated financial support, or (ii) as a group, the holders of the equity investment at risk do not have both the power to direct the activities of the legal entity that most significantly impact the entity's economic performance, and the obligation to absorb the expected losses or the right to receive expected residual returns of the legal entity. The Company considers itself to be the primary beneficiary of a VIE if the Company has both the power to direct the activities that most significantly affect the VIE's economic performance and the obligation to absorb the losses of, or right to receive benefits from, the VIE that could be potentially significant to the VIE. If the Company, or any of the Company's related parties which have a variable interest in the VIE, individually lack the necessary power and benefits criteria, but the related party group as a whole has the necessary power and benefits, the Company determines which of the related party group members is most closely associated with the VIE and considers that party to be the primary beneficiary. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company has not consolidated any VIE's. See Note 11 for further information regarding the Company's involvement and variable interests in related parties and entities controlled by related parties.

Related Party Debt Instruments

The Company evaluates each of its related party debt instruments (Note 4) with embedded features under FASB ASC Topic 815, *Derivatives and Hedging* ("ASC 815"). More specifically, the Company evaluates all of the stated and implied substantive terms and features of the debt, including: (i) whether the debt included redemption features, (ii) how and when any redemption features could be exercised and settled, and (iii) the existence and nature of any conversion rights.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC Topic 820, *Fair Value Measurements and Disclosures* ("ASC 820"), establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement.

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Financial instruments reported at fair value on a recurring basis include cash equivalents. The following tables present the cash and cash equivalents carried at fair value in accordance with the hierarchy discussed above:

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2014				
Cash	$ 277	$ —	$ —	$ 277
Money market mutual funds	—	844	—	844
	$ 277	$ 844	$ —	$1,121

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
June 30, 2015 (unaudited)				
Cash	$ 174	$ —	$ —	$ 174
Money market mutual funds	—	85,041	—	85,041
	$ 174	$ 85,041	$ —	$85,215

As of December 31, 2013, the Company had no money market mutual funds carried at fair value on a recurring basis.

Management estimates that the carrying amounts of its accounts receivable, accounts payable, accrued expenses, and related party payables approximate fair value due to the short-term nature of those instruments.

Certain debt instruments (Note 4) outstanding at December 31, 2014 accrue interest at below market rates. The Company has recorded a discount to the face value of the instrument to account for the difference between the present value of the debt at an estimated market rate versus face value. Accordingly, management believes that the carrying values of all debt instruments approximate fair value.

The Company has determined that it is not practicable to estimate the fair value of cost method investments. The Company has not identified any events or changes in circumstances that would have an adverse effect on the fair value of its cost method investments.

Convertible Preferred Stock and Preferred Units

In accordance with the guidance in FASB ASC Topic 480, *Distinguishing Liabilities from Equity*, outstanding shares of convertible preferred stock and preferred units (Note 6), were classified outside of permanent equity and within temporary equity, as of December 31, 2013 and 2014, and June 30, 2015 (unaudited) due to their associated redemption features and liquidation preferences. At each reporting date, each series of convertible preferred stock and preferred units is accreted and stated at the amounts in which each series is currently redeemable, which is also equal to the aggregate liquidation preference at that date.

The Company evaluated each series of its convertible preferred stock and preferred units and determined that each individual series is considered a debt host under ASC 815. In making this determination, the

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Company's analysis followed the whole instrument approach which compares an individual feature against the entire convertible preferred stock or preferred unit instrument which includes that feature. The Company's analysis was based on a consideration of the economic characteristics and risks of each series of convertible preferred stock and preferred units. More specifically, the Company evaluated all of the stated and implied substantive terms and features, including: (i) whether the convertible preferred stock and preferred units included redemption features, (ii) how and when any redemption features could be exercised, (iii) whether the holders of convertible preferred stock and preferred units were entitled to dividends and how those dividends were calculated, (iv) the voting rights of the convertible preferred stock and preferred units, and (v) the existence and nature of any conversion rights. As a result of the Company's conclusion that the convertible preferred stock and preferred units represent a debt host, the redemption features of all series of convertible preferred stock and preferred units are considered to be clearly and closely related to the associated debt host instruments. Accordingly, the redemption features of all series of convertible preferred stock are not considered embedded derivatives that require bifurcation. The Company also concluded that the conversion rights under the convertible preferred stock are not clearly and closely related to the debt host instruments. However, the Company concluded that the conversion rights do not meet the net settlement criteria of a derivative and, therefore, are not considered embedded derivatives that require bifurcation.

The Company accounts for potential beneficial conversion features of convertible preferred stock under FASB ASC 470-20, *Debt with Conversion and Other Options*. At the time of each of the issuances of convertible preferred stock, the Company's common stock into which each series of the Company's convertible preferred stock is convertible had an estimated fair value less than the effective conversion prices of the convertible preferred stock. Therefore, there was no intrinsic value on the respective issuance dates.

Revenue Recognition

The Company primarily generates revenue through license agreements with third parties which may grant rights to the research, development, and commercialization of product candidates using the Company's NAV® Technology. Additionally, the Company has generated revenue from grant programs and sales of licensed reagents to customers for use in research and development.

The Company recognizes revenue in accordance with FASB ASC Topic 605, *Revenue Recognition* ("ASC 605"). Accordingly, revenue is recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company's balance sheets.

The Company analyzes its revenue arrangements based on the guidance in FASB ASC Topic 605-25, *Revenue Recognition-Multiple-Element Arrangements* ("ASC 605-25"). Pursuant to the guidance in ASC 605-25, the Company evaluates multiple-element arrangements to determine (i) the deliverables included in the arrangement, and (ii) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires management to make judgments about the individual deliverables and whether such deliverables are

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separable from the other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially in the control of the Company. The Company does not have any material revenue arrangements that contain multiple deliverables.

License Revenue and License Revenue From Related Party. The terms of the Company's license agreements require delivery of an intellectual property license for use of the Company's intellectual property in research and/or commercial development of product candidates for various diseases. License agreements generally have a term equal to the life of the intellectual property, but are terminable at the option of the licensee. Non-refundable payments to the Company under these arrangements may include: (i) up-front license fees, (ii) option fees to exercise commercial licenses, (iii) annual maintenance fees, (iv) sublicense fees, (v) payments based on the achievement of certain milestones based solely on the efforts of the licensees, and (vi) royalties on product sales.

Nonrefundable up-front license fees are recognized as revenue upon delivery of the license provided there are no undelivered elements in the arrangement and the necessary criteria under ASC 605 for revenue recognition have been met.

Options to exercise commercial licenses are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the licensee will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the overall objective of the arrangement, the benefit the licensee might obtain from the arrangement without exercising the option, the cost to exercise the option and the likelihood that the option will be exercised. For arrangements under which an option is considered substantive, the Company does not consider the item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable arrangement consideration, provided the option is not priced at a significant and incremental discount. Conversely, for arrangements under which an option is not considered substantive or if an option is priced at a significant and incremental discount, the Company would consider the item underlying the option to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), all of the options included in the Company's license agreements have been determined to be substantive, and none of the options are priced at a significant and incremental discount. Option fees are recognized as revenue upon exercise and delivery of the underlying commercial license, provided there are no undelivered elements in the arrangement and the necessary criteria under ASC 605 for revenue recognition have been met.

Annual maintenance fees under the Company's license agreements do not represent a separate deliverable aside from the delivery of the license since the Company has no further obligations under the agreements. Accordingly, annual maintenance fees are recognized as revenue when billable under the agreement, provided the price is fixed or determinable and collectability is deemed reasonably assured.

Sublicense fees are payable to the Company upon the receipt of certain fees by the licensee from any sublicensees. Sublicense fees received by the Company are recognized as revenue when the price is fixed or determinable and collectability is deemed reasonably assured.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. The Company evaluates factors such as the scientific, clinical, regulatory, commercial, and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the

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respective milestone in making this assessment. There is considerable judgment involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. The Company has concluded that all of the clinical, regulatory, and commercial milestones pursuant to its license agreements are substantive. Milestone payments are recognized as revenue upon achievement of the milestone by the licensee, provided that all other revenue recognition criteria are satisfied.

The Company recognizes royalty revenue, if any, in the period of sale of the related product(s) based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, and provided that all other revenue recognition criteria under ASC 605 are satisfied. To date, the Company has not generated any royalty revenues.

Please refer to Note 11 for information regarding license revenue from related party.

Grant Revenue. Grant revenue is generated through research and development grant programs offered by the U.S. federal government and the European Union. Government grants provide funds for certain types of expenditures in connection with research and development activities over a contractually defined period. Revenue related to government grants is recognized in the period during which the related costs are incurred and the related services are rendered, provided that the applicable performance obligations under the grants have been met. Funds received under grants are recorded as revenue if the Company is deemed to be the principal participant and primary obligor in the contract arrangements because the activities under the contracts are part of the Company's development programs. If the Company is not the principal participant or primary obligor, the grant proceeds are recorded as a reduction to research and development expense.

The Company's grants contain refund provisions. The Company reviews those refund provisions to determine the likelihood of repayment. If the likelihood of repayment of the grant is determined to be remote, the grant is recognized as revenue. If the probability of repayment is determined to be more than remote, the Company records the amount of potential repayment of the grant as a liability, until such time that the grant requirements have been satisfied. Funds received in advance of the performance of the services are recorded as deferred revenue. Please refer to Note 7 for further information regarding the Company's grant agreements.

Reagent Sales. Reagent sales consist of sales of licensed reagents to third parties for use in research and development. Revenue from reagent sales is recognized upon delivery to customers, provided that all other revenue recognition criteria under ASC 605 are satisfied. Licensed reagents are primarily manufactured by a related party (Note 11).

Research and Development Expenses

Research and development costs are charged to expense as costs are incurred. Research and development costs include allocated salaries and benefits, other personnel costs, facilities costs, overhead costs, preclinical and clinical contract services, regulatory, manufacturing, and other related costs. Up-front fees incurred in obtaining technology licenses, are charged to research and development expense as incurred if the technology licensed has no alternative future use. Advance payments for goods or services for future research and development activities are deferred and expensed as the goods are delivered or the related services are performed.

The Company estimates preclinical studies and clinical trial expenses based on the services performed pursuant to contracts with research institutions and clinical research organizations that conduct and manage preclinical studies and clinical trials on the Company's behalf. In accruing service fees, the Company estimates the time period over which services will be performed and the level of effort to be expended in each period.

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These estimates are based on communications with the third party service providers and the Company's estimates of accrued expenses using information available at each balance sheet date. If the actual timing of the performance of services or the level of effort varies from the estimate, the Company will adjust the accrual accordingly. There have been no significant changes from the Company's original estimates in any of the periods presented.

Stock-based Compensation

The Company accounts for its stock-based compensation awards in accordance with FASB ASC Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees, including grants of employee stock options, to be recognized in the statement of operations based on their fair values.

The Company's stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards to employees with service-based vesting conditions is recognized on a straight-line basis based on the estimated grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to non-employees with service-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, which is generally the vesting term, on a straight-line basis. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the estimated grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. Compensation expense related to awards to non-employees with performance-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

The Company estimates the fair value of its option awards to employees and directors using the Black-Scholes option-pricing model, which requires the input of and subjective assumptions, including (i) the fair value of the underlying common stock, (ii) the expected stock price volatility, (iii) the calculation of expected term of the award, (iv) the risk-free interest rate, and (v) expected dividends. Due to the lack of company specific historical and implied volatility data of its common stock, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. When selecting these public companies on which it has based its expected stock price volatility, the Company selected companies with comparable characteristics to it, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected term of the stock-based awards. The Company computes historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of the stock-based awards. The Company has estimated the expected term of its employee stock options using the "simplified" method, whereby, the expected term equals the arithmetic average of the vesting term and the original contractual term of the option due to its lack of sufficient historical data. The risk-free interest rates for periods within the expected term of the option are based on the U.S. Treasury securities with a maturity date commensurate with the expected term of the associated award. The Company has never paid, and does not expect to pay dividends in the foreseeable future.

The Company is also required to estimate forfeitures at the time of grant and revise those estimates in subsequent periods if actual forfeitures differ from its estimates. To date, a forfeiture rate of zero has been used to calculate stock-based compensation expense. To the extent that actual forfeitures differ from the Company's estimates, the differences are recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest.

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The Company has granted stock options to non-employees as compensation for advisory services provided to the Company. Consistent with the guidance in FASB ASC Topic 505-50, *Equity-Based Payments to Non-Employees*, the fair value of each non-employee stock option is estimated at the date of grant using the Black-Scholes option-pricing model with assumptions generally consistent with those used for employee stock options, with the exception of expected term, which is based on the contractual life.

Income Taxes

Income taxes are recorded in accordance with FASB ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. The Company recognizes deferred tax assets and liabilities for the expected future tax consequences of events that have been included in the financial statements or tax returns. Deferred tax assets and liabilities are determined based on the difference between the financial statement and tax bases of assets and liabilities using enacted tax rates in effect for the year in which the differences are expected to reverse. Valuation allowances are provided, if based upon the weight of available evidence, it is more likely than not that some or all of the deferred tax assets will not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company did not have any significant uncertain tax positions.

The Company will recognize interest and penalties related to uncertain tax positions in income tax expense. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had no accrued interest or penalties related to uncertain tax positions and no amounts have been recognized in the Company's statements of operations.

Net Loss Per Share

On September 16, 2014, the Company converted from an LLC to a C-corporation. Upon the conversion, every 50 Class A Units, Series A Preferred Units, and Series B Preferred Units (Note 6) held were converted into 1 share of common stock, Series A convertible preferred stock, and Series B convertible preferred stock, respectively. Class A Units of the LLC had similar rights and characteristics of common stock issued upon the conversion. In calculating net loss per share, the Company retrospectively applied the effects of the conversion to the number of Class A Units, Series A Preferred Units, and Series B Preferred Units outstanding prior to the conversion. Net loss per share for periods prior to the conversion to a C-corporation refers to net loss per Class A Unit.

The Company computes net loss per share in conformity with the two-class method required for participating securities. The Company considers all series of convertible preferred stock and preferred units to be participating securities, as the holders of convertible preferred stock are entitled to receive preferential dividends in the event that a dividend is paid on common stock and the holders of preferred units prior to the conversion to a C-corporation were entitled to a preferred return in the event that operating distributions were given to the Class A Unit holders of the LLC. The holders of convertible preferred stock and preferred units do not have a contractual obligation to share in the losses of the Company. As such, the Company's net losses for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2015 (unaudited) were not allocated to these participating securities. Additionally, net income for the six months ended June 30, 2014 (unaudited) was not allocated to participating securities because after subtracting accretion/decretion and dividends on preferred units, there was no net income remaining to be allocated to participating securities.

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Basic net loss per share is calculated by dividing net loss applicable to holders of common stock by the weighted-average shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is calculated by adjusting weighted-average shares outstanding for the dilutive effect of common stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the diluted net loss per share calculation, convertible preferred stock, stock options, and debt instruments containing share settlement options are considered to be common stock equivalents but have been excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net loss per share until the contingency has been fully met. Accordingly, basic and diluted net loss per share and unit were the same for all periods presented.

The following potentially dilutive securities have been excluded from the computations of diluted weighted-average shares outstanding as of December 31, 2013 and 2014, and June 30, 2014 and 2015 (unaudited) as they would be anti-dilutive:

	December 31,		June 30,	
	2013	2014	2014	2015
			(unaudited)	
Series A convertible preferred stock and preferred units	2,393	2,393	2,393	2,393
Series B convertible preferred stock and preferred units	1,906	1,906	1,906	1,906
Series C convertible preferred stock	—	—	—	4,632
Series D convertible preferred stock	—	—	—	7,367
Stock options issued and outstanding	—	2,107	—	3,063
Debt with share settlement option (Note 4)	—	3,715	—	—
	4,299	10,121	4,299	19,361

Amounts in the table above reflect the common stock equivalents of the noted instruments.

The following table summarizes the calculation of the unaudited pro forma basic and diluted net loss per share, for the year ended December 31, 2014 and the six months ended June 30, 2015:

	December 31, 2014	June 30, 2015
		(unaudited)
Numerator:		
Net loss applicable to common stockholders	$ (4,003)	$(10,301)
Denominator:		
Weighted-average basic and diluted common shares	6,943	13,149
Basic and diluted net loss per common share	$ (0.58)	$ (0.78)

Comprehensive Income (Loss)

The Company's comprehensive income (loss) is equal to its net income (loss) for all periods presented.

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Recently Announced Accounting Pronouncements

In February 2015, the FASB issued ASU 2015-2, *Consolidation (Topic 810): Amendments to the Consolidation Analysis*, which provides clarification regarding the guidance surrounding consolidation of certain legal entities. This guidance is effective for annual and interim periods beginning after December 15, 2015. The Company is evaluating the application of this ASU, but has not yet determined the potential effects it may have on the Company's financial statements.

In August 2014, the FASB issued ASU 2014-15, *Presentation of Financial Statements—Going Concern (Subtopic 205-40): Disclosure of Uncertainties about an Entity's Ability to Continue as a Going Concern*, requiring management to evaluate whether events or conditions could impact an entity's ability to continue as a going concern and to provide disclosures if necessary. Management will be required to perform the evaluation within one year after the date that the financial statements are issued. Disclosures will be required if conditions give rise to substantial doubt and the type of disclosure will be determined based on whether management's plans will be able to alleviate the substantial doubt. The ASU will be effective for the first annual period ending after December 15, 2016, and for annual periods and interim periods thereafter with early application permitted. The Company is evaluating the application of this ASU, but has not yet determined the potential effects it may have on the Company's financial statements.

In June 2014, the FASB issued ASU No. 2014-12, *Compensation—Stock Compensation (Topic 718): Accounting for Share-Based Payments When the Terms of an Award Provide That a Performance Target Could be Achieved after the Requisite Service Period*, which requires the Company to assess share-based awards with performance targets that could be achieved after the requisite service period for potential treatment as performance conditions. Under the ASU, compensation expense is to be recognized when the performance target is deemed probable and should represent the compensation expense attributable to the periods for which service has already been rendered. If the performance target is reached prior to achievement of the service period, the remaining unrecognized compensation cost should be recognized over the remaining service period. The ASU is effective for annual and interim periods beginning after December 15, 2015 with early adoption permitted. The Company has evaluated the application of this ASU, and determined that it does not have a material effect on the Company's financial statements.

In May 2014, the FASB issued ASU No. 2014-09, *Revenue from Contracts with Customers* (Topic 606), which supersedes the revenue recognition requirements in ASC 605, *Revenue Recognition*. This ASU is based on the principle that revenue is recognized to depict the transfer of goods or services to customers in an amount that reflects the consideration to which the entity expects to be entitled in exchange for those goods or services. The ASU also requires additional disclosure about the nature, amount, timing and uncertainty of revenue and cash flows arising from customer contracts, including significant judgments and changes in judgments and assets recognized from costs incurred to obtain or fulfill a contract. The ASU was originally effective January 1, 2017, however, on April 1, 2015, the FASB voted to propose a deferral of the effective date by one year until January 1, 2018, but will permit entities to adopt the standard as of the original effective date. The Company is evaluating the application of this ASU, but has not yet determined the potential effects it may have on the Company's financial statements.

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3. Property and Equipment, Net

Property and equipment, net consists of the following as of June 30, 2015 (unaudited):

	June 30, 2015 (unaudited)
Computer equipment and software	$ 240
Furniture and fixtures	85
Leasehold improvements	2
Total property and equipment	327
Accumulated depreciation and amortization	(15)
Property and equipment, net	$ 312

As of December 31, 2013 and 2014, the Company had recorded no property and equipment because the Company's resources used in operations were provided by a related party (Note 11). Accordingly, no depreciation or amortization expense has been recorded for the years ended December 31, 2013 and 2014 or the six months ended June 30, 2014 (unaudited). The Company recorded $15 of depreciation expense for the six months ended June 30, 2015 (unaudited).

4. Related Party Debt Instruments

Due to Related Party Under Services Agreement

Until January 31, 2015, the Company was party to a services agreement with FoxKiser LLP ("FoxKiser"), a related party (Note 11). Under the services agreement, the Company paid a fixed monthly fee and a support fee to FoxKiser. Amounts outstanding under the services agreement in excess of 30 days from their due date accrued interest at 1.5 percent per month, compounding monthly.

The Company entered into an agreement (the "Initial Conversion Agreement") with FoxKiser in which all principal and interest owed under the services agreement as of September 30, 2013, which was $5,892, may be settled at the option of FoxKiser, in whole or in part, (i) upon the next issuance of preferred equity securities by the Company, in shares of such preferred equity securities issued at a price per share equal to the issuance price of such shares, or (ii) if an issuance of preferred equity securities has not taken place by December 31, 2013, in shares of common equity securities of the Company at a price per share equal to the fair value of such common equity securities as determined by the Board of Directors. The debt is deemed fully settled upon conversion into equity securities of the Company.

On October 30, 2013, in conjunction with the Company's issuance of Series B Preferred Units (Note 6), FoxKiser converted the entire $5,892 outstanding under the Initial Conversion Agreement into 71,160 Series B Preferred Units at a price per unit of $0.082798.

As of December 31, 2013, amounts due to FoxKiser under the services agreement of $655 were not subject to the Initial Conversion Agreement. Total interest expense incurred under the services agreement for the year ended December 31, 2013 was $611.

On July 31, 2014, the Company entered into another agreement (the "Second Conversion Agreement") with FoxKiser. Under the Second Conversion Agreement, all principal and interest owed under the services agreement

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between the Company and FoxKiser on or after July 31, 2014, with a maximum amount of $2,000, may be settled at the option of FoxKiser, in whole or in part, (i) upon the next issuance of preferred equity securities by the Company, in shares of such preferred equity securities issued at a price per share equal to the issuance price of such shares, or (ii) if an issuance of preferred equity securities has not taken place by December 31, 2014, in shares of common equity securities of the Company at a price per share equal to the fair value of such common equity securities as determined by the Board of Directors. The debt is deemed fully settled upon conversion into equity securities of the Company.

As of December 31, 2014, the Company had accrued $1,423 payable to FoxKiser under the services agreement, which may be settled in preferred or common shares in accordance with the Second Conversion Agreement. Total interest expense incurred under the services agreement for the year ended December 31, 2014, and the six months ended June 30, 2014 and 2015 (unaudited) was $190, $111, and $7, respectively. At June 30, 2015 (unaudited), there were no amounts outstanding and all agreements with FoxKiser have been terminated.

Related Party Promissory Notes

On July 31, 2014, the Company received $1,800 in exchange for a promissory note issued to FoxKiser. On September 15, 2014, the Company received $600 in exchange for a second promissory note issued to FoxKiser. Both promissory notes accrued interest at the Short-Term Applicable Federal Rate (0.34% at December 31, 2014), compounding annually, and were payable on demand by FoxKiser at the earlier of December 31, 2014 or the next issuance of preferred equity securities by the Company.

Both promissory notes may be settled at the option of FoxKiser, in whole or in part, (i) upon the next issuance of preferred equity securities by the Company, in shares of such preferred equity securities issued at a price per share equal to the issuance price of such shares, or (ii) if an issuance of preferred equity securities has not taken place by December 31, 2014, in shares of common equity securities of the Company at a price per share equal to the fair value of such common equity securities as determined by the Board of Directors. The debt is fully settled upon conversion into equity securities of the Company.

The promissory notes with FoxKiser bear interest at below-market rates. Accordingly, the Company imputed interest on the promissory notes and recorded a discount equal to the difference between the face value of the promissory notes and the present value of the notes at an estimated market rate of 15 percent. The aggregate discount of $128 on the promissory notes was amortized using the effective interest method through December 31, 2014, at which date the notes became payable upon demand by FoxKiser. The discount was recorded as additional paid-in capital from FoxKiser due to the related party nature of the borrowing arrangements. Amortization of the discount is recorded as interest expense in the statements of operations. Interest expense, including imputed interest, incurred under the promissory notes for the year ended December 31, 2014 was $131. As of December 31, 2014, the promissory notes had an outstanding principal of $2,400, and accrued interest of $3. At June 30, 2015 (unaudited), there were no amounts outstanding and all agreements with FoxKiser have been terminated.

The Company evaluated the embedded features of each of its debt instruments under ASC 815. The Company has concluded that the redemption features, including all put and call features, with the exception of settlement upon a liquidation or change in control as discussed further below, are clearly and closely related to the debt host instruments and, therefore, are not considered embedded derivatives that require bifurcation. Additionally, the Company concluded that the share settlement rights of the debt instruments do not require bifurcation as embedded derivatives because in the event of a settlement in shares, the debt is settled in a variable number of equity securities with an aggregate fair value equaling the debt principal outstanding on the debt host instruments.

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Pursuant to the terms of the Initial Conversion Agreement, Second Conversion Agreement, and each of the promissory notes with FoxKiser, in the event of a liquidation or change in control, as defined in the agreements, the Company shall pay two times (2x) the principal and accrued interest then outstanding in order to settle the debt. The Company evaluated this redemption feature in accordance with ASC 815 and determined that it is an embedded derivative that should be bifurcated from each of the debt host instruments. However, due to the low probability of a liquidation or change in control event, the Company has determined that the liability associated with this derivative instrument is de minimis as of December 31, 2014. As discussed below, upon the conversion of the debt instruments into Series C Convertible Preferred Stock in January 2015, this redemption feature is no longer outstanding.

On January 13, 2015, in conjunction with the Company's issuance of Series C Preferred Stock (Note 6), FoxKiser elected its share settlement options and converted $1,389 of the amount due under the services agreement and $2,403 of principal and interest due under the promissory notes, for a total of $3,792, into 585 shares of Series C Preferred Stock at a per share price of $6.477. No amounts were outstanding under the services agreement or promissory notes as of June 30, 2015 (unaudited).

5. Commitments and Contingencies

Lease Agreements

The Company has entered into an operating lease for laboratory space in Philadelphia, Pennsylvania for use in its research and development activities. The lease is renewed in six-month terms and as of December 31, 2014 and June 30, 2015 (unaudited), the lease had an expiration date of June 30, 2015 and December 31, 2015, respectively. Monthly rent under the lease agreement is $4.

Effective January 31, 2015, the services agreement with FoxKiser (Note 11) was terminated and the Company entered into an operating lease with FoxKiser for office space in Washington, D.C. The lease agreement, which has a month-to-month term, required monthly payments of $20. The lease was terminated on April 30, 2015. The Company incurred rent expense of $60 to FoxKiser during the six months ended June 30, 2015 (unaudited), of which $40 is included in general and administrative expenses and $20 is included in research and development expenses in the statement of operations.

In March 2015, the Company entered into a 5.5 year, non-cancelable operating lease for office space in Rockville, Maryland. The lease commenced in April 2015, and expires in September 2020. The Company has options to extend the lease for up to 6 years. Initial monthly payments required under the lease are $24 and escalate annually in accordance with the lease. The Company records rent expense on a straight-line basis over the term of the lease.

As of June 30, 2015 (unaudited), future minimum lease payments under non-cancelable operating leases are as follows:

	Operating Leases
2015 (remained of year)	$ 83
2016	295
2017	302
2018	311
2019	320
2020	266
Total minimum lease payments	$ 1,577

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Rent expense under all operating leases for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), was $45, $45, $23, and $139, respectively.

License Agreements Granted to the Company

See Note 11 for information regarding licenses granted to the Company by related parties.

These agreements may require the Company to make future payments relating to sublicense fees, milestone fees for milestones not met as of December 31, 2014 and June 30, 2015 (unaudited), and royalties on future sales of licensed products. Additionally, the Company may be responsible for the cost of the maintenance of the intellectual property as incurred by its licensors.

ARIAD Pharmaceuticals, Inc. On November 19, 2010, the Company entered into a license agreement with ARIAD Pharmaceuticals, Inc. (“ARIAD”), for exclusive, worldwide rights to certain patents owned and exclusively licensed by ARIAD. In consideration for the license, the Company issued Class A Units to ARIAD with a fair value of $726. Under the terms of the agreement, the Company is obligated to pay ARIAD royalties on net sales, and sublicense fees, if any. Additionally, the Company is obligated to pay ARIAD up to $2,300 and annual maintenance fees of $50 upon the achievement of various milestones. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), no milestones have been achieved and accordingly no milestone payments or maintenance fees were payable to ARIAD. Additionally, the Company has not incurred any royalties or sublicense fees payable to ARIAD since the inception of the agreement.

Regents of the University of Minnesota. On November 10, 2014, the Company entered into a license agreement with Regents of the University of Minnesota (“Minnesota”), for an exclusive license under certain patent rights to commercialize products covered by the licensed patent rights in any country or territory in which a licensed patent has been issued and is unexpired, or a licensed patent application is pending. In consideration for the license, the Company paid an up-front fee of $25 and reimbursed Minnesota for patent maintenance expenses of $9. Under the terms of the agreement, the Company is obligated to pay Minnesota annual maintenance fees between $5 and $15 per year on each anniversary date of the agreement. Additionally, the Company is obligated to pay royalties on net sales and sublicense fees, if any, and up to $125 per licensed product upon the achievement of various milestones. As of December 31, 2014 and June 30, 2015 (unaudited), no milestones have been achieved, and accordingly, no milestone payments were payable to Minnesota. Additionally, the Company has not incurred any royalties or sublicense fees payable to Minnesota since the inception of the agreement. During the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited), the Company incurred $34 and $1, respectively, in expenses under the agreement with Minnesota for up-front license fees and patent maintenance expenses. Up-front license fees are included in research and development expenses and patent maintenance is included in general administrative expenses in the statements of operations.

Guarantees and Indemnifications

In the normal course of business, the Company enters into agreements that contain a variety of representations and provide for general indemnification. The Company’s exposure under these agreements is unknown because it involves claims that may be made against the Company in the future. To date, the Company has not paid any claims or been required to defend any action related to its indemnification obligations. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded any related liabilities.

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6. Capitalization

As of June 30, 2015 (unaudited), the authorized capital stock of the Company included 23,100 shares of common stock, par value $0.0001 per share, and 16,298 shares of preferred stock, par value $0.0001 per share. The Company's authorized preferred stock included 2,393 shares designated as Series A Preferred Stock, 1,906 shares designated as Series B Preferred Stock, 4,632 shares designated as Series C Preferred Stock, and 7,367 shares designated as Series D Preferred Stock. Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock are herein collectively referred to as "Preferred Stock."

As of December 31, 2014, the authorized capital stock of the Company included 9,500 shares of common stock, par value $0.001 per share, and 4,299 shares of preferred stock, par value $0.0001 per share. The Company's authorized preferred stock included 2,393 shares designated as Series A Preferred Stock and 1,906 shares designated as Series B Preferred Stock.

As of December 31, 2013, the Company was an LLC. The authorized units of the Company included 132,148 Class A Units, 119,656 Series A Preferred Units, and 95,315 Series B Preferred Units. Series A Preferred Units and Series B Preferred Units are herein collectively referred to as "Preferred Units."

Convertible Preferred Stock and Preferred Units

On December 31, 2010, the Company issued 119,656 Series A Preferred Units at a per unit price of $0.0251 for aggregate proceeds of $3,000. Upon the issuance of the Series A Preferred Units, holders of such units were entitled to a pre-tax cumulative internal rate of return of 8 percent per annum, compounding annually (the "Preferred Return"). Series A Preferred Units were redeemable by the holder upon the majority vote of all Series A Preferred Unit holders on a per unit basis on or after March 1, 2016. The redemption price of the Series A Preferred Units was equal to the original issue price of each unit held plus any amounts due under the Preferred Return on the redemption date. The holders of Series A Preferred Units were entitled to one vote per unit held on all matters brought before the members of the Company. In the event of liquidation, dissolution, or winding up of the Company, distributions were to be made, first, to the holders of Series A Preferred Units, pro rata at an amount equal to the original issue price of such units plus the amount to which the holders were entitled to under the Preferred Return; second, to the holders of Series A Preferred Units and Class A Units (discussed further below), pro rata in accordance with their respective percentage interests including Class B Units (discussed further below) held pursuant to the 2009 Equity Plan. In the event of an operating distribution, distributions were to be made first, to the holders of Series A Preferred Units, pro rata at an amount equal to the original issue price of such units plus the amount to which the holders were entitled to under the Preferred Return; second, to the holders of Series A Preferred Units and Class A Units, pro rata in accordance with their respective percentage interests.

On October 30, 2013, the Company issued 95,315 Series B Preferred Units at a per unit price of $0.082798 for an aggregate amount of $7,857, net of issuance costs of $35. The aggregate purchase price of $7,857 included $1,965 of net cash proceeds from new investors and the conversion $5,892 of debt under the Initial Conversion Agreement (Note 4) with FoxKiser.

Upon the issuance of the Series B Preferred Units, holders of Preferred Units were entitled to the Preferred Return. Preferred Units were redeemable by the holder upon the majority vote of all Preferred Unit holders on a per unit basis, on or after October 30, 2018. The redemption price of the Preferred Units was equal to the original issue price of each unit held plus any amounts due under the Preferred Return on the redemption date. The holders of Preferred Units were entitled to one vote per unit held on all matters brought before the members of

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the Company. In the event of liquidation, dissolution, or winding up of the Company, distributions were to be made, first, to the holders of Preferred Units, pro rata at an amount equal to the original issue price of such units plus the amount to which the holders were entitled to under the Preferred Return; second, to the holders of Preferred Units and Class A Units, pro rata in accordance with their respective percentage interests including Class B Units held pursuant to the 2009 Equity Plan. In the event of an operating distribution, distributions were to be made first, to the holders of Preferred Units, pro rata at an amount equal to the original issuance price of such units plus the amount to which the holders were entitled to under the Preferred Return; second, to the holders of Preferred Units and Class A Units, pro rata in accordance with their respective percentage interests.

Conversion to C-corporation

On September 16, 2014, the Company converted from an LLC to a C-corporation. Upon the conversion, Preferred Units were subject to a 50 to 1 reverse stock split, and converted into Series A Preferred Stock and Series B Preferred Stock of the Company. Specifically, the 119,656 Series A Preferred Units issued and outstanding on the conversion date were converted into 2,393 shares of Series A Preferred Stock, and the 95,315 Series B Preferred Units issued and outstanding on the conversion date were converted into 1,906 shares of Series B Preferred Stock.

Upon the conversion to a C-corporation and filing of the Company's Certificate of Incorporation on September 16, 2014, and as of December 31, 2014, rights, preferences, and privileges of Series A Preferred Stock and Series B Preferred Stock consisted of the following:

Dividends. The holders of Series A Preferred Stock and Series B Preferred Stock were entitled to receive dividends, in preference to common stock, on a pro rata basis, at a dividend rate equal to $0.1003 and $0.331192 per annum for each share of Series A Preferred Stock and Series B Preferred stock, respectively. Dividends were cumulative and accrued on each share of Series A Preferred Stock and Series B Preferred Stock from the respective original date of issuance of each share. After payment dividends to holders of Series A Preferred Stock and Series B Preferred Stock, any additional dividends were to be made to the holders of Series A Preferred Stock, Series B Preferred Stock, and common stock in proportion to the number of shares of common stock that would be held by each holder if all shares of Series A Preferred Stock and Series B Preferred Stock were converted to common stock at the then effective conversion rate. As of December 31, 2014 and June 30, 2015 (unaudited), the Company has not declared or paid any dividends or operating distributions since inception.

Liquidation Preference. In the event of a liquidation event, as defined below, either voluntary or involuntary, the holders of Series A Preferred Stock and Series B Preferred Stock had preference over common stock to any proceeds from liquidation at an amount equal to the original issuance price per share of Series A Preferred Stock and Series B Preferred Stock plus any accrued but unpaid dividends, whether declared or not, and any other declared but unpaid dividends. A liquidation event includes (i) the sale or disposition of substantially all of the Company's assets or the exclusive license of substantially all of the Company's intellectual property, (ii) a merger or consolidation in which the stockholders of the Company prior to the transaction no longer hold at least 50 percent of the voting power of the merged or consolidated entity, (iii) a transaction, or series of transactions, which results in a single party, or group of affiliated entities representing a single party, owning 50 percent of more the Company's equity securities, or (iv) a liquidation, dissolution, or winding up of the Company. For purposes of the liquidation preference, the original issuance price of Series A Preferred Stock and Series B Preferred Stock was $1.255 and $4.1399 per share, respectively. If proceeds from the liquidation event were insufficient to pay the entire liquidation preference to holders of Series A Preferred Stock and Series B Preferred Stock, then the proceeds were to be distributed ratably among the holders of Series A Preferred Stock and Series B Preferred Stock in proportion to the total preferential amount each holder was

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entitled to under the liquidation preference. Upon full payment of the liquidation preference, any remaining proceeds were to be distributed among the holders of Series A Preferred Stock and Series B Preferred Stock and common stock pro rata based on the number of shares of common stock, assuming full conversion of all Series A Preferred Stock and Series B Preferred Stock into common stock, held by each holder. For purposes of determining the amount each holder of Preferred Stock was entitled to receive with respect to a liquidation event, holders of Series A Preferred Stock and Series B Preferred Stock were deemed to have their shares converted into shares of common stock immediately prior to the liquidation event if, as a result of an actual conversion, the holder would receive an aggregate amount greater than the amount that would be distributed if the holder's preferred shares had not been converted into common stock. If the holder was deemed to have converted shares of Series A Preferred Stock and Series B Preferred Stock into shares of common stock for purposes of the liquidation preference, then the holder was not entitled to receive any distribution that would be made to holders of preferred shares that were not converted.

Redemption. Series A Preferred Stock and Series B Preferred Stock was redeemable upon the majority vote of all Series A Preferred Stock and Series B Preferred Stock holders on a per share basis, after October 30, 2018. The redemption price of the Series A Preferred Stock and Series B Preferred Stock was equal to the original issue price of each share held plus all accrued but unpaid dividends, whether or not declared, and was to be paid in three annual installments beginning on the first redemption date. For purposes of the redemption price, the original issuance price of Series A Preferred Stock and Series B Preferred Stock was $1.255 and $4.1399 per share, respectively.

Conversion. Each share of Series A Preferred Stock and Series B Preferred Stock was convertible at the option of the holder at any point in time into fully paid and non-assessable shares of common stock. Upon conversion, the Series A Preferred Stock and Series B Preferred Stock would be fully settled. Each share of Series A Preferred Stock and Series B Preferred Stock was convertible into that number of shares of common stock as determined by dividing the original issuance price of such share by the applicable conversion price. For purposes of determining the conversion rate, the original issuance price of Series A Preferred Stock and Series B Preferred Stock was $1.255 and $4.1399 per share, respectively. As of December 31, 2014, the conversion rate was 1:1, but was subject to future adjustments to the conversion price upon the occurrence of certain events including (i) certain future issuances of common stock at a price less than the conversion price in effect on the date of such issuance, and (ii) future stock splits, subdivisions, or combinations of outstanding common stock.

Each share of Series A Preferred Stock and Series B Preferred Stock would automatically convert into shares of common stock at the applicable conversion rate upon (i) a qualified public offering, as defined in the Certificate of Incorporation, at a per share price no less than $20.6995 per share (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) and $50,000 in aggregate gross proceeds, prior to deduction of underwriting discounts and commissions, or (ii) the majority vote of the holders of Series A Preferred Stock and Series B Preferred Stock on a per share and as-converted to common stock basis.

Voting. The holders of each share of Series A Preferred Stock and Series B Preferred Stock had the right to one vote for each share of common stock into which the shares could then be converted. Holders of Series A Preferred Stock and Series B Preferred Stock had full voting rights and powers equal to those of common stock holders.

As long as shares of Series A Preferred Stock remained outstanding, the holders of Series A Preferred Stock, voting as a separate class, were entitled to elect three directors to the Board of Directors. As long as shares of Series B Preferred Stock remained outstanding, the holders of Series B Preferred Stock, voting as a separate

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class, were entitled to elect one director to the Board of Directors. The holders of outstanding common stock, voting as a separate class, were entitled to elect one director to the Board of Directors. The holders of Preferred Stock and common stock, voting together as a single class on an as-converted basis for Preferred Stock, were entitled to elect any remaining directors of the Company.

The Company evaluated the conversion of Series A Preferred Units and Series B Preferred Units into Series A Preferred Stock and Series B Preferred Stock, respectively, giving consideration to all changes in the rights, preferences, and privileges of each class of securities. As a result of the conversion, holders of previously issued preferred units were given a conversion option to convert their units to common stock. Additionally, dividends on the newly issued preferred shares were no longer compounded annually as they were under the Preferred Return, which decreased the liquidation and redemption values of the securities. Management determined that the changes to these rights, privileges, and preferences should be accounted for as a modification of the securities.

Issuance of Series C Preferred Stock (unaudited)

On January 13, 2015, the Company completed the issuance and sale of 4,632 shares of Series C Preferred Stock, par value $0.0001 per share, at a per share price of $6.477 for aggregate gross proceeds of $30,000. The aggregate purchase price of $30,000 included $26,021 of cash proceeds, net of issuance costs of $187, and the conversion $3,792 of debt as discussed in Notes 4 and 11. Along with the issuance of the Series C Preferred Stock, the Company increased the number of shares reserved for future issuance under the 2014 Stock Plan to 2,800.

Extinguishment of Preferred Stock (unaudited)

In connection with the issuance of the Series C Preferred Stock in January 2015, the rights, preferences, and privileges of Series A Preferred Stock and Series B Preferred Stock then outstanding were modified. More specifically, Series C Preferred Stock received preference in dividends and liquidation proceeds over Series A Preferred Stock and Series B Preferred Stock. Additionally, the dividend rights changed from cumulative dividend rights to non-cumulative dividend rights, and all accrued but unpaid cumulative dividends on the Series A Preferred Stock and Series B Preferred Stock as of January 13, 2015 were forfeited. As a result of this modification, the redemption value and liquidation preferences of Series A Preferred Stock and Series B Preferred Stock, which were previously equal to original issue price plus accrued but unpaid cumulative dividends, were reduced to original issue price plus non-cumulative dividends declared. Additionally, the redemption date of Series A Preferred Stock and Series B Preferred Stock was changed from October 30, 2018 to December 31, 2019.

The Company has accounted for the amendment to the rights, preferences, and privileges of the Series A Preferred Stock and Series B Preferred Stock as an extinguishment of the original convertible preferred stock and issuance of new convertible preferred stock due to the significance of the modifications to the substantive contractual terms of the convertible preferred stock and the associated fundamental changes to the nature of the convertible preferred stock. Accordingly, upon extinguishment the Company recorded a loss of $1,317 on the Series A Preferred Stock and a gain of $2,076 on the Series B Preferred Stock within stockholders' equity (deficit) equal to the difference between the fair value of the new shares of preferred stock issued and the carrying amount of the old shares of preferred stock extinguished. The Company allocated the entire net gain on extinguishment of convertible preferred stock of $759 to additional paid-in capital. The net gain on extinguishment is reflected in the calculation of net loss available to common stockholders in accordance with FASB ASC Topic 260, *Earnings per Share*. The fair value of the Series A Preferred Stock and Series B Preferred Stock was determined using the option-pricing method ("OPM") back-solve method on the per share price of Series C Preferred Stock to estimate aggregate equity value. The OPM was used to allocate equity value to the Series A Preferred Stock and Series B Preferred stock using Black-Scholes option-pricing model.

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Issuance of Series D Preferred Stock (unaudited)

On May 15, 2015, the Company completed the sale and issuance of 7,367 shares of Series D Preferred Stock, par value $0.0001 per share, at a per share price of $9.5699 for proceeds of $67,998, net of issuance costs of $2,502. Along with the issuance of the Series D Preferred Stock, the Company increased the number of shares reserved for future issuance under the 2014 Stock Plan to 4,100.

In connection with the issuance of the Series D Preferred Stock, the Company amended and restated its Certificate of Incorporation. As of June 30, 2015 (unaudited), rights, preferences, and privileges of Preferred Stock consisted of the following:

Dividends. The holders of Series D Preferred Stock are entitled to receive dividends, in preference to Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and common stock when and if declared by the Board of Directors. After payment of dividends to holders of Series D Preferred Stock, holders of Series C Preferred Stock are entitled to receive dividends in preference to Series A Preferred Stock, Series B Preferred Stock, and common stock. After payment of dividends to holders of Series C Preferred Stock, holders of Series A Preferred Stock and Series B Preferred Stock are entitled to receive dividends in preference to common stock. Dividends to holders of Preferred Stock are non-cumulative and have a dividend rate equal to $0.1003, $0.331192, $0.51816, and $0.765592 per annum for each share of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock, respectively. After payment of dividends to holders of Preferred Stock, any additional dividends are to be made to the holders of Preferred Stock and common stock in proportion to the number of shares of common stock that would be held by each holder if all shares of Preferred Stock were converted to common stock at the then effective conversion rate.

Liquidation Preference. In the event of a liquidation event, as defined below, either voluntary or involuntary, the holders of Series D Preferred Stock have preference over Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and common stock to any proceeds from liquidation. Upon payment of the liquidation preference to holders of Series D Preferred Stock, holders of Series C Preferred Stock have preference in liquidation proceeds over holders of Series A Preferred Stock, Series B Preferred Stock, and common stock. Upon payment of the liquidation preference to holders of Series C Preferred Stock, holders of Series A Preferred Stock and Series B Preferred Stock have preference in liquidation proceeds over holders of common stock. Liquidation preferences of Preferred Stock are at an amount equal to the original issuance price per share of Preferred Stock plus any accrued but unpaid dividends, whether declared or not, and any other declared but unpaid dividends. A liquidation event includes (i) the sale or disposition of substantially all of the Company’s assets or the exclusive license of substantially all of the Company’s intellectual property, (ii) a merger or consolidation in which the stockholders of the Company prior to the transaction no longer hold at least 50 percent of the voting power of the merged or consolidated entity, (iii) a transaction, or series of transactions, which results in a single party, or group of affiliated entities representing a single party, owning 50 percent or more of the Company’s equity securities, or (iv) a liquidation, dissolution, or winding up of the Company. For purposes of the liquidation preference, the original issuance price of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is $1.255, $4.1399, $6.477, and $9.5699 per share, respectively. If proceeds from the liquidation event are insufficient to pay the entire liquidation preference to holders of any series of Preferred Stock, then the proceeds are to be distributed ratably among the holders of that series of Preferred Stock in proportion to the total preferential amount each holder is entitled to under the liquidation preference. Upon full payment of the liquidation preference, any remaining proceeds are to be distributed among the holders of Preferred Stock and common stock pro rata based on the number of shares of common stock, assuming full conversion of all Preferred Stock into common stock, held by each holder. For purposes of determining the amount each holder of Preferred Stock is entitled to receive with respect to a

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liquidation event, holders of Preferred Stock are deemed to have their shares of Preferred Stock converted into shares of common stock immediately prior to the liquidation event if, as a result of an actual conversion, the holder would receive an aggregate amount greater than the amount that would be distributed if the holder's Preferred Shares had not been converted into common stock. If the holder is deemed to have converted shares of Preferred Stock into shares of common stock for purposes of the liquidation preference, then the holder is not entitled to receive any distribution that would be made to holders of Preferred Stock that were not converted.

Redemption. As of June 30, 2015 (unaudited), all series of Preferred Stock are redeemable upon the majority vote of all Preferred Stock holders on a per share basis, after December 31, 2019. The redemption price of the Preferred Stock is equal to the original issue price of each share held plus all accrued but unpaid dividends, whether or not declared, and is to be paid in three annual installments beginning on the first redemption date. For purposes of the redemption price, the original issuance price of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is $1.255, $4.1399, $6.477, and $9.5699 per share, respectively.

Conversion. Each share of Preferred Stock is convertible at the option of the holder at any point in time into fully paid and non-assessable shares of common stock. Upon conversion, the Preferred Stock is fully settled. Each share of Preferred Stock is convertible into that number of shares of common stock as determined by dividing the original issuance price of such share by the applicable conversion price. For purposes of determining the conversion rate, the original issuance price of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock is $1.255, $4.1399, $6.477, and $9.5699 per share, respectively. As of June 30, 2015 (unaudited), the conversion rate was 1:1 for each series of Preferred Stock, but was subject to future adjustments to the conversion price upon the occurrence of certain events including (i) certain future issuances of common stock at a price less than the conversion price in effect on the date of such issuance, and (ii) future stock splits, subdivisions, or combinations of outstanding common stock.

Each share of Preferred Stock shall automatically convert into shares of common stock at the applicable conversion rate upon (i) a qualified public offering, as defined in the Certificate of Incorporation, of at least $40,000 in aggregate gross proceeds, prior to deduction of underwriting discounts and commissions, or (ii) the majority vote of the holders of Preferred Stock on a per share and as-converted to common stock basis.

Voting. The holders of each share of Preferred Stock has the right to one vote for each share of common stock into which the Preferred Stock could then be converted. Holders of Preferred Stock have full voting rights and powers equal to those of common stock holders.

As long as shares of Series A Preferred Stock remain outstanding, the holders of Series A Preferred Stock, voting as a separate class, are entitled to elect three directors to the Board of Directors. As long as shares of Series B Preferred Stock remain outstanding, the holders of Series B Preferred Stock, voting as a separate class, are entitled to elect one director to the Board of Directors. As long as shares of Series C Preferred Stock remain outstanding, the holders of Series C Preferred Stock, voting as a separate class, are entitled to elect one director to the Board of Directors. As long as shares of Series D Preferred Stock remain outstanding, the holders of Series D Preferred Stock, voting as a separate class, are entitled to elect one director to the Board of Directors. The holders of outstanding common stock, voting as a separate class, are entitled to elect one director to the Board of Directors. The holders of Preferred Stock and common stock, voting together as a single class on an as-converted basis for Preferred Stock, are entitled to elect any remaining directors of the Company.

Common Stock, Class A Units, and Class B Units

The Company's authorized Class A Units were initially issued for the contributions of the various members, which included capital, services, and intellectual property. Additionally, certain members received Class A Units

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in accordance with anti-dilution provisions in the Company's LLC agreement. In conjunction with the October 30, 2013 issuance of Series B Preferred Units, certain holders of Class A Units received an aggregate of 19,476 additional Class A Units as a result of the anti-dilution provisions. Upon the completion of the Series B Preferred Units issuance, all anti-dilution provisions had been fully utilized and were no longer outstanding.

Upon the Company's conversion from an LLC to a C-corporation on September 16, 2014, the 132,148 of then issued and outstanding Class A Units were subject to a 50-to-1 reverse unit split, and converted into 2,643 shares of common stock.

Upon the conversion to a C-corporation and filing of the Company's Certificate of Incorporation on September 16, 2014, and as of December 31, 2014 and June 30, 2015 (unaudited), dividend, liquidation, and voting rights of the holders of shares of common stock are subject to and qualified by the rights, preferences, and privileges of the holders of shares of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock. The common stock has the following characteristics:

Dividends. The holders of common stock are entitled to receive dividends, if and when declared by the Board of Directors. Cash dividends may not be declared or paid to holders of common stock until paid on each series of outstanding Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock in accordance with their respective terms.

Liquidation. After payment to the holders of Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock and Series D Preferred Stock, of their liquidation preferences, the holders of common stock are entitled to share ratably in the Company's assets available for distribution to stockholders, in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon the occurrence of a liquidation event, as defined by the Certificate of Incorporation.

Voting. The holders of shares of common stock are entitled to one vote for each share of common stock held.

Reserved for Future Issuance. The Company's reserved shares of common stock for future issuance related to potential conversion of the Series A Preferred Stock, Series B Preferred Stock, Series C Preferred Stock, and Series D Preferred Stock, exercise of stock options, and share settlement of debt as of December 31, 2014 and June 30, 2015 (unaudited) are as follows:

	December 31, 2014	June 30, 2015 (unaudited)
Series A convertible preferred stock	2,393	2,393
Series B convertible preferred stock	1,906	1,906
Series C convertible preferred stock	—	4,632
Series D convertible preferred stock	—	7,367
Options to purchase common stock	2,500	3,990
Debt with share settlement option (Note 4)	3,715	—
	10,514	20,288

Class B Units. On December 9, 2009, the Company entered into the 2009 Equity Incentive Plan. Under the 2009 Equity Incentive Plan, which was administered by the Board of Managers, the Company was authorized to grant Class B Units. Class B Units were designed to provide equity incentive compensation to managers,

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employees, advisory board members, and consultants of the Company, with such terms and conditions including vesting and forfeiture determined by the Board of Managers in its sole discretion. The Class B Units represented a profits interest in the Company as that term is defined in the Internal Revenue Code. The holders of Class B Units had no voting power and were only eligible for distributions resulting from a qualified liquidity event of the Company, as defined by the LLC agreement, and if the proceeds from such event exceed a fixed distribution threshold per unit. Distribution thresholds were determined by the Board of Managers for each Class B Unit awarded. In the case of a qualified public offering, as defined in the 2009 Equity Incentive Plan, the Class B Units would convert into shares of restricted common stock and continue to vest in accordance with each award agreement. The Class B Units were non-transferable and upon termination of service, the Company had the option to purchase all vested units from the award holder. The Company did not repurchase any Class B Units.

As of December 31, 2013, the Company had authorized up to 24,500 Class B Units for issuance, 22,828 of which were issued and subject to vesting conditions set forth in each Class B Unit award. Upon the Company's conversion from an LLC to a C-corporation on September 16, 2014, all outstanding Class B Units were terminated along with the 2009 Equity Incentive Plan, and the Company executed the 2014 Stock Plan and granted stock options. See Note 8 for further information regarding the 2014 Stock Plan.

Management evaluated the Class B Unit awards and determined that they should be accounted for as a share-based payments in accordance with ASC 718. However, since no distribution is to be made to Class B Unit holders unless a qualified liquidity event occurs, management has determined that the awards are subject to both a service condition (vesting period) and a performance condition (qualified liquidity event). Additionally, the awards only convert into restricted common stock upon the event of a qualified public offering. Management did not consider a qualified liquidity event or public offering to be probable at any point during the outstanding terms of the Class B Units, and accordingly, no compensation expense was recorded in connection with the awards.

The Company accounted for the termination of the Class B Units and simultaneous grant of stock options under the 2014 Stock Plan as a modification to share-based payments under ASC 718. Since the performance conditions under the Class B Unit awards were deemed improbable of achievement, no incremental compensation cost from the modification is recognized. See Note 8 for information on stock-based compensation expense regarding stock options issued by the Company in 2014.

7. Significant Agreements

See Note 11 for significant agreements with related parties and Note 5 for license agreements granted to the Company.

License Agreements

The Company has granted a number of intellectual property licenses to other biotechnology and pharmaceutical companies. The terms of the licenses vary, however licenses may be exclusive or non-exclusive and may be sublicensable by the licensee. Licenses may grant intellectual property rights for purposes of internal and preclinical research and development only, or may include the rights, or options to obtain future rights, to commercialize drug therapies for specific diseases. License agreements generally have a term equal to the life of the underlying patents and are terminable only at the option of the licensee. License agreements may require licensees to pay non-refundable up-front fees, option fees, and annual maintenance fees. Additional contingent consideration under the licenses may include sublicense fees, milestone fees, and royalties on net sales of commercialized products. Sublicense fees vary by license and range from a mid-single-digit percentage to a low-double-digit percentage of license fees received by licensees as a result of sublicenses. Royalties on net sales of commercialized products vary by license and range from a mid-single-digit percentage to a low-teen percentage of net sales by licensees.

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Milestone fees are payable to the Company upon specific clinical and regulatory developments by licensees. As of December 31, 2014, the Company's current license agreements, excluding additional licenses that could be granted upon the exercise of options by licensees, could result in aggregate milestone fees payable to the Company of up to $500 upon the submission of preclinical regulatory filings, $8,550 upon the commencement of various stages of clinical trials in humans, $17,000 upon the submission of regulatory approval filings, and $39,500 upon the approval of commercial products by regulatory agencies.

On July 19, 2013, the Company granted an exclusive commercial license to Audentes. The license required an up-front fee of $600, $300 of which was payable in cash and the remainder in common stock of Audentes. As discussed in Note 2, the investment in Audentes is accounted for under the cost method. The carrying value of the equity investment in Audentes was $300 at December 31, 2013 and 2014, and June 30, 2015 (unaudited), and is included in cost method investments on the balance sheets.

During the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), the Company recognized license revenue from up-front fees and option fees for commercial licenses of $3,400, $4,150, $3,600, and $1,000, respectively, as well as license revenue from maintenance fees and research licenses of $355, $425, $105, and $320, respectively, under the license agreements. As of December 31, 2014, since inception, the Company had not recognized any revenue related to milestone fees under the license agreements. During the six months ended June 30, 2015 (unaudited), the Company recognized license revenue from milestone fees of $250. As of December 31, 2014 and June 30, 2015 (unaudited), since inception, the Company has not recognized any revenue under the license agreements related to sublicense fees or royalties on net sales. As of December 31, 2014 and June 30, 2015 (unaudited), the Company had accounts receivable of $200 and $130, respectively, related to the license agreements. As of December 31, 2013, the Company had no accounts receivable related to the license agreements.

Grant Programs

MeuSIX. In December 2012, as part of a consortium of research and development entities called MeuSIX, the Company was awarded a long-term grant by the European Commission's Seventh Framework Program, to perform preclinical and clinical research and development services for the treatment of MPS VI, a severe lysosomal storage disorder. Under the grant agreement, the Company is reimbursed by the grantor for 75 percent of qualified research and development costs, up to approximately €2,273 (approximately $2,927 based on the average conversion rate for the grant period to date through June 30, 2015) of such costs over the five year grant period. Funds received under the grant are subject to refund in the case of non-compliance with the provisions of the grant, which include, but are not limited to, the eligibility of costs, calculation of personnel rates, selection of subcontractors, and other provisions. As of December 31, 2014 and June 30, 2015 (unaudited), the Company is in compliance with all provisions of the grants and no refunds are payable to the grantor. During the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), the Company incurred $882, $1,109, $427, and $761, respectively, of research and development costs under the grant program. The Company recorded grant revenue of $661, $832, $320, and $243 related to the grant program for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), respectively. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had $62, $865, and $536 respectively, of accounts receivable under the grant program, of which $51, $320, and $0 respectively, is included in unbilled receivables on the balance sheets.

Federal Grants. The Company has received grant awards from agencies of the U.S. federal government to support critical research and development projects for the Company. In 2010, the Company was awarded two grants from the National Institute of Health ("NIH") in amounts totaling $3,063. In 2012, the Company was

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awarded two additional grants from the NIH totaling $515. In 2013, the Company was awarded an additional grant from the NIH totaling $261. Funds received under the grants are subject to refund in the case of non-compliance with the provisions of the grant, which include, but are not limited to, the eligibility of costs, calculation of personnel rates, selection of subcontractors, and other provisions. As of December 31, 2014 and June 30, 2015 (unaudited), the Company is in compliance with all provisions of the grants and no refunds are payable to the grantor. As a result of the NIH grants, the Company has recorded revenue from reimbursement of qualified research and development costs of $1,303, $387, $170, and $46 for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), respectively. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had $61, $51, and $0, respectively, of accounts receivable under the NIH grants. As of January 2015, all NIH grants were completed.

8. Stock-based Compensation

On September 16, 2014, the Board of Directors adopted the 2014 Stock Plan (the “Plan”). As of December 31, 2014 and June 30, 2015 (unaudited), the number of shares of common stock authorized for issuance under the Plan was 2,500 and 4,100, respectively.

The Plan provides for the issuance of stock options, restricted stock awards, and unrestricted stock awards to employees, members of the Board of Directors, and consultants of the Company. The Company has not granted restricted or unrestricted stock awards under the Plan since its inception. Options generally expire ten years following the date of grant. Options typically vest over a period of four years, but vesting provisions can vary by award based on the discretion of the Board of Directors. Certain awards issued by the Company include performance conditions that must be achieved in order for vesting to occur. Options to purchase common stock carry an exercise price equal to the estimated fair value of the Company’s common stock on the date of grant. Generally options to purchase shares of the Company’s common stock are exercised by payment of the exercise price in cash. Upon the termination of service, except by death or disability, of a holder of stock options awarded under the Plan, all unvested options are forfeited and vested options may be exercised within three months of termination by the holder. Shares of common stock issued as a result of awards under the Plan may be subject to repurchase provisions as designated in each individual award agreement.

Shares of common stock underlying awards previously issued under the Plan which are reacquired by the Company, withheld by the Company in payment of the purchase price, exercise price, or withholding taxes, expired, cancelled due to forfeiture, or otherwise terminated other than by exercise, are added to the number of shares of common stock available for issuance under the Plan. Shares available for issuance under the Plan may be authorized but unissued shares of the Company’s common stock or common stock reacquired by the Company and held in treasury. The Plan expires in September 2024, ten years from the date it was approved by the Board of Directors.

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The following table summarizes stock option activity under the Plan:

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value(a)
Outstanding at December 31, 2013	—	$ —	—	$ —
Granted	2,132	$ 0.85		
Exercised	(2)	$ 0.85		
Cancelled or forfeited	(23)	$ 0.85		
Outstanding at December 31, 2014	2,107	$ 0.85	9.8	$ 379
Granted (unaudited)	1,064	$ 3.76		
Exercised (unaudited)	(108)	$ 0.85		
Cancelled or forfeited (unaudited)	—			
Outstanding at June 30, 2015 (unaudited)	3,063	$ 1.86	9.5	$ 15,436
Exercisable at December 31, 2014	759	$ 0.85	9.7	$ 137
Vested and expected to vest at December 31, 2014	2,107	$ 0.85	9.7	$ 379
Exercisable at June 30, 2015 (unaudited)	878	$ 1.22	9.3	$ 4,986
Vested and expected to vest at June 30, 2015 (unaudited)	3,063	$ 1.86	9.5	$ 15,436

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the estimated fair value of the common stock for the options that were in the money at December 31, 2014 and June 30, 2015 (unaudited)

As of December 31, 2014 and June 30, 2015 (unaudited), 391 and 927 shares of common stock, respectively, were available for future grants under the Plan. The weighted-average grant date fair value of options granted during the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited) was $0.51 and $2.27, respectively. During the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited), the total number of stock options exercised was 2 and 108, respectively, resulting in total proceeds of $1 and $92, respectively. The total intrinsic value of options exercised during the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited) was $0 and $314, respectively.

Stock-based compensation expense for the year ended December 31, 2014 and six months ended June 30, 2015 (unaudited) relates solely to stock options granted under the Plan. The Company has recorded aggregate stock-based compensation expense related to the issuance of stock option awards to employees and non-employees in the statement of operations for the year ended December 31, 2014 and six months ended June 30, 2015 (unaudited) as follows:

	December 31, 2014	June 30, 2015 (unaudited)
Research and development	$ 60	$ 312
General and administrative	259	399
	$ 319	$ 711

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There were no options granted or outstanding prior to the year ended December 31, 2014. No stock-based compensation expense was recorded for the year ended December 31, 2013 and the six months ended June 30, 2014 (unaudited).

Valuation of Common Stock. The Company estimates the fair value of common stock underlying stock option awards at the grant date of the award. Valuation estimates are prepared by management in accordance with the framework of the *American Institute of Certified Public Accountants Practice Aid*, *Valuation of Privately-Held-Company Equity Securities Issued as Compensation* (the "AICPA Practice Guide"), as well as through independent third-party valuations, and are approved by the Company's Board of Directors.

July 31, 2014 Valuation. Stock options granted by the Company during the year ended December 31, 2014 assumed a fair value of underlying common stock based on a valuation performed as of July 31, 2014. For the July 31, 2014 valuation, the Company estimated aggregate equity value of the business using a combination of the market multiple approach (20% weighting) and back-solve method of the OPM (80% weighting).

The market multiple approach estimates the fair value of a company by applying market multiples of comparable publicly-traded companies and publicly disclosed financial data to arrive at estimated fair value. The Company applied a market multiple of revenue of comparable publicly-traded companies to its estimated revenue for the year ended December 31, 2014 to arrive at an estimated equity value. Consideration was given to differences between the Company and the selected guideline public companies in terms of size, anticipated profitability, market size, and other critical characteristics that generally reflect an investor's assessment of the business and financial risks inherent in the industry.

The OPM back-solve method derives the implied equity value for one type of equity security from a contemporaneous transaction involving another type of equity security. The Company applied the OPM back-solve method to solve for the equity value and corresponding value of common stock based on the price per unit of the Series B Preferred Units issued in October 2013.

The OPM treats common stock and convertible preferred stock as call options on an equity value, with exercise prices based on the liquidation preference of the convertible preferred stock. Therefore, the common stock has value only if the funds available for distribution to the stockholders exceed the value of the liquidation preference at the time of a liquidity event such as a merger, sale, or IPO, assuming the enterprise has funds available to make a liquidation preference meaningful and collectible by the stockholders. The common stock is modeled to be a call option with a claim on the enterprise at an exercise price equal to the remaining value immediately after the convertible preferred stock is liquidated. The OPM uses the Black-Scholes option-pricing model to price the call options. The OPM is appropriate to use when the range of possible future outcomes is so difficult to predict that forecasts would be highly speculative.

The following table summarizes the significant assumptions used in the OPM to determine the fair value of the Company's common stock of $0.85 per share as of July 31, 2014:

Years to liquidity event	3.0
Annual volatility	65%
Risk-free interest rate	1.0%
Discount for lack of marketability	41.0%

April 30, 2015 Valuation. Stock options granted by the Company during the six months ended June 30, 2015 (unaudited) assumed a fair value of underlying common stock based on a valuation performed as of April 30, 2015. For the April 30, 2015 valuation, the Company used a hybrid of the probability-weighted expected return method ("PWERM") (15% weighting), and the OPM (85% weighting), which is referred to as the hybrid method.

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Under the PWERM, share value is derived from the probability-weighted present value of expected future investment returns, considering possible outcomes available to the Company, as well as the economic and control rights of each share class. The PWERM in the Company's April 30, 2015 valuation assumes an IPO date five months from the valuation date based on the Board of Directors' assessment of the Company's prospects, the Company's investors' motivations, and market conditions. The PWERM considers two possible outcomes: (i) a future equity value upon an IPO at the high end of an estimated range and (ii) a future equity value upon an IPO at the lower end of an estimated range. In order to estimate the range of potential future equity values upon an IPO for the PWERM, the Company considered the pre-money enterprise values at the IPO date of comparable companies which had undergone IPOs in recent periods prior to April 30, 2015. The Company placed a 35% weighting on the higher end of the range of expected future equity values, and a 65% weighting on the lower end of the range, based on the stage of development of its internal drug candidates versus the comparable publicly-held companies which generally had further developed drug pipelines at the date of their IPOs. The future equity value at the expected IPO date under each scenario was allocated to each series of Preferred Stock and common stock assuming conversion of all preferred series to common. The Company then discounted the values of each class of equity in the IPO scenarios at an appropriate risk-adjusted rate. The Company assumed a risk-adjusted rate of 20% for the common shares. The risk-adjusted rates were based on studies of the rates of return expected by venture capital investors, as presented in the AICPA Practice Aid.

Under the PWERM, the Company applied a discount for lack of marketability ("DLOM") to the value indicated for its common stock. The Company's estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount between 11% and 20%, which was used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

Under the OPM, the Company applied the OPM back-solve method to solve for the equity value and corresponding value of common stock based on the price per share of its Series D Preferred Stock issued in May 2015. Given the proximity to the Series D Preferred Stock financing, and the fact that the Series D Preferred Stock issuance included and was led by unrelated investors, the Company believes the per share issuance price of the Series D Preferred Stock provides an indication of the fair value of its equity as of April 30, 2015. The values indicated for the preferred and common shares by the IPO scenario and the OPM scenario were probability weighted to calculate the weighted value as of the April 30, 2015 valuation date.

Under the OPM, the Company estimated the time to liquidity as 2.5 years based on then-current plans and estimates of the Board of Directors and management regarding a liquidity event. The anticipated timing of a liquidity event was management's estimate in the event that the Company's planned IPO does not occur. The risk-free rate was estimated as the interpolated 2.5 year yield on government bonds.

Under the OPM, the Company estimated volatility to be 82% at the valuation date. To arrive at this number, historical volatilities of comparable publicly-traded companies were analyzed, most of which are significantly more developed than the Company.

Under the OPM, the Company applied a DLOM to the value indicated for its common stock. The estimate of the appropriate DLOM took into consideration put option methodologies consistent with the AICPA Practice Aid. A put option model indicated a discount between 29% and 57%, which was used as an appropriate range for a DLOM. To arrive at the selected DLOM, qualitative adjustments were made based on empirical data from generally accepted studies on restricted stock and pre-IPO discounts.

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For the April 30, 2015 valuation, the Company estimated the fair value of its common stock by assigning an 85% weighting to the estimated fair value using the OPM back-solve method and a 15% weighting to the PWERM method. The Company believes that the 85% weighting on the OPM back-solve method is appropriate due to the proximity of the sale and issuance of Series D Preferred Stock in May 2015. The 15% weighting for the IPO scenario was deemed appropriate because at the time of the valuation, the Company believed that there was the possibility of following a successful Series D Preferred Stock financing with an IPO.

The following table summarizes the significant assumptions used to determine the fair value of the Company's common stock of $3.76 per share as of April 30, 2015 using the hybrid method:

	OPM	PWERM
Weighting	85%	15%
Equity value	$130,700	$338,100
Years to liquidity event	2.5	0.4
Annual volatility	82%	N/A
Risk-free interest rate	0.75%	N/A
Weighted average cost of capital	N/A	20%
Discount for lack of marketability	35%	15%
Estimated per share fair value of common stock	$ 2.08	$ 13.25

Stock Options Granted to Employees. For the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited), the Company recorded $299 and $308, respectively, of stock-based compensation expense related to employees' stock options. The fair value of options granted to employees was estimated at the date of grant using the Black-Scholes valuation model with the following weighted-average assumptions for the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited):

	December 31, 2014	June 30, 2015 (unaudited)
Expected volatility	64%	64%
Expected term (in years)	6.0	6.1
Risk-free interest rate	2.0%	1.7%
Expected dividend yield	0.0%	0.0%

As of December 31, 2014 and June 30, 2015 (unaudited), there was $692 and $2,488, respectively, of unrecognized stock-based compensation expense related to employees' awards that is expected to be recognized over a weighted-average period of 3.8 and 3.5 years, respectively.

Stock Options Granted to Non-employees. Stock-based compensation expense related to stock options granted to non-employees is recognized as the stock options are earned. The Company believes that the estimated fair value of the stock options is more readily measurable than the fair value of the services rendered. For the year ended December 31, 2014 and the six months ended June 30, 2015 (unaudited), the Company recorded $20 and $403, respectively, of stock-based compensation expense related to non-employees' stock options, which is included in research and development expense in the statements of operations.

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The Company used the following weighted-average assumptions in estimating non-employees stock-based compensation expense:

	December 31, 2014	June 30, 2015 (unaudited)
Expected volatility	65%	67%
Expected term (in years)	9.9	9.8
Risk-free interest rate	2.4%	1.7%
Expected dividend yield	0.0%	0.0%

9. Retirement Plan

As of December 31, 2013 and 2014, the Company did not sponsor any retirement plans. In February 2015, the Company established a defined-contribution retirement plan under Section 401(k) of the Internal Revenue Code ("the 401(k) Plan"). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pre-tax basis. The Company matches employee deferrals up to 5.75 percent of eligible compensation. For the six months ended June 30, 2015 (unaudited), the Company contributed $64 in matching contributions to the 401(k) Plan.

10. Income Taxes

From inception through September 16, 2014, the Company was a Delaware LLC for federal and state income tax purposes, and therefore, all items of income or loss through September 16, 2014 flowed through to the members of the LLC. Effective September 16, 2014, the Company converted from an LLC to a C-corporation for federal and state income tax purposes. Prior to the conversion to a C-corporation, the Company did not record deferred tax assets or liabilities or have any net operating loss ("NOL") carryforwards for federal income tax purposes. However, as of December 31, 2013, the Company had recorded a deferred tax asset for state income taxes, which consisted primarily of NOL carryforwards for state jurisdictions that did not recognize the Company's LLC status.

Effective upon the conversion to a C-corporation, the Company became subject to income tax at the federal and state levels. Accordingly, as of December 31, 2014, the Company recorded a deferred tax asset for federal and state income taxes, which consists primarily of NOL carryforwards.

As all of the Company's income is generated in the U.S., and attributable to the U.S. jurisdiction, there are no foreign income tax expenses for the years ended December 31, 2013 and 2014.

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The Company did not record a current or deferred income tax expense or benefit for the years ended December 31, 2013 and 2014. Since the Company was an LLC for the year ended December 31, 2013, the Company was not subject to federal income tax. A reconciliation of income tax expense (benefit) computed at the statutory federal income tax rate of 34 percent for the year to income tax expense (benefit) as reflected in the financial statements for the years ended December 31, 2013 and 2014 is as follows:

	2013	2014
Federal income tax expense (benefit) at statutory rate	$ —	$(1,089)
State income tax expense (benefit), net of federal benefit	(537)	(264)
Change in income tax rates upon conversion to C corp	—	427
Federal deferred tax assets upon conversion to C corp	—	(105)
Step-up in assets upon conversion to C corp	—	(448)
Stock-based compensation expense for incentive stock options	—	98
Imputed interest on related party promissory notes	—	52
Taxable gain upon conversion to C corp	—	73
Other non-deductible expenses	—	4
Change in valuation allowance	537	1,252
Total tax expense (benefit)	$ —	$ —

The significant components of the Company's deferred tax assets as of December 31, 2013 and 2014 are as follows:

	2013	2014
Deferred tax assets:		
Net operating loss carryforwards	$ 1,031	$ 1,844
Step-up in assets upon conversion to C corp	—	438
Stock-based compensation expense for non-qualified stock options	—	31
Accruals and other	72	42
Total deferred tax assets	1,103	2,355
Valuation allowance	(1,103)	(2,355)
Net deferred tax assets	$ —	$ —

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for deferred tax assets as of December 31, 2013 and 2014, and June 30, 2015 (unaudited). The valuation allowance increased approximately $537 and $1,252 during the years ended December 31, 2013 and 2014, respectively, due primarily to the conversion to a C-corporation and the federal and state net operating losses generated during the periods.

As of December 31, 2013 and 2014, the Company had U.S. federal NOL carryforwards of approximately $0 and $2,979, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2034. As of December 31, 2013 and 2014, the Company also had U.S. state NOL carryforwards of approximately $10,341 and $13,406, respectively, which may be available to offset future income tax liabilities and expire at various dates through 2034.

Under the provisions of the Internal Revenue Code, the NOL carryforwards are subject to review and possible adjustment by the Internal Revenue Service and state tax authorities. NOL carryforwards may become

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subject to an annual limitation in the event of certain cumulative changes in the ownership interest of significant shareholders over a three-year period in excess of 50 percent, as defined under Sections 382 and 383 of the Internal Revenue Code, respectively, as well as similar state provisions. This could limit the amount of tax attributes that can be utilized annually to offset future taxable income or tax liabilities. The amount of the annual limitation is determined based on the value of the Company immediately prior to the ownership change. Subsequent ownership changes may further affect the limitation in future years. The Company has completed several financings since its inception which may have resulted in a change in control as defined by Sections 382 and 383 of the Internal Revenue Code, or could result in a change in control in the future.

The Company files income tax returns in the United States at the federal level and in states in which the Company conducts business activities. The federal and state income tax returns are generally subject to tax examinations for the tax years ended December 31, 2011 through December 31, 2014. To the extent the Company has tax attribute carryforwards, the tax years in which the attribute was generated may still be adjusted upon examination by the Internal Revenue Service or state tax authorities to the extent utilized in a future period.

11. Related Party Transactions

The Trustees of the University of Pennsylvania

On February 20, 2009, the Company entered into a license agreement, as amended, with The Trustees of the University of Pennsylvania ("Penn") for exclusive, worldwide rights to certain patents owned by Penn. Under the terms of the agreement, in consideration for the license the Company issued to Penn 24.5 percent of then outstanding membership interest in the LLC on a fully diluted basis after issuance. The Company is obligated to pay Penn royalties on net sales, and sublicense fees, if any. Additionally, the Company is obligated to reimburse Penn for certain costs incurred related to the maintenance of the licensed patents. Penn also provides manufacturing services and research and development services to the Company.

Expenses incurred by the Company under its license from Penn, as well as for manufacturing and research and development for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), are as follows:

	December 31,		June 30,	
	2013	2014	2014	2015
			(unaudited)	
Sublicense fees	$ 76	$ 443	$ 371	$ 157
Royalties on sales of reagents	18	17	12	6
Maintenance of licensed patents	120	256	158	200
Manufacturing of reagents for sale	143	92	82	40
Research and development services	2,778	1,286	482	2,644
	$3,135	$2,094	$1,105	$3,047

Sublicense fees are included in licensing costs to related parties in the statements of operations. Royalties on sales of reagents and manufacturing of reagents for sale are included in costs of reagent sales in the statements of operations. Maintenance of licensed patents is included in general and administrative expenses in the statements of operations. Research and development services are included in research and development expenses in the statements of operations. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had accrued $1,986, $1,732, and $163, respectively, in expenses payable to Penn which are included in other related party payables on the balance sheets.

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GlaxoSmithKline LLC

On March 6, 2009, the Company entered into a license agreement, as amended, with GlaxoSmithKline LLC ("GSK") for exclusive, worldwide rights to certain patents owned by Penn and exclusively licensed to GSK. Under the terms of the agreement, in consideration for the license the Company issued to GSK 19.9 percent of then outstanding membership interest in the LLC on a fully diluted basis after issuance. The Company is obligated to pay GSK royalties on net sales, and sublicense fees, if any. Additionally, the Company is obligated to reimburse GSK for certain costs incurred and invoiced to the Company related to the maintenance of the licensed patents. The Company is obligated pay GSK up to $1,650 upon the achievement of various milestones. As of June 30, 2015 (unaudited), no milestones have been achieved and accordingly no milestone payments were payable to GSK which are included in other related party payables on the balance sheets.

Expenses incurred by the Company under its license from GSK for the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), are as follows:

	December 31,		June 30,	
	2013	2014	2014	2015
			(unaudited)	
Sublicense fees	$ 76	$443	$371	$157
Royalties on sales of reagents	11	10	7	4
Maintenance of licensed patents	455	432	216	417
	$542	$885	$594	$578

Sublicense fees are included in licensing costs to related parties in the statements of operations. Royalties on sales of reagents are included in costs of reagent sales in the statements of operations. Maintenance of licensed patents is included in general and administrative expenses in the statements of operations. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited) the Company had accrued $1,517, $2,028, and $1,756, respectively, payable to GSK under the license agreement.

Dimension Therapeutics, Inc.

On October 30, 2013, the Company granted an exclusive, sublicensable, worldwide commercial license to Dimension for preclinical and clinical research and development, and commercialization of drug therapies using the Company's licensed patents for the treatment of hemophilia A and hemophilia B, as well as a one year option to obtain exclusive licenses for the commercialization of two other diseases to be elected by Dimension in the future. The agreement requires on-going annual maintenance fees of $35, for each indication elected by Dimension, beginning in October 2014. The agreement also requires Dimension to pay royalties on net sales, if any, to the Company at an amount intended to approximate the royalties that will be due by the Company to Penn and GSK on such sales. In consideration for the license granted, Dimension issued the Company, and various members, directors, and executives of the Company, an aggregate total of 10,000 shares of its common stock, with an estimated fair value of $2,700. The Company recorded $2,700 as revenue upon delivery of the license. Of the 10,000 shares, a total of 10 shares were issued to the Company, with an estimated fair value of $3, which is included in cost method investments on the balance sheets. In consideration for the efforts by the various members, directors, and executives of the Company which were responsible for executing the license agreement with Dimension, the Company recorded expenses equal to the estimated fair value of the 9,990 shares of common stock of Dimension received by those parties of $2,697, which is included in general and administrative expenses in the statements of operations. In accordance with its revenue recognition policy, the Company has determined that the $2,700 in revenue from the license granted to Dimension should be recognized in full upon

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the delivery of the license, as the Company has no further significant performance obligations under the agreement. Additionally, the Company determined that the $2,697 of general and administrative expenses to related parties should be recognized in full upon the execution of the agreement with Dimension, as those parties have no further performance obligations to the Company.

In addition to related parties of the Company holding common stock in Dimension as a result of the license agreement noted above, three of the Company's board members served on the board of directors of Dimension on the effective the date of the license. Management has evaluated consolidation guidance under ASC 810 and determined that Dimension is considered a variable interest entity, however, it does not consolidate Dimension because it lacks the power to direct the activities of the VIE that most significantly impact the VIE's economic performance. The Company holds an equity interest in Dimension and also has a license agreement granting Dimension the right to use the Company's intellectual property. The carrying amount of the investment in Dimension as of December 31, 2013 and 2014, and June 30, 2015 (unaudited) was $3 and the receivables due from Dimension as of December 31, 2013 and 2014, and June 30, 2015 (unaudited) were $924, $750, and $0. The Company believes it is not exposed to any significant losses or off-balance sheet risk as a result of its involvement in Dimension, and the Company's equity at risk in Dimension is not material to the financial statements.

In connection with the license agreement granted to Dimension, the Company entered into an arrangement with Penn and Dimension in which the Company helped coordinate and manage research and development activities performed by Penn on behalf of Dimension. Under the arrangement, Dimension reimbursed the Company at an amount equal to costs incurred and paid to Penn, and the Company retains rights to certain intellectual property discovered under the contracted research and development. Due to the uncertainty of any future intellectual property rights that may be discovered and retained by the Company, and because such intellectual property would have no future alternative use due to the stage of development of the drug therapies under development, the Company has not recognized any benefit from the arrangement as consideration paid by Dimension to the Company as a result of the license agreement. Management has evaluated the facts and circumstances of the arrangements with regards to ASC 605-45 *Revenue Recognition-Principal Agent Considerations* and determined that the proceeds received from Dimension should be recorded on a net basis. Accordingly, proceeds received from Dimension under the arrangement were recorded as a reduction of research and development expense in the statements of operations. For the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), the Company recorded research and development expense to Penn, and related reimbursements from Dimension of $924, $6,177, $1,353, and $0, respectively, for a net cost of $0 for all periods. As of December 31, 2013 and 2014, and June 30, 2015 (unaudited), the Company had recorded payables of $924, $750, and $0, respectively, to Penn and related party receivables of $924, $750, and $0, respectively, from Dimension under the arrangement. As of June 30, 2015 (unaudited), the final payments under this arrangement were received by the Company and paid to Penn, and the arrangement was ended.

In September 2014, Dimension elected its third indication under the license agreement, and the license was amended to extend the term of the option to elect the fourth and final disease indication for an additional six months. In consideration for the extension of the option, Dimension paid an extension fee of $150. In January 2015, Dimension elected its fourth and final indication under the license.

In March 2015, the Company entered into an option and license agreement with Dimension that grants Dimension options to commercial exclusive licenses for four new disease indications to be elected by Dimension in the future. If elected, each option carries an option fee of $1,000 payable to the Company upon exercise, and annual maintenance fees of $50. Additionally, for each option exercised, Dimension is obligated to pay the Company up to $9,000 upon achievement of various substantive milestones, as well as mid to upper-single-digit

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percentage royalties on net sales of licensed products and mid-single-digit to low double-digit percentage sublicense fees, if any. In May 2015, Dimension exercised its first option under the agreement and paid $1,000 to the Company. In August 2015, Dimension exercised its second option under the agreement and paid $1,000 to the Company.

During the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), the Company recognized $2,700, $220, $0, and $1,000, respectively, in license revenue from license agreements with Dimension.

During the year ended December 31, 2014, the Company received $200 from Dimension for the purchase of materials owned by the Company and used in the Company's manufacturing process for research and development and clinical trials. The $200 is recognized as a gain on disposal of the material as the material is delivered to Dimension. For the year ended December 31, 2014 and the six months ended June 30, 2014 and 2015 (unaudited), the Company recognized gains of $47, $24, and $21, respectively, related to the purchased material which is included in other operating income in the statements of operations. As of December 31, 2014 and June 30, 2015 (unaudited), the Company recorded an advance payment liability of $153 and $132, respectively, for proceeds received for undelivered material.

FoxKiser

The Company was party to a services agreement, as amended from time to time, with FoxKiser, a stockholder of the Company and affiliate of one current and one former member of the Board of Directors, which was terminated in January 2015. Under the agreement, the Company paid a fixed monthly fee plus an additional support fee, as determined by FoxKiser on a monthly basis, as consideration for office facilities, equipment, supplies, general management, accounting, financial management, human resources, legal, secretarial, regulatory compliance, and other services provided to the Company. As discussed in Note 4, amounts outstanding to FoxKiser in excess of 30 days from their due date accrue interest at 1.5 percent per month, compounding monthly. The Company allocates the service fees from FoxKiser between research and development and general and administrative expense. For the years ended December 31, 2013 and 2014, and the six months ended June 30, 2014 and 2015 (unaudited), costs incurred under the services agreement with FoxKiser were as follows:

	December 31,		June 30,	
	2013	2014	2014	2015
			(unaudited)	
Research and development	$1,111	$1,283	$ 555	$148
General and administrative	1,469	1,696	735	197
	$2,580	$2,979	$1,290	$345

As of December 31, 2013 and 2014, the Company had recorded $655 and $1,423, respectively, payable to FoxKiser under the services agreement. As discussed in Note 4 and Note 6, amounts owed under the services agreement were converted into Series B Preferred Units on October 30, 2013 and Series C Preferred Stock on January 13, 2015. In January 2015, the services agreement was terminated and the remaining amounts due to the FoxKiser under the agreement were paid in full in cash. The Company also entered into promissory notes with FoxKiser as discussed in Note 4.

12. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence relative to certain estimates or to identify

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matters that require additional disclosure. For its financial statements as of December 31, 2014 and for the year then ended, the Company has completed an evaluation of all subsequent events through July 1, 2015, the date these financial statements were available to be issued, to ensure that the financial statements include appropriate disclosure of events both recognized in the financial statements as of December 31, 2014, and events which occurred subsequently but were not recognized in the financial statements.

Subsequent Events (unaudited)

For its financial statements as of June 30, 2015, the Company evaluated subsequent events through August 10, 2015, the date on which those financial statements were issued to ensure that the financial statements include appropriate disclosure of events both recognized in the financial statements as of June 30, 2015, and events which occurred subsequently but were not recognized in the financial statements. The Company has concluded that no subsequent event has occurred that requires disclosure, except as previously disclosed in the footnotes to the financial statements.

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Shares

REGENXBIO Inc.

Common Stock

PROSPECTUS

MORGAN STANLEY *BofA MERRILL LYNCH* *PIPER JAFFRAY*

CHARDAN CAPITAL MARKETS, LLC

, 2015

Until , all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.

PART II

INFORMATION NOT REQUIRED IN THE PROSPECTUS

Item 13. Other Expenses of Issuance and Distribution.

The following table presents the costs and expenses, other than underwriting discounts and commissions, payable in connection with the sale of common stock being registered. All amounts are estimates except the SEC registration fee, the FINRA filing fee, and the NASDAQ listing fee. Except as otherwise noted, all the expenses below will be paid by us.

SEC registration fee	$ 11,620
FINRA filing fee	$ 15,500
NASDAQ Listing fee	$ 225,000
Printing and engraving expenses	*
Legal fees and expenses	*
Accounting fees and expenses	*
Blue sky fees and expenses	*
Transfer agent fees	$ 10,000
Miscellaneous fees and expenses	*
Total	*

* To be completed by amendment.

Item 14. Indemnification of Directors and Officers.

In connection with the completion of this offering, the Registrant's restated certificate of incorporation will contain provisions that eliminate, to the maximum extent permitted by the General Corporation Law of the State of Delaware, the personal liability of the Registrant's directors for monetary damages for breach of their fiduciary duties as directors. The Registrant's amended and restated bylaws to be in effect immediately prior to the completion of this offering provide that the Registrant must indemnify its directors and officers and may indemnify its employees and other agents to the fullest extent permitted by the General Corporation Law of the State of Delaware.

Sections 145 and 102(b)(7) of the General Corporation Law of the State of Delaware provide that a corporation may indemnify any person made a party to an action by reason of the fact that he or she was a director, officer, employee or agent of the corporation or is or was serving at the request of a corporation against expenses (including attorneys' fees), judgments, fines and amounts paid in settlement actually and reasonably incurred by him or her in connection with such action if he or she acted in good faith and in a manner he or she reasonably believed to be in, or not opposed to, the best interests of the corporation and, with respect to any criminal action or proceeding, had no reasonable cause to believe his or her conduct was unlawful, except that, in the case of an action by or in right of the corporation, no indemnification may generally be made in respect of any claim as to which such person is adjudged to be liable to the corporation.

The Registrant has entered into indemnification agreements with its directors and executive officers, in addition to the indemnification provided for in its amended and restated bylaws, and intends to enter into indemnification agreements with any new directors and executive officers in the future.

The Registrant has purchased and intends to maintain insurance on behalf of any person who is or was a director or officer of the Registrant against any loss arising from any claim asserted against him or her and incurred by him or her in any such capacity, subject to certain exclusions.

The Underwriting Agreement, the form of which is attached as Exhibit 1.1 hereto, provides for indemnification by the underwriters of the Registrant and its executive officers and directors, and by the Registrant of the underwriters, for certain liabilities, including liabilities arising under the Securities Act and affords certain rights of contribution with respect thereto.

See also "Undertakings" set out in response to Item 17 herein.

Item 15. Recent Sales of Unregistered Securities.

Set forth below is information regarding the shares of common stock and preferred stock issued, and options granted, by us since August 17, 2012 that were not registered under the Securities Act of 1933.

(1) Under the ReGenX Biosciences, LLC (our predecessor entity) 2009 Equity Plan, we granted an aggregate of 6,420,000 of Class B Units at a distribution threshold of $0.02382 per unit as profits interests to certain of our employees.

(2) In October 2013, we issued and sold an aggregate of 95,314,803 Series B Preferred Units (pre-Conversion) to investors for an aggregate purchase price of approximately $7.9 million.

(3) In September 2014, we converted from a Delaware limited liability company named ReGenX Biosciences, LLC (the LLC) to a Delaware corporation named REGENXBIO Inc. (the Conversion). Pursuant to the Conversion, we issued (i) 2,642,963 shares of common stock upon the conversion of 132,148,224 Class A units of the LLC, (ii) 1,906,295 shares of Series A Preferred Stock upon the conversion of 119,656,372 Series A Preferred units of the LLC and (iii) 2,393,127 shares of Series B Preferred Stock upon the conversion of 95,314,803 shares of Series B Preferred units of the LLC.

(4) Under our 2014 Stock Plan, (i) from September 2014 to November 2014, we granted stock options to purchase an aggregate of 2,132,400 shares of our common stock at an exercise price of $0.85 per share to certain of our employees, officers, consultants and advisors and (ii) in May 2015, we granted stock options to purchase an aggregate of 1,063,900 shares of our common stock at an exercise price of $3.76 per share to certain of our employees, officers, consultants and advisors.

(5) In January 2015, we issued and sold an aggregate of 4,631,774 shares of Series C convertible preferred stock to investors for an aggregate purchase price of $30.0 million.

(6) In May 2015, we issued and sold an aggregate of 7,366,849 shares of Series D convertible preferred stock to investors for an aggregate purchase price of $70.5 million.

(7) In October 2014, an advisor exercised an option to purchase 1,900 shares of our common stock.

(8) In March 2015, a former employee exercised an option to purchase 7,800 shares of our common stock.

(9) In May 2015, an employee exercised an option to purchase 100,000 shares of our common stock.

(10) In July 2015, an employee exercised an option to purchase 10,150 shares of our common stock.

The offers, sales, grants and issuances of the securities described in paragraphs (1), (7), (8), (9) and (10) were deemed to be exempt from registration under the Securities Act in reliance on Rule 701. The recipients of such securities were our employees, officers, bona fide consultants and advisors and received the securities under the ReGenX Biosciences, LLC 2009 Equity Plan. Appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us.

The offer, sale, and issuance of the securities described in paragraphs (2), (3), (5) and (6) were deemed to be exempt from registration under the Securities Act in reliance on Section 4(a)(2) of the Securities Act in that the issuance of the securities to the accredited investors did not involve a public offering. The recipients of the securities in these transactions acquired the securities for investment only and not with a view to or for sale in connection with any distribution thereof, and appropriate legends were affixed to the securities issued in these transactions. The recipients of the securities in these transactions were accredited investors under Rule 501 of Regulation D.

The offers, sales, grants and issuances of the securities described in paragraph (4) were deemed to be exempt from registration under the Securities Act in reliance on Rule 701. The recipients of such securities were

our employees, officers, bona fide consultants and advisors and received the securities under our 2014 Stock Plan. Appropriate legends were affixed to the securities issued in these transactions. Each of the recipients of securities in these transactions had adequate access, through employment, business or other relationships, to information about us.

Item 16. Exhibits and Financial Statement Schedules.

(a) Exhibits

The exhibits to the registration statement are listed in the Exhibit Index attached hereto and incorporated by reference herein.

(b) Financial Statement Schedules

Schedules not listed above have been omitted because the information required to be set forth therein is not applicable or is shown in the financial statements or notes thereto.

Item 17. Undertakings.

Insofar as indemnification for liabilities arising under the Securities Act of 1933 may be permitted to directors, officers and controlling persons of the registrant pursuant to the foregoing provisions, or otherwise, the registrant has been advised that, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act of 1933, and is, therefore, unenforceable. In the event that a claim for indemnification against such liabilities (other than the payment by the registrant of expenses incurred or paid by a director, officer or controlling person of the registrant in the successful defense of any action, suit or proceeding) is asserted by such director, officer or controlling person in connection with the securities being registered, the registrant will, unless in the opinion of its counsel the matter has been settled by controlling precedent, submit to a court of appropriate jurisdiction the question whether such indemnification by it is against public policy as expressed in the Securities Act of 1933, and will be governed by the final adjudication of such issue.

The undersigned Registrant hereby undertakes to provide the underwriters, at the closing specified in the underwriting agreement, certificates in such denominations and registered in such names as required by the underwriters to permit prompt delivery to each purchaser.

The undersigned registrant hereby undertakes that:

(1) For purposes of determining any liability under the Securities Act of 1933, the information omitted from a form of prospectus filed as part of this registration statement in reliance upon Rule 430A and contained in the form of prospectus filed by the Registrant pursuant to Rule 424(b)(1) or (4) or 497(h) under the Securities Act of 1933 shall be deemed to be part of this registration statement as of the time it was declared effective.

(2) For the purpose of determining any liability under the Securities Act of 1933, each post-effective amendment that contains a form of prospectus shall be deemed to be a new registration statement relating to the securities offered therein, and the offering of such securities at that time shall be deemed to be the initial *bona fide* offering thereof.

(3) For the purpose of determining liability under the Securities Act to any purchaser, each prospectus filed pursuant to Rule 424(b) as part of a registration statement relating to an offering, other than registration statements relying on Rule 430B or other than prospectuses filed in reliance on Rule 430A, shall be deemed to be part of and included in the registration statement as of the date it is first used after effectiveness. Provided, however, that no statement made in a registration statement or prospectus that is part of the registration statement or made in a document incorporated or deemed incorporated by reference into the registration statement or prospectus that is part of the registration statement will, as

to a purchaser with a time of contract of sale prior to such first use, supersede or modify any statement that was made in the registration statement or prospectus that was part of the registration statement or made in any such document immediately prior to such date of first use.

(4) In a primary offering of securities of the undersigned registrant pursuant to this registration statement, regardless of the underwriting method used to sell the securities to the purchaser, if the securities are offered or sold to such purchaser by means of any of the following communications, the undersigned registrant will be a seller to the purchaser and will be considered to offer or sell such securities to such purchaser:

(i) Any preliminary prospectus or prospectus of the undersigned registrant relating to the offering required to be filed pursuant to Rule 424;

(ii) Any free writing prospectus relating to the offering prepared by or on behalf of the undersigned registrant or used or referred to by the undersigned registrant;

(iii) The portion of any other free writing prospectus relating to the offering containing material information about the undersigned registrant or its securities provided by or on behalf of the undersigned registrant; and

(iv) Any other communication that is an offer in the offering made by the undersigned registrant to the purchaser.

SIGNATURES

Pursuant to the requirements of the Securities Act of 1933, the registrant has duly caused this Registration Statement to be signed on its behalf by the undersigned, thereunto duly authorized, in the City of Rockville, State of Maryland, on this 17th day of August, 2015.

REGENXBIO INC.

By: /s/ Kenneth T. Mills
Kenneth T. Mills
President and Chief Executive Officer

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below hereby constitutes and appoints Kenneth T. Mills and Sara Garon Berl, and each of them, as his or her true and lawful attorney-in-fact and agent with full power of substitution, for him or her in any and all capacities, to sign any and all amendments to this registration statement (including post-effective amendments) and any registration statement related thereto filed pursuant to Rule 462(b) increasing the number of securities for which registration is sought, and to file the same, with all exhibits thereto and other documents in connection therewith, with the SEC, granting unto said attorney-in-fact and agent full power and authority to do and perform each and every act and thing requisite and necessary to be done in connection therewith, as fully for all intents and purposes as he or she might or could do in person, hereby ratifying and confirming all that said attorney-in-fact and agent, or his or her substitute, may lawfully do or cause to be done by virtue hereof.

Pursuant to the requirements of the Securities Act of 1933, as amended, this Registration Statement has been signed by the following persons in the capacities and on the dates indicated:

Signature	Title	Date
/s/ Kenneth T. Mills Kenneth T. Mills	Chief Executive Officer, President and Director (Principal Executive Officer)	August 17, 2015
/s/ Vittal Vasista Vittal Vasista	Chief Financial Officer (Principal Financial and Accounting Officer)	August 17, 2015
/s/ Donald J. Hayden, Jr. Donald J. Hayden, Jr.	Chairman of the Board of Directors	August 17, 2015
/s/ Luke M. Beshar Luke M. Beshar	Director	August 17, 2015
/s/ Edgar G. Engleman, M.D. Edgar G. Engleman, M.D.	Director	August 17, 2015
/s/ Allan M. Fox Allan M. Fox	Director	August 17, 2015
/s/ A.N. “Jerry” Karabelas, Ph.D. A.N. “Jerry” Karabelas, Ph.D.	Director	August 17, 2015
/s/ Camille Samuels Camille Samuels	Director	August 17, 2015

EXHIBIT INDEX

Exhibit	Description
1.1*	Form of Underwriting Agreement
3.1	Restated Certificate of Incorporation, as amended (currently in effect)
3.2	Bylaws (currently in effect)
3.3	Form of Restated Certificate of Incorporation (to be effective immediately prior to the closing of this offering)
3.4	Form of Amended and Restated Bylaws (to be effective immediately prior to the closing of this offering)
4.1	Specimen stock certificate evidencing the shares of common stock
4.2	Amended and Restated Investors' Rights Agreement dated as of May 15, 2015
5.1*	Opinion of Gunderson Dettmer Stough Villeneuve Franklin & Hachigian, LLP
10.1	Form of Indemnity Agreement for directors and officers
10.2+	2014 Stock Plan, as amended
10.3+	2015 Equity Incentive Plan and form of option agreement thereunder
10.4+	2015 Employee Stock Purchase Plan
10.5+	Employment Agreement effective as of June 30, 2015 between the Registrant and Kenneth T. Mills.
10.6+	Employment Agreement effective as of June 30, 2015 between the Registrant and Stephen Yoo, M.D.
10.7+	Employment Agreement effective as of June 30, 2015 between the Registrant and Vittal Vasista
10.8+	Independent Director Compensation Policy
10.9†	License Agreement effective February 24, 2009 between the Registrant and The Trustees of the University of Pennsylvania
10.10†	First Amendment to License Agreement dated March 6, 2009 between the Registrant and The Trustees of the University of Pennsylvania
10.11†	Second Amendment to License Agreement effective September 9, 2014 between the Registrant and The Trustees of the University of Pennsylvania
10.12†	License Agreement dated March 6, 2009 between the Registrant and SmithKline Beecham Corporation d/b/a GlaxoSmithKline
10.13	Amendment to License Agreement dated April 15, 2009 between the Registrant and SmithKline Beecham Corporation d/b/a GlaxoSmithKline
10.14†	License Agreement dated April 10, 2014 between the Registrant and AAVLife
10.15†	License Agreement dated July 9, 2013 between the Registrant and Audentes Therapeutics, Inc.
10.16†	License Agreement dated March 21, 2014 between the Registrant and AveXis, Inc.
10.17†	License Agreement dated November 22, 2010 between the Registrant and Baxalta US Inc. (as assignee of Baxter Healthcare Corporation, as assignee of Chatham Therapeutics, LLC)

Exhibit	Description
10.18†	License Agreement dated October 30, 2013 between the Registrant and Dimension Therapeutics, Inc.
10.19†	First Amendment to License Agreement dated June 18, 2014 between the Registrant and Dimension Therapeutics, Inc.
10.20†	Second Amendment to License Agreement dated September 29, 2014 between the Registrant and Dimension Therapeutics, Inc.
10.21†	Option and License Agreement dated March 10, 2015 between the Registrant and Dimension Therapeutics, Inc.
10.22†	License Agreement dated March 5, 2014 between the Registrant and Laboratorios Del Dr. Esteve, S.A.
10.23†	License Agreement dated December 2, 2013 between the Registrant and Lysogene Société par Actions Simplifiée
10.24†	License Agreement dated May 28, 2014 between the Registrant and Voyager Therapeutics, Inc.
10.25†	Exclusive Patent License Agreement dated November 10, 2014 between the Registrant and the Regents of the University of Minnesota
10.26	Lease dated March 6, 2015 between the Registrant and BMR-Medical Center Drive LLC
10.27†	Development, Manufacturing, and Testing Standard Terms and Conditions dated April 3, 2015 between the Registrant and WuXi AppTec, Inc.
10.28†	Cooperation Agreement dated May 28, 2015 between the Registrant and WuXi AppTec, Inc.
10.29+	REGENXBIO Inc. Management Cash Incentive Plan
10.30	Board of Managers Agreement dated February 6, 2013 between the Registrant and Donald J. Hayden, Jr.
16.1	Letter from Baker Tilly Virchow Krause, LLP addressed to the SEC provided in connection with the change in independent accountant
23.1	Consent of PricewaterhouseCoopers LLP, independent registered public accounting firm
23.2*	Consent of Gunderson Dettmer Stough Villeneuve Franklin & Hachigian, LLP (included in Exhibit 5.1)
24.1	Power of Attorney (included on signature page)

* To be filed by amendment.
\+ Indicates management contract or compensatory plan.
† Portions of this exhibit (indicated by asterisks) have been omitted pursuant to a request for confidential treatment. The omitted portions of this exhibit have been filed with the SEC.

RESTATED
CERTIFICATE OF INCORPORATION
OF
REGENXBIO INC.

(Pursuant to Sections 242 and 245 of the
General Corporation Law of the State of Delaware)

REGENXBIO Inc., a corporation organized and existing under and by virtue of the provisions of the General Corporation Law of the State of Delaware (the "General Corporation Law"),

DOES HEREBY CERTIFY:

FIRST: That the name of this corporation is REGENXBIO Inc. and that this corporation was originally incorporated pursuant to the General Corporation Law on September 16, 2014 under the name REGENXBIO Inc.

SECOND: That the Board of Directors duly adopted resolutions proposing to amend and restate the Certificate of Incorporation of this corporation, declaring said amendment and restatement to be advisable and in the best interests of this corporation and its stockholders, and authorizing the appropriate officers of this corporation to solicit the consent of the stockholders therefor, which resolution setting forth the proposed amendment and restatement is as follows:

RESOLVED, that the Certificate of Incorporation of this corporation be amended and restated in its entirety as follows:

ARTICLE I

The name of this corporation is REGENXBIO Inc.

ARTICLE II

The address of the corporation's registered office in the State of Delaware is Corporation Trust Center, 1209 Orange Street, in the City of Wilmington, 19801, County of New Castle. The name of its registered agent at such address is The Corporation Trust Company.

ARTICLE III

The nature of the business or purposes to be conducted or promoted is to engage in any lawful act or activity for which corporations may be organized under the General Corporation Law.

ARTICLE IV

A. Authorization of Stock. This corporation is authorized to issue two classes of stock to be designated, respectively, common stock and preferred stock. The total number of shares that this corporation is authorized to issue is 39,398,045. The total number of shares of common stock authorized to be issued is 23,100,000 par value $0.0001 per share (the "Common Stock"). The total number of shares of preferred stock authorized to be issued is 16,298,045, par value $0.0001 per share (the "Preferred Stock"), of which 2,393,127 shares are designated as "Series A Preferred Stock", 1,906,295 shares are designated as "Series B Preferred Stock", 4,631,774 are designated as "Series C Preferred Stock" and 7,366,849 are designated as "Series D Preferred Stock".

B. Rights, Preferences and Restrictions of Preferred Stock. The rights, preferences, privileges and restrictions granted to and imposed on the Preferred Stock are as set forth below in this Article IV(B).

1. Dividend Provisions.

(a) The holders of shares of Series D Preferred Stock shall be entitled to receive dividends, out of any assets legally available therefor, prior and in preference to any declaration or payment of any dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of this corporation) on the Series C Preferred Stock, Series B Preferred Stock, Series A Preferred Stock or Common Stock of this corporation at the applicable Dividend Rate (as defined below), payable *pro rata* based on the aggregate amount of accrued dividends per share of Series D Preferred Stock as set forth in this subsection 1(a) of Article IV(B) when, as and if declared by the Board of Directors. Such dividends shall not be cumulative. The holders of the outstanding Series D Preferred Stock can waive any dividend preference that such holders shall be entitled to receive under this subsection 1(a) of Article IV(B) upon the affirmative vote or written consent of the holders of at least a majority of the shares of Series D Preferred Stock then outstanding, which vote or written consent must include at least three of the Requisite Lead Investors (as defined in that certain Amended and Restated Voting Agreement dated on or about the Filing Date (as defined below) by and among this corporation and the other parties thereto, as amended from time to time (the "Voting Agreement")).

(b) Subject to the rights of the holders of Series D Preferred Stock set forth in subsection 1(a) of Article IV(B) above, the holders of shares of Series C Preferred Stock shall be entitled to receive dividends, out of any assets legally available therefor, prior and in preference to any declaration or payment of any dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of this corporation) on the Series B Preferred Stock, Series A Preferred Stock or Common Stock of this corporation, at the applicable Dividend Rate, payable *pro rata* based on the aggregate amount of accrued dividends per share of Series C Preferred Stock as set forth in this subsection 1(b) of Article IV(B) when, as and if declared by the Board of Directors. Such dividends shall not be cumulative. The holders of the outstanding Series C Preferred Stock can waive any dividend preference that such holders shall be entitled to

receive under this subsection 1(b) of Article IV(B) upon the affirmative vote or written consent of the holders of at least a majority of the shares of Series C Preferred Stock then outstanding, which vote or written consent must include at least two of the Series C Lead Investors (as defined in the Voting Agreement).

(c) Subject to the rights of the holders of Series D Preferred Stock and Series C Preferred Stock set forth in subsection 1(a) and subsection 1(b), respectively, of Article IV(B) above, the holders of shares of Series B Preferred Stock and Series A Preferred Stock shall be entitled to receive dividends, out of any assets legally available therefor, prior and in preference to any declaration or payment of any dividend (payable other than in Common Stock or other securities and rights convertible into or entitling the holder thereof to receive, directly or indirectly, additional shares of Common Stock of this corporation) on the Common Stock of this corporation, at the applicable Dividend Rate, payable *pro rata* based on the aggregate amount of accrued dividends per share of Series B Preferred Stock and Series A Preferred Stock as set forth in this subsection 1(c) of Article IV(B) when, as and if declared by the Board of Directors. Such dividends shall not be cumulative. The holders of the outstanding Series B Preferred Stock and Series A Preferred Stock can waive any dividend preference that such holders shall be entitled to receive under this subsection 1(c) of Article IV(B) upon the affirmative vote or written consent of the holders of at least fifty-five percent (55%) of the shares of Series B Preferred Stock and Series A Preferred Stock then outstanding (voting together as a single class and not as separate series, and on an as-converted basis).

(d) Subject to the rights of the holders of Preferred Stock set forth in subsection 1(a), subsection 1(b) and subsection 1(c) of Article IV(B) above, after payment of such dividends, any additional dividends or distributions shall be distributed among all holders of Common Stock and Preferred Stock in proportion to the number of shares of Common Stock that would be held by each such holder if all shares of Preferred Stock were converted to Common Stock at the then effective conversion rate.

(e) For purposes of this subsection 1 of Article IV(B), “Dividend Rate” shall mean $0.1003 per annum for each share of Series A Preferred Stock, $0.331192 per annum for each share of Series B Preferred Stock, $0.51816 per annum for each share of Series C Preferred Stock and $0.765592 per annum for each share of Series D Preferred Stock (each as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like).

2. Liquidation Preference.

(a) In the event of any Liquidation Event (as defined below), either voluntary or involuntary, the holders of Series D Preferred Stock shall be entitled to receive out of the proceeds or assets of this corporation available for distribution to its stockholders (the “Proceeds”), prior and in preference to any distribution of the Proceeds to the holders of Series C Preferred Stock, Series B Preferred Stock, Series A Preferred Stock and Common Stock by reason of their ownership thereof, an amount per share equal to the sum of the applicable Original Issue Price (as defined below) for the Series D Preferred Stock, plus declared but unpaid dividends on such share. If, upon the occurrence of a Liquidation Event, the Proceeds thus distributed among the holders of the Series D Preferred Stock shall be insufficient to permit the

payment to such holders of the full preferential amounts, then the entire Proceeds legally available for distribution shall be distributed ratably among the holders of the Series D Preferred Stock in proportion to the full preferential amount that each such holder is otherwise entitled to receive under this subsection (a) of Article IV(B). For purposes of this Restated Certificate of Incorporation, "Original Issue Price" shall mean $1.255 per share for each share of the Series A Preferred Stock, $4.1399 per share for each share of Series B Preferred Stock, $6.477 per share for each share of Series C Preferred Stock and $9.5699 per share for each share of Series D Preferred Stock (each as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like with respect to such series of Preferred Stock).

(b) In the event of any Liquidation Event, either voluntary or involuntary, after payments of all preferential amounts required to be paid to the holders of the shares of Series D Preferred Stock required by subsection 2(a) of Article IV(B), the holders of Series C Preferred Stock shall be entitled to receive out of any remaining Proceeds, prior and in preference to any distribution of the Proceeds to the holders of Series B Preferred Stock, Series A Preferred Stock and Common Stock by reason of their ownership thereof, an amount per share equal to the sum of the applicable Original Issue Price for the Series C Preferred Stock, plus declared but unpaid dividends on such share. If, upon the occurrence of a Liquidation Event, the Proceeds thus distributed among the holders of the Series C Preferred Stock shall be insufficient to permit the payment to such holders of the full preferential amounts, then the entire Proceeds legally available for distribution shall be distributed ratably among the holders of the Series C Preferred Stock in proportion to the full preferential amount that each such holder is otherwise entitled to receive under this subsection 2(b) of Article IV(B).

(c) In the event of any Liquidation Event, either voluntary or involuntary, after payments of all preferential amounts required to be paid to the holders of the shares of Series D Preferred Stock and Series C Preferred Stock required by subsection 2(a) and subsection 2(b), respectively, of Article IV(B), the holders of Series B Preferred Stock and Series A Preferred Stock shall be entitled on a pari *passu basis* among such series of Preferred Stock to receive out of any remaining Proceeds, prior and in preference to any distribution of the Proceeds to the holders of Common Stock by reason of their ownership thereof, an amount per share equal to the sum of the applicable Original Issue Price (as defined below) for such series of Preferred Stock, plus declared but unpaid dividends on such share. If, upon the occurrence of a Liquidation Event, the Proceeds thus distributed among the holders of the Series B Preferred Stock and Series A Preferred shall be insufficient to permit the payment to such holders of the full preferential amounts, then the entire Proceeds legally available for distribution shall be distributed ratably on a *pari passu* basis among the holders of such series of Preferred Stock in proportion to the full preferential amount that each such holder is otherwise entitled to receive under this subsection 2(c) of Article IV(B).

(d) Upon completion of the distribution required by subsection 2(a), subsection 2(b) and subsection 2(c) of Article IV(B), all of the remaining Proceeds shall be distributed among the holders of Preferred Stock and Common Stock pro rata based on the number of shares of Common Stock held by each (assuming full conversion of all such Preferred Stock).

(e) Notwithstanding the above, for purposes of determining the amount each holder of shares of Preferred Stock is entitled to receive with respect to a Liquidation Event, each such holder of shares of a series of Preferred Stock shall be deemed to have converted (regardless of whether such holder actually converted) such holder's shares of such series into shares of Common Stock immediately prior to the Liquidation Event if, as a result of an actual conversion, such holder would receive, in the aggregate, an amount greater than the amount that would be distributed to such holder if such holder did not convert such series of Preferred Stock into shares of Common Stock. If any such holder shall be deemed to have converted shares of Preferred Stock into Common Stock pursuant to this paragraph, then such holder shall not be entitled to receive any distribution that would otherwise be made to holders of Preferred Stock that have not converted (or have not been deemed to have converted) into shares of Common Stock.

(f) (i) For purposes of this Section 2 of Article IV(B), a "Liquidation Event" shall include (A) the closing of the sale, transfer or other disposition of all or substantially all of this corporation's assets or the exclusive license of all or substantially all of the corporation's intellectual property, (B) the consummation of the merger or consolidation of this corporation with or into another entity (except a merger or consolidation in which the holders of capital stock of this corporation immediately prior to such merger or consolidation continue to hold at least 50% of the voting power of the capital stock of this corporation or the surviving or acquiring entity), (C) the closing of the transfer (whether by merger, consolidation or otherwise), in one transaction or a series of related transactions, to a person or group of affiliated persons (other than an underwriter of this corporation's securities), of this corporation's securities if, after such closing, such person or group of affiliated persons would hold 50% or more of the outstanding voting stock of this corporation (or the surviving or acquiring entity) or (D) a liquidation, dissolution or winding up of this corporation; provided, however, that a transaction shall not constitute a Liquidation Event if its sole purpose is to change the state of this corporation's incorporation or to create a holding company that will be owned in substantially the same proportions by the persons who held this corporation's securities immediately prior to such transaction. The treatment of any particular transaction or series of related transactions as a Liquidation Event may be waived by the vote or written consent of the holders of a majority of the outstanding Preferred Stock (voting together as a single class and not as separate series, and on an as-converted basis), which vote or written consent must include at least three of the Requisite Lead Investors.

(ii) In any Liquidation Event, if Proceeds received by this corporation or its stockholders is other than cash, its value will be deemed its fair market value. Any securities shall be valued as follows:

(A) Securities not subject to investment letter or other similar restrictions on free marketability covered by (B) below:

(1) If traded on a securities exchange, the value shall be deemed to be the average of the closing prices of the securities on such exchange over the twenty (20) trading-day period ending three (3) trading days prior to the closing of the Liquidation Event;

(2) If actively traded over-the-counter, the value shall be deemed to be the average of the closing bid or sale prices (whichever is applicable) over the twenty (20) trading-day period ending three (3) trading days prior to the closing of the Liquidation Event; and

(3) If there is no active public market, the value shall be the fair market value thereof, as mutually determined by this corporation and the holders of a majority of the voting power of all then outstanding shares of Preferred Stock, which must include at least three of the Requisite Lead Investors.

(B) The method of valuation of securities subject to investment letter or other restrictions on free marketability (other than restrictions arising solely by virtue of a stockholder's status as an affiliate or former affiliate) shall be to make an appropriate discount from the market value determined as above in subsection 2(d)(ii)(A)(1), subsection 2(d)(ii)(A)(2) or subsection 2(d)(ii)(A)(3) of Article IV(B) to reflect the approximate fair market value thereof, as mutually determined by this corporation and the holders of a majority of the voting power of all then outstanding shares of such Preferred Stock, which must include at least three of the Requisite Lead Investors.

(C) The foregoing methods for valuing non-cash consideration to be distributed in connection with a Liquidation Event shall, with the appropriate approval of the definitive agreements governing such Liquidation Event by the stockholders under the General Corporation Law and Section 6 of this Article IV(B), be superseded by the determination of such value set forth in the definitive agreements governing such Liquidation Event.

(iii) In the event the requirements of this Section 2 of Article IV(B) are not complied with, this corporation shall forthwith either:

(A) cause the closing of such Liquidation Event to be postponed until such time as the requirements of this Section 2 of Article IV(B) have been complied with; or

(B) cancel such transaction, in which event the rights, preferences and privileges of the holders of the Preferred Stock shall revert to and be the same as such rights, preferences and privileges existing immediately prior to the date of the first notice referred to in subsection 2(d)(iv) of Article IV(B) hereof.

(iv) This corporation shall give each holder of record of Preferred Stock written notice of such impending Liquidation Event not later than twenty (20) days prior to the stockholders' meeting called to approve such transaction, or twenty (20) days prior to the closing of such transaction, whichever is earlier, and shall also notify such holders in writing of the final approval of such transaction. The first of such notices shall describe the material terms and conditions of the impending transaction and the provisions of this Section 2 of Article IV(B), and this corporation shall thereafter give such holders prompt notice of any material changes. The transaction shall in no event take place sooner than twenty (20) days after this corporation has given the first notice provided for herein or sooner than ten (10) days after

this corporation has given notice of any material changes provided for herein; provided, however, that subject to compliance with the General Corporation Law such periods may be shortened or waived upon the written consent of the holders of Preferred Stock that represent a majority of the voting power of all then outstanding shares of such Preferred Stock (voting together as a single class and not as separate series, and on an as-converted basis), which consent must include at least three of the Requisite Lead Investors.

(g) Allocation of Contingent Consideration. In the event of a deemed Liquidation Event pursuant to subsection 2(e)(i) of Article IV(B), if any portion of the consideration payable to the stockholders of this corporation is placed into escrow and/or is payable to the stockholders of this corporation subject to contingencies, the definitive agreement with respect to such deemed Liquidation Event shall provide that the portion of such consideration that is placed in escrow and/or subject to any contingencies (the "Contingent Consideration") shall be allocated among the holders of capital stock of this corporation in accordance with subsection 2(a), subsection 2(b) and subsection 2(c) of Article IV(B) as if all of consideration ultimately payable in the transaction, including the Contingent Consideration, is paid without restrictions at the time of closing the deemed Liquidation Event (so that the Contingent Consideration shall be allocated among the holders of capital stock of this corporation pro rata based on the amount of such consideration otherwise payable to each stockholder pursuant to this Section 2 of Article IV(B)).

3. Redemption.

(a) At any time after December 31, 2019, but within sixty (60) days after the receipt by this corporation of a written request from the holders of not less than a majority of the then outstanding Preferred Stock (voting together as a single class and on an as-converted basis, which written consent must include at least three of the Requisite Lead Investors) that all of the then outstanding shares of Preferred Stock be redeemed, provided that all such notices are received within a period of thirty (30) consecutive days, this corporation shall, to the extent it may lawfully do so, redeem in three (3) annual installments (each payment date being referred to herein as a "Redemption Date") the then outstanding shares of Preferred Stock by paying in cash therefor a sum per share equal to the applicable Original Issue Price for such shares of Preferred Stock (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) plus all accrued but unpaid dividends on such share (whether or not declared) (the "Redemption Price"). The number of shares of each series of Preferred Stock that this corporation shall be required to redeem on any one Redemption Date shall be equal to the amount determined by dividing (i) the aggregate number of shares of such series of Preferred Stock outstanding immediately prior to such Redemption Date by (ii) the number of remaining Redemption Dates (including the Redemption Date to which such calculation applies). Any redemption of a series of Preferred Stock effected pursuant to this subsection 3(a) of Article IV(B) shall be made on a pro rata basis among the holders of such series of Preferred Stock in proportion to the aggregate Redemption Price of each such holder of Preferred Stock would otherwise be entitled to receive on the applicable Redemption Date.

(b) Not less than forty (40) days prior to each Redemption Date, written notice shall be mailed, first class postage prepaid, to each holder of record (at the close of business on the business day next preceding the day on which notice is given) of Preferred Stock,

at the address last shown on the records of this corporation for such holder, notifying such holder of the redemption to be effected on the applicable Redemption Date, specifying the number and series of the shares of Preferred Stock to be redeemed from such holder, the Redemption Prices for each series of Preferred Stock and the place at which payment may be obtained and calling upon such holder to surrender to this corporation, in the manner and at the place designated, his, her or its certificate or certificates representing the shares to be redeemed, if any (the "Redemption Notice"). If this corporation receives, on or prior to the twentieth (20th) day after the date of delivery of the initial Redemption Notice to a holder of Preferred Stock, written notice from such holder that such holder elects to be excluded from the redemption provided in this Section 3 of Article IV(B), then the Preferred Stock registered on the books of this corporation in the name of such holder at the time of the corporation's receipt of such notice shall thereafter be "Excluded Stock". Excluded Stock shall not be redeemed or redeemable pursuant to this Section 3, whether on such Redemption Date or thereafter. Except as provided in subsection (3)(c) of Article IV(B), on or after each Redemption Date, each holder of Preferred Stock on such Redemption Date shall surrender to this corporation the certificate or certificates representing such shares, in the manner and at the place designated in the Redemption Notice, and thereupon the applicable Redemption Price of such shares shall be payable to the order of the person whose name appears on such certificate or certificates as the owner thereof and each surrendered certificate shall be cancelled. In the event less than all the shares represented by any such certificate are redeemed, a new certificate shall be issued representing the unredeemed shares.

(c) From and after each Redemption Date, unless there shall have been a default in payment of the Redemption Price, all rights of the holders of shares of Preferred Stock designated for redemption on such Redemption Date in the Redemption Notice as holders of Preferred Stock (except the right to receive the applicable Redemption Price without interest upon surrender of their certificate or certificates) shall cease with respect to such shares, and such shares shall not thereafter be transferred on the books of this corporation or be deemed to be outstanding for any purpose whatsoever. If the funds of this corporation legally available for redemption of shares of Preferred Stock on a Redemption Date are insufficient to redeem the total number of shares of Preferred Stock to be redeemed on such date, those funds that are legally available will be used to redeem the maximum possible number of such shares ratably among the holders of such shares to be redeemed in proportion to the aggregate Redemption Price that each such holder would be entitled to receive pursuant to Section 3(a) of Article IV(B). The shares of Preferred Stock not redeemed shall remain outstanding and entitled to all the rights and preferences provided herein. At any time thereafter when additional funds of this corporation are legally available for the redemption of shares of Preferred Stock, such funds will immediately be used to redeem the balance of the shares that this corporation has become obliged to redeem on any Redemption Date but that it has not redeemed.

4. Conversion. The holders of the Preferred Stock shall have conversion rights as follows (the "Conversion Rights"):

(a) Right to Convert. Each share of Preferred Stock shall be convertible, at the option of the holder thereof, at any time after the date of issuance of such share and on or prior to the fifth day prior to the Redemption Date, if any, as may have been fixed in any Redemption Notice with respect to such share of the Preferred Stock, at the office of

this corporation or any transfer agent for such stock, into such number of fully paid and nonassessable shares of Common Stock as is determined by dividing the applicable Original Issue Price for such series by the applicable Conversion Price for such series (the conversion rate for a series of Preferred Stock into Common Stock is referred to herein as the "Conversion Rate" for such series), determined as hereafter provided, in effect on the date the certificate is surrendered for conversion. The initial Conversion Price per share for each series of Preferred Stock shall be the Original Issue Price applicable to such series; provided, however, that the Conversion Price for the Preferred Stock shall be subject to adjustment as set forth in subsection 4(d) of Article IV(B).

(b) Automatic Conversion. Each share of Preferred Stock shall automatically be converted into shares of Common Stock at the Conversion Rate at the time in effect for such series of Preferred Stock immediately upon the earlier of (i) the closing of this corporation's sale of its Common Stock in a firm commitment underwritten public offering pursuant to a registration statement on Form S-1 under the Securities Act of 1933, as amended, of not less than $40,000,000 in the aggregate of gross proceeds, prior to deduction of underwriting discount and commissions (a "Qualified Public Offering") or (ii) the date, or the occurrence of an event, specified by vote or written consent or agreement of the holders of a majority of the then outstanding shares of Preferred Stock (voting together as a single class and not as separate series, and on an as-converted basis), which vote or written consent must include at least three of the Requisite Lead Investors.

(c) Mechanics of Conversion. Before any holder of Preferred Stock shall be entitled to voluntarily convert the same into shares of Common Stock, he or she shall surrender the certificate or certificates therefor, duly endorsed, at the office of this corporation or of any transfer agent for the Preferred Stock, and shall give written notice to this corporation at its principal corporate office, of the election to convert the same and shall state therein the name or names in which the certificate or certificates for shares of Common Stock are to be issued. This corporation shall, as soon as practicable thereafter, issue and deliver at such office to such holder of Preferred Stock, or to the nominee or nominees of such holder, a certificate or certificates for the number of shares of Common Stock to which such holder shall be entitled as aforesaid. Such conversion shall be deemed to have been made immediately prior to the close of business on the date set forth for conversion in the written notice of the election to convert irrespective of the surrender of the shares of Preferred Stock to be converted, and the person or persons entitled to receive the shares of Common Stock issuable upon such conversion shall be treated for all purposes as the record holder or holders of such shares of Common Stock as of such date. If the conversion is in connection with an underwritten offering of securities registered pursuant to the Securities Act of 1933, as amended, the conversion may, at the option of any holder tendering Preferred Stock for conversion, be conditioned upon the closing with the underwriters of the sale of securities pursuant to such offering, in which event the persons entitled to receive the Common Stock upon conversion of the Preferred Stock shall not be deemed to have converted such Preferred Stock until immediately prior to the closing of such sale of securities. If the conversion is in connection with Automatic Conversion provisions of subsection 4(b)(ii) of Article IV(B) above, such conversion shall be deemed to have been made on the conversion date described in the stockholder consent approving such conversion, and the persons entitled to receive shares of Common Stock issuable upon such conversion shall be treated for all purposes as the record holders of such shares of Common Stock as of such date.

(d) Conversion Price Adjustments of Preferred Stock for Certain Dilutive Issuances, Splits and Combinations. The Conversion Price of the Preferred Stock shall be subject to adjustment from time to time as follows:

(i) (A) If this corporation shall issue, on or after the date upon which this Restated Certificate of Incorporation is accepted for filing by the Secretary of State of the State of Delaware (the "Filing Date"), any Additional Stock (as defined below) without consideration or for a consideration per share less than the Conversion Price applicable to a series of Preferred Stock in effect immediately prior to the issuance of such Additional Stock, the Conversion Price for such series in effect immediately prior to each such issuance shall forthwith (except as otherwise provided in this clause (i)) be adjusted to a price (calculated to the nearest one-thousandth of a cent) determined by multiplying such Conversion Price by a fraction, the numerator of which shall be the number of shares of Common Stock Outstanding (as defined below) immediately prior to such issuance plus the number of shares of Common Stock that the aggregate consideration received by this corporation for such issuance would purchase at such Conversion Price; and the denominator of which shall be the number of shares of Common Stock Outstanding (as defined below) immediately prior to such issuance plus the number of shares of such Additional Stock. For purposes of this subsection 4(d)(i)(A) of Article IV(B), the term "Common Stock Outstanding" shall mean and include the following: (1) outstanding Common Stock, (2) Common Stock issuable upon conversion of outstanding Preferred Stock, (3) Common Stock issuable upon exercise of outstanding stock options and (4) Common Stock issuable upon exercise (and, in the case of warrants to purchase Preferred Stock, conversion) of outstanding warrants. Shares described in (1) through (4) above shall be included whether vested or unvested, whether contingent or non-contingent and whether exercisable or not yet exercisable. In the event that this corporation issues or sells, or is deemed to have issued or sold, shares of Additional Stock that results in an adjustment to a Conversion Price pursuant to the provisions of this subsection 4(d) of Article IV(B) (the "First Dilutive Issuance"), and this corporation then issues or sells, or is deemed to have issued or sold, shares of Additional Stock in a subsequent issuance other than the First Dilutive Issuance that would result in further adjustment to a Conversion Price (a "Subsequent Dilutive Issuance") pursuant to the same instruments as the First Dilutive Issuance, then and in each such case upon a Subsequent Dilutive Issuance the applicable Conversion Price for each series of Preferred Stock shall be reduced to the applicable Conversion Price that would have been in effect had the First Dilutive Issuance and each Subsequent Dilutive Issuance all occurred on the closing date of the First Dilutive Issuance.

(B) No adjustment of the Conversion Price for the Preferred Stock shall be made in an amount less than one-tenth of one cent per share. Except to the limited extent provided for in subsection 4(d)(i)(E)(3) and subsection 4(d)(i)(E) (4) of Article IV(B), no adjustment of such Conversion Price pursuant to this subsection 4(d)(i) of Article IV(B) shall have the effect of increasing the Conversion Price above the Conversion Price in effect immediately prior to such adjustment.

(C) In the case of the issuance of Additional Stock for cash, the consideration shall be deemed to be the amount of cash paid therefor before deducting any reasonable discounts, commissions or other expenses allowed, paid or incurred by this corporation for any underwriting or otherwise in connection with the issuance and sale thereof.

(D) In the case of the issuance of the Additional Stock for a consideration in whole or in part other than cash, the consideration other than cash shall be deemed to be the fair market value thereof as determined by the Board of Directors, with the approval of the holders of at least a majority of the then-outstanding shares of Series D Preferred Stock, irrespective of any accounting treatment.

(E) In the case of the issuance of options to purchase or rights to subscribe for Common Stock, securities by their terms convertible into or exchangeable for Common Stock or options to purchase or rights to subscribe for such convertible or exchangeable securities, the following provisions shall apply for purposes of determining the number of shares of Additional Stock issued and the consideration paid therefor:

(1) The aggregate maximum number of shares of Common Stock deliverable upon exercise (assuming the satisfaction of any conditions to exercisability, including without limitation, the passage of time, but without taking into account potential antidilution adjustments) of such options to purchase or rights to subscribe for Common Stock shall be deemed to have been issued at the time such options or rights were issued and for a consideration equal to the consideration (determined in the manner provided in subsection 4(d)(i)(C) and subsection 4(d)(i)(D) of Article IV(B)), if any, received by this corporation upon the issuance of such options or rights plus the minimum exercise price provided in such options or rights (without taking into account potential antidilution adjustments) for the Common Stock covered thereby.

(2) The aggregate maximum number of shares of Common Stock deliverable upon conversion of, or in exchange (assuming the satisfaction of any conditions to convertibility or exchangeability, including, without limitation, the passage of time, but without taking into account potential antidilution adjustments) for, any such convertible or exchangeable securities or upon the exercise of options to purchase or rights to subscribe for such convertible or exchangeable securities and subsequent conversion or exchange thereof shall be deemed to have been issued at the time such securities were issued or such options or rights were issued and for a consideration equal to the consideration, if any, received by this corporation for any such securities and related options or rights (excluding any cash received on account of accrued interest or accrued dividends), plus the minimum additional consideration, if any, to be received by this corporation (without taking into account potential antidilution adjustments) upon the conversion or exchange of such securities or the exercise of any related options or rights (the consideration in each case to be determined in the manner provided in subsection 4(d)(i)(C) and subsection 4(d)(i)(D) of Article IV(B)).

(3) In the event of any change in the number of shares of Common Stock deliverable or in the consideration payable to this corporation upon exercise of such options or rights or upon conversion of or in exchange for such convertible or exchangeable securities, the Conversion Price of the Preferred Stock, to the extent in any way affected by or computed using such options, rights or securities, shall be recomputed to reflect such change, but no further adjustment shall be made for the actual issuance of Common Stock or any payment of such consideration upon the exercise of any such options or rights or the conversion or exchange of such securities.

(4) Upon the expiration of any such options or rights, the termination of any such rights to convert or exchange or the expiration of any options or rights related to such convertible or exchangeable securities, the Conversion Price of the Preferred Stock, to the extent in any way affected by or computed using such options, rights or securities or options or rights related to such securities, shall be recomputed to reflect the issuance of only the number of shares of Common Stock (and convertible or exchangeable securities that remain in effect) actually issued upon the exercise of such options or rights, upon the conversion or exchange of such securities or upon the exercise of the options or rights related to such securities.

(5) The number of shares of Additional Stock deemed issued and the consideration deemed paid therefor pursuant to subsection 4(d)(i)(E)(1) and subsection 4(d)(i)(E)(2) of Article IV(B) shall be appropriately adjusted to reflect any change, termination or expiration of the type described in either subsection 4(d)(i)(E)(3) or (4) of Article IV(B).

(ii) "Additional Stock" shall mean any shares of Common Stock issued (or deemed to have been issued pursuant to subsection 4(d)(i)(E) of Article IV(B)) by this corporation on or after the Filing Date other than (1) the following shares of Common Stock and (2) shares of Common Stock deemed issued pursuant to the following Options and Convertible Securities (clauses (1) and (2), collectively, "Exempted Securities"):

(A) Common Stock issued pursuant to a split or subdivision of the Common Stock as described in subsection 4(d)(iii) of Article IV(B) hereof;

(B) Up to an aggregate of 4,100,000 shares of Common Stock (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) issued to employees, directors, consultants and other service provides for the primary purpose of soliciting or retaining their services pursuant to plans or agreements approved by this corporation's Board of Directors and shares of Common Stock in excess of 4,100,000 shares (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) issued to employees, directors, consultants and other service providers for the primary purpose of soliciting or retaining their services pursuant to plans or agreements approved by this corporation's Board of Directors, and the holders of at least a majority of the then-outstanding shares of Series D Preferred Stock;

(C) Common Stock issued pursuant to a Qualified Public Offering;

(D) Common Stock issued pursuant to the conversion or exercise of convertible or exercisable securities outstanding on the Filing Date;

(E) (x) Up to the Allocated Amount of shares of Common Stock issued in connection with a bona fide business acquisition by this corporation, whether by merger, consolidation, sale of assets, sale or exchange of stock or otherwise, provided, such issuance is approved by the Board of Directors and (y) shares of Common Stock in excess of the Allocated Amount issued in connection with a bona fide business acquisition by

this corporation, whether by merger, consolidation, sale of assets, sale or exchange of stock or otherwise, provided, such issuance is approved by the Board of Directors, and the holders of at least a majority of the then-outstanding shares of Series D Preferred Stock;

(F) Common Stock issued or deemed issued pursuant to subsection 4(d)(i)(E) of Article IV(B) as a result of a decrease in the Conversion Price of any series of Preferred Stock resulting from the operation of Section 4(d) of Article IV(B);

(G) Common Stock issued upon conversion of the Preferred Stock; or

(H) (x) Up to the Allocated Amount of shares of Common Stock issued to persons or entities in connection with sponsored research, collaboration, technology license, development, OEM, marketing or other similar agreements or strategic partnerships, provided such issuances are approved by the Board of Directors and (y) shares of Common Stock in excess of the Allocated Amount issued to persons or entities in connection with sponsored research, collaboration, technology license, development, OEM, marketing or other similar agreements or strategic partnerships, provided such issuances are approved by the Board of Directors, and the holders of at least a majority of the then-outstanding shares of Series D Preferred Stock, and are primarily for non-equity financing purposes.

For the purpose of this subsection 4(d)(ii) of Article IV(B), "Allocated Amount" shall mean 1,152,050 shares of Common Stock (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) *minus* the aggregate number of shares of Common Stock (as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like) issued after the Filing Date pursuant to subsection 4(d)(ii)(E)(x) of Article IV(B) or subsection 4(d)(ii)(H)(x) of Article IV(B).

(iii) In the event this corporation should at any time or from time to time after the Filing Date fix a record date for the effectuation of a split or subdivision of the outstanding shares of Common Stock or the determination of holders of Common Stock entitled to receive a dividend or other distribution payable in additional shares of Common Stock or other securities or rights convertible into, or entitling the holder thereof to receive directly or indirectly, additional shares of Common Stock (hereinafter referred to as "Common Stock Equivalents") without payment of any consideration by such holder for the additional shares of Common Stock or the Common Stock Equivalents (including the additional shares of Common Stock issuable upon conversion or exercise thereof), then, as of such record date (or the date of such dividend distribution, split or subdivision if no record date is fixed), the Conversion Price of the Preferred Stock shall be appropriately decreased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be increased in proportion to such increase of the aggregate of shares of Common Stock outstanding and those issuable with respect to such Common Stock Equivalents with the number of shares issuable with respect to Common Stock Equivalents determined from time to time in the manner provided for deemed issuances in subsection 4(d)(i)(E) of Article IV(B).

(iv) If the number of shares of Common Stock outstanding at any time after the Filing Date is decreased by a combination of the outstanding shares of

Common Stock, then, following the record date of such combination, the Conversion Price for the Preferred Stock shall be appropriately increased so that the number of shares of Common Stock issuable on conversion of each share of such series shall be decreased in proportion to such decrease in outstanding shares.

(e) Other Distributions. In the event this corporation shall declare a distribution payable in securities of other persons, evidences of indebtedness issued by this corporation or other persons, assets (excluding cash dividends) or options or rights not referred to in subsection 4(d)(iii) of Article IV(B), then, in each such case for the purpose of this subsection 4(e) of Article IV(B), the holders of the Preferred Stock shall be entitled to a proportionate share of any such distribution as though they were the holders of the number of shares of Common Stock of this corporation into which their shares of Preferred Stock are convertible as of the record date fixed for the determination of the holders of Common Stock of this corporation entitled to receive such distribution.

(f) Recapitalizations. If at any time or from time to time there shall be a recapitalization of the Common Stock (other than a subdivision, combination or merger or sale of assets transaction provided for elsewhere in this Section 4 of Article IV(B) or in Section 2 of Article IV(B)) provision shall be made so that the holders of the Preferred Stock shall thereafter be entitled to receive upon conversion of the Preferred Stock the number of shares of stock or other securities or property of this corporation or otherwise, to which a holder of Common Stock deliverable upon conversion would have been entitled on such recapitalization. In any such case, appropriate adjustment shall be made in the application of the provisions of this Section 4 with respect to the rights of the holders of the Preferred Stock after the recapitalization to the end that the provisions of this Section 4 (including adjustment of the Conversion Price then in effect and the number of shares purchasable upon conversion of the Preferred Stock) shall be applicable after that event as nearly equivalently as may be practicable.

(g) No Impairment. This corporation will not, without the appropriate vote of the stockholders under the General Corporation Law or Section 6 of this Article IV(B), by amendment of its Restated Certificate of Incorporation or through any reorganization, recapitalization, transfer of assets, consolidation, merger, dissolution, issue or sale of securities or any other voluntary action, avoid or seek to avoid the observance or performance of any of the terms to be observed or performed hereunder by this corporation, but will at all times in good faith assist in the carrying out of all the provisions of this Section 4 of Article IV(B) and in the taking of all such action as may be necessary or appropriate in order to protect the Conversion Rights of the holders of the Preferred Stock against impairment.

(h) No Fractional Shares and Certificate as to Adjustments.

(i) No fractional shares shall be issued upon the conversion of any share or shares of the Preferred Stock and the aggregate number of shares of Common Stock to be issued to particular stockholders, shall be rounded down to the nearest whole share and this corporation shall pay in cash the fair market value of any fractional shares as of the time when entitlement to receive such fractions is determined. Whether or not fractional shares would be issuable upon such conversion shall be determined on the basis of the total number of shares of Preferred Stock the holder is at the time converting into Common Stock and the number of shares of Common Stock issuable upon such conversion.

(ii) Upon the occurrence of each adjustment or readjustment of the Conversion Price of Preferred Stock pursuant to this Section 4 of Article IV(B), this corporation, at its expense, shall promptly compute such adjustment or readjustment in accordance with the terms hereof and prepare and furnish to each holder of Preferred Stock a certificate setting forth such adjustment or readjustment and showing in detail the facts upon which such adjustment or readjustment is based. This corporation shall, upon the written request at any time of any holder of Preferred Stock, furnish or cause to be furnished to such holder a like certificate setting forth (A) such adjustment and readjustment, (B) the Conversion Price for such series of Preferred Stock at the time in effect, and (C) the number of shares of Common Stock and the amount, if any, of other property that at the time would be received upon the conversion of a share of Preferred Stock.

(i) Notices of Record Date. In the event of any taking by this corporation of a record of the holders of any class of securities for the purpose of determining the holders thereof who are entitled to receive any dividend (other than a cash dividend) or other distribution, this corporation shall mail to each holder of Preferred Stock, at least ten (10) days prior to the date specified therein, a notice specifying the date on which any such record is to be taken for the purpose of such dividend or distribution, and the amount and character of such dividend or distribution.

(j) Reservation of Stock Issuable Upon Conversion. This corporation shall at all times reserve and keep available out of its authorized but unissued shares of Common Stock, solely for the purpose of effecting the conversion of the shares of the Preferred Stock, such number of its shares of Common Stock as shall from time to time be sufficient to effect the conversion of all outstanding shares of the Preferred Stock; and if at any time the number of authorized but unissued shares of Common Stock shall not be sufficient to effect the conversion of all then outstanding shares of the Preferred Stock, in addition to such other remedies as shall be available to the holder of such Preferred Stock, this corporation will take such corporate action as may, in the opinion of its counsel, be necessary to increase its authorized but unissued shares of Common Stock to such number of shares as shall be sufficient for such purposes, including, without limitation, engaging in best efforts to obtain the requisite stockholder approval of any necessary amendment to this Restated Certificate of Incorporation.

(k) Waiver of Adjustment to Conversion Price. Notwithstanding anything herein to the contrary, any downward adjustment of the Conversion Price of any series of Preferred Stock may be waived, either prospectively or retroactively and either generally or in a particular instance, by the consent or vote of the holders of at least fifty-five percent (55%) of the outstanding shares of such series of Preferred Stock; provided, however, that in the case of the Series D Preferred Stock, such waiver must include at least three of the Requisite Lead Investors. Any such waiver shall bind all future holders of shares of such series of Preferred Stock.

(l) GSK Anti-Dilution. Until such time as the corporation or any predecessor entity has raised an aggregate amount of financing from the sale of its equity

securities equal to $10,000,000 in cash or in-kind (meaning the fair market value of office facilities, equipment and supplies transferred or made available to, and services performed for the benefit of, the corporation or any predecessor entity by the service provider under the that certain Services Agreement by and between Holdings and the corporation or any predecessor entity and the sole member of Holdings, dated as of February 27, 2009, each of which shall have a dollar value pursuant to the terms of such Services Agreement) (the "GSK Dilution Threshold") (other than issuances of securities as compensation or as part of an incentive arrangement pursuant to an equity incentive plan adopted by the corporation's Board of Directors or in connection with a split, dividend or similar event with respect to any class or type of securities), the corporation shall issue to GSK (as defined in that certain Amended and Restated Investors' Right Agreement dated on or about the Filing Date by and among this corporation and the other parties thereto, as amended from time to time (the "IRA")). such number of additional shares of Common Stock as is necessary to maintain GSK's Proportional Share (as defined below) as it was prior to the issuance of such shares of Common Stock. "GSK's Proportional Share" shall mean (A) the aggregate number of shares of Common Stock then owned by GSK, divided by (B) the aggregate number of shares of Common Stock then outstanding, in each case assuming the exercise or conversion of all securities exercisable for or convertible into Common Stock. For every issuance of additional shares of Common Stock by the corporation in connection with any financing (or portion thereof) received by the corporation above the GSK Dilution Threshold, GSK shall have no right to receive additional shares of Common Stock pursuant to this subsection 4(l) of Article IV(B) and GSK's Proportional Share shall be diluted to the same extent as other holders of Common Stock. The GSK Dilution Threshold was met on October 30, 2013.

(m) API Anti-Dilution. Until such time as the corporation or any predecessor entity has raised an aggregate amount of financing from the sale of its equity securities equal to $12,000,000 in cash, including $1,500,000 in cash from investors (the "API Dilution Threshold") (other than issuances of securities as compensation or as part of an incentive arrangement pursuant to an equity incentive plan adopted by the corporation's Board of Directors or in connection with a split, dividend or similar event with respect to any class or type of securities), the corporation shall issue to API (as defined in the IRA) such number of additional shares of Common Stock as is necessary to maintain API's Proportional Share (as defined below) as it was prior to the issuance of such shares of Common Stock. "API's Proportional Share" shall mean (A) the aggregate number of shares of Common Stock then owned by API, divided by (B) the aggregate number of shares of Common Stock then outstanding, in each case assuming the exercise or conversion of all securities exercisable for or convertible into Common Stock. It is acknowledged and agreed that API's Proportional Share as of October 30, 2013 was one-tenth (.10). For every issuance of additional shares of Common Stock by the corporation in connection with any financing (or portion thereof) received by the corporation above the API Dilution Threshold, API shall have no right to receive additional shares of Common Stock pursuant to this subsection 4(m) of Article IV(B) and API's Proportional Share shall be diluted to the same extent as other holders of Common Stock. The API Dilution Threshold was met on October 30, 2013.

5. Voting Rights.

(a) General Voting Rights. The holder of each share of Preferred Stock shall have the right to one vote for each share of Common Stock into which such Preferred Stock could then be converted, and with respect to such vote, such holder shall have full voting rights and powers equal to the voting rights and powers of the holders of Common Stock, and shall be entitled, notwithstanding any provision hereof, to notice of any stockholders' meeting in accordance with the Bylaws of this corporation, and except as provided by law or in subsection 5(b) of Article IV(B) below with respect to the election of directors by the separate class vote of the holders of Common Stock, shall be entitled to vote, together with holders of Common Stock, with respect to any question upon which holders of Common Stock have the right to vote. Fractional votes shall not, however, be permitted and any fractional voting rights available on an as-converted basis (after aggregating all shares into which shares of Preferred Stock held by each holder could be converted) shall be rounded to the nearest whole number (with one-half being rounded upward).

(b) Voting for the Election of Directors. As long as any shares of Series A Preferred Stock originally issued remain outstanding, the holders of Series A Preferred Stock, voting as a separate class, shall be entitled to elect three (3) directors of this corporation at any election of directors. As long as any shares of Series B Preferred Stock originally issued remain outstanding, the holders of the Series B Preferred Stock, voting as a separate class, shall be entitled to elect one (1) director of this corporation at any election of directors. As long as any shares of Series C Preferred Stock originally issued remain outstanding, the holders of the Series C Preferred Stock, voting as a separate class, shall be entitled to elect one (1) director of this corporation at any election of directors. As long as any shares of Series D Preferred Stock originally issued remain outstanding, the holders of the Series D Preferred Stock, voting as a separate class, shall be entitled to elect one (1) director of this corporation at any election of directors. The holders of outstanding Common Stock, voting as a separate class, shall be entitled to elect one (1) director of this corporation at any election of directors. The holders of Preferred Stock and Common Stock (voting together as a single class and not as separate series, and on an as-converted basis) shall be entitled to elect any remaining directors of this corporation.

Notwithstanding the provisions of Section 223(a)(1) and 223(a)(2) of the General Corporation Law, any vacancy, including newly created directorships resulting from any increase in the authorized number of directors or amendment of this Restated Certificate of Incorporation, and vacancies created by removal or resignation of a director, may be filled by a majority of the directors then in office, though less than a quorum, or by a sole remaining director, and the directors so chosen shall hold office until the next annual election and until their successors are duly elected and shall qualify, unless sooner displaced; provided, however, that where such vacancy occurs among the directors elected by the holders of a class or series of stock, the holders of shares of such class or series may override the Board's action to fill such vacancy by (i) voting for their own designee to fill such vacancy at a meeting of this corporation's stockholders or (ii) written consent, if the consenting stockholders hold a sufficient number of shares to elect their designee at a meeting of the stockholders. Any director may be removed during his or her term of office, either with or without cause, by, and only by, the affirmative vote of the holders of the shares of the class or series of stock entitled to elect such director or directors, given either at a special meeting of such stockholders duly called for that purpose or pursuant to a written consent of stockholders, and any vacancy thereby created may be filled by the holders of that class or series of stock represented at the meeting or pursuant to written consent.

6. Preferred Stock Protective Provisions. So long as at least 478,626 shares of Series A Preferred Stock, 381,259 shares of Series B Preferred Stock, 926,377 shares of Series C Preferred Stock or 1,473,369 shares of Series D Preferred Stock remain outstanding (each such share number as adjusted for any stock splits, stock dividends, combinations, subdivisions, recapitalizations or the like), this corporation shall not (by amendment, merger, consolidation or otherwise) without (in addition to any other vote required by law or the Restated Certificate of Incorporation) first obtaining the approval by vote or written consent, as provided by law, of the holders of a majority of the then outstanding shares of Preferred Stock (voting together as a single class and not as separate series, and on as as-converted basis), which vote or written consent must include at least three of the Requisite Lead Investors:

(a) consummate a Liquidation Event or effect any other merger or consolidation;

(b) amend, alter, repeal or waive any provision of this corporation's Restated Certificate of Incorporation or Bylaws;

(c) create, or authorize the creation of, or issue or obligate itself to issue any equity security (including any other security convertible into or exercisable for any such equity security) having a preference over, or being on a parity with, any series of Preferred Stock with respect to dividends, liquidation or redemption, other than the issuance of any authorized but unissued shares of Preferred Stock designated in this Restated Certificate of Incorporation (including any security convertible into or exercisable for such shares of Preferred Stock);

(d) (i) reclassify, alter or amend any existing class and series of this corporation's capital stock that is pari passu with the Preferred Stock with respect to dividends, liquidation or redemption, if such reclassification, alteration or amendment would render such other security senior to the Preferred Stock in respect of any such right, preference or privilege, or (ii) reclassify, alter or amend any existing class and series of this corporation's capital stock that is junior to the Preferred Stock in respect to dividends, liquidation or redemption, if such reclassification, alteration or amendment would render such other security senior to or pari passu with the Preferred Stock in respect of any such right, preference or privilege;

(e) redeem, repurchase or otherwise acquire (or pay into or set aside for a sinking fund for such purpose) (or permit any subsidiary to redeem, repurchase or otherwise acquire) any share or shares of Preferred Stock or Common Stock; provided, however, that this restriction shall not apply to (i) the repurchase of shares of Common Stock at the lower of the original purchase price or the then-current fair market value thereof from employees, officers, directors, consultants or other persons performing services for this corporation or any subsidiary pursuant to agreements under which this corporation has the option to repurchase such shares upon the occurrence of certain events, such as the termination of employment or service, or pursuant to a right of first refusal; or (ii) the redemption of any share or shares of Preferred Stock in accordance with Section 3 of Article IV(B);

(f) change the authorized number of directors of this corporation;

(g) create, or authorize the creation of, or issue, or authorize the issuance of any indebtedness for borrowed money, or permit any subsidiary to take any such action with respect to any indebtedness for borrowed money, if the aggregate indebtedness of the corporation and its subsidiaries for borrowed money following such action, other than equipment leases, would exceed 10% of the corporation's total assets; or

(h) create, or hold capital stock or other equity security in, any subsidiary that is not wholly owned (either directly or through one or more other subsidiaries) by this corporation, or sell, transfer or otherwise dispose of any capital stock or other equity security of any direct or indirect subsidiary of this corporation, or permit any direct or indirect subsidiary to sell, lease, transfer, exclusively license or otherwise dispose (in a single transaction or series of related transactions) of all or substantially all of the assets of such subsidiary.

7. Status of Redeemed or Converted Stock. In the event any shares of Preferred Stock shall be redeemed or converted pursuant to Section 3 of Article IV(B) or Section 4 of Article IV(B) hereof, the shares so redeemed or converted shall be cancelled and shall not be issuable by this corporation. The Restated Certificate of Incorporation of this corporation shall be appropriately amended to effect the corresponding reduction in this corporation's authorized capital stock.

8. Notices. Any notice required by the provisions of this Article IV(B) to be given to the holders of shares of Preferred Stock shall be deemed given (i) if deposited in the United States mail, postage prepaid, and addressed to each holder of record at his, her or its address appearing on the books of this corporation, (ii) if such notice is provided by electronic transmission in a manner permitted by Section 232 of the General Corporation Law, or (iii) if such notice is provided in another manner then permitted by the General Corporation Law.

C. Common Stock. The rights, preferences, privileges and restrictions granted to and imposed on the Common Stock are as set forth below in this Article IV(C).

1. Dividend Rights. Subject to the prior rights of holders of all classes of stock at the time outstanding having prior rights as to dividends, the holders of the Common Stock shall be entitled to receive, when, as and if declared by the Board of Directors, out of any assets of this corporation legally available therefor, any dividends as may be declared from time to time by the Board of Directors.

2. Liquidation Rights. Upon the liquidation, dissolution or winding up of this corporation, the assets of this corporation shall be distributed as provided in Section 2 of Article IV(B) hereof.

3. Redemption. The Common Stock is not redeemable at the option of the holder.

4. General Voting Rights. The holder of each share of Common Stock shall have the right to one vote for each such share, and shall be entitled to notice of any stockholders' meeting in accordance with the Bylaws of this corporation, and shall be entitled to vote upon such matters and in such manner as may be provided by law; provided, however, that, except as otherwise required by law, holders of Common Stock, as such, shall not be entitled to vote on any amendment to the Restated Certificate of Incorporation that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together with the holders of one or more other such series, to vote thereon pursuant to the Restated Certificate of Incorporation or pursuant to the General Corporation Law. The number of authorized shares of Common Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by the affirmative vote of the holders of a majority of the stock of this corporation entitled to vote, irrespective of the provisions of Section 242(b)(2) of the General Corporation Law.

ARTICLE V

Except as otherwise provided in this Restated Certificate of Incorporation, in furtherance and not in limitation of the powers conferred by statute, the Board of Directors is expressly authorized to make, repeal, alter, amend and rescind any or all of the Bylaws of this corporation.

ARTICLE VI

The number of directors of this corporation shall be determined in the manner set forth in the Bylaws of this corporation.

ARTICLE VII

Elections of directors need not be by written ballot unless the Bylaws of this corporation shall so provide.

ARTICLE VIII

Meetings of stockholders may be held within or without the State of Delaware, as the Bylaws of this corporation may provide. The books of this corporation may be kept (subject to any provision contained in the statutes) outside the State of Delaware at such place or places as may be designated from time to time by the Board of Directors or in the Bylaws of this corporation.

ARTICLE IX

A director of this corporation shall not be personally liable to this corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability (i) for any breach of the director's duty of loyalty to this corporation or its stockholders, (ii) for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law, (iii) under Section 174 of the General Corporation Law, or (iv) for any transaction from which the director derived any improper personal benefit. If the General Corporation Law is amended after approval by the stockholders of this Article IX to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of this corporation shall be eliminated or limited to the fullest extent permitted by the General Corporation Law as so amended.

Any amendment, repeal or modification of the foregoing provisions of this Article IX by the stockholders of this corporation shall not adversely affect any right or protection of a director of this corporation existing at the time of, or increase the liability of any director of this corporation with respect to any acts or omissions of such director occurring prior to, such amendment, repeal or modification.

ARTICLE X

This corporation reserves the right to amend, alter, change or repeal any provision contained in this Certificate of Incorporation, in the manner now or hereafter prescribed by statute, and all rights conferred upon stockholders herein are granted subject to this reservation.

ARTICLE XI

To the fullest extent permitted by applicable law, this corporation is authorized to provide indemnification of (and advancement of expenses to) directors, officers, employees and agents of this corporation (and any other persons to which General Corporation Law permits this corporation to provide indemnification) through Bylaw provisions, agreements with such persons, vote of stockholders or disinterested directors or otherwise, in excess of the indemnification and advancement otherwise permitted by Section 145 of the General Corporation Law, subject only to limits created by applicable General Corporation Law (statutory or non-statutory), with respect to actions for breach of duty to this corporation, its stockholders, and others.

Any amendment, repeal or modification of the foregoing provisions of this Article XI shall not adversely affect any right or protection of a director, officer, employee, agent or other person existing at the time of, or increase the liability of any such person with respect to any acts or omissions of such person occurring prior to, such amendment, repeal or modification.

ARTICLE XII

This corporation renounces any interest or expectancy of this corporation in, or in being offered an opportunity to participate in, an Excluded Opportunity. An "Excluded Opportunity" is any matter, transaction or interest that is presented to, or acquired, created or developed by, or which otherwise comes into the possession of, (i) any director of this corporation who is not an employee of this corporation or any of its subsidiaries, or (ii) any holder of Preferred Stock or any partner, member, director, stockholder, employee or agent of any such holder, other than someone who is an employee of this corporation or any of its subsidiaries (collectively, "Covered Persons"), unless such matter, transaction or interest is presented to, or acquired, created or developed by, or otherwise comes into the possession of, a Covered Person expressly and solely in such Covered Person's capacity as a director of this corporation. No amendment or repeal of this Article XII shall apply to or have any effect on the liability or alleged liability of any Covered Persons, for or with respect to any opportunities of which such Covered Person become aware prior to such amendment or repeal.

* * *

THIRD: The foregoing amendment and restatement was approved by the holders of the requisite number of shares of said corporation in accordance with Section 228 of the General Corporation Law.

FOURTH: That said Restated Certificate of Incorporation, which restates and integrates and further amends the provisions of this corporation's Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the General Corporation Law.

IN WITNESS WHEREOF, this Restated Certificate of Incorporation has been executed by a duly authorized officer of this corporation on this 14th day of May, 2015.

/s/ Kenneth T. Mills
Kenneth T. Mills, President and CEO

SIGNATURE PAGE TO RESTATED CERTIFICATE OF INCORPORATION FOR REGENXBIO INC.

BYLAWS OF
REGENXBIO INC.
(A DELAWARE CORPORATION)

TABLE OF CONTENTS

BYLAWS
OF
REGENEX BIOSCIENCES, INC.

ARTICLE I
OFFICES

1.1 **Registered Office**. The registered office shall be in the City of Wilmington, County of New Castle, State of Delaware.

1.2 **Offices**. The corporation may also have offices at such other places both within and without the State of Delaware as the Board of Directors may from time to time determine or the business of the corporation may require.

ARTICLE II
MEETINGS OF STOCKHOLDERS

2.1 **Location**. All meetings of the stockholders for the election of directors shall be held in the City of Washington, District of Columbia, at such place as may be fixed from time to time by the Board of Directors, or at such other place either within or without the State of Delaware as shall be designated from time to time by the Board of Directors and stated in the notice of the meeting; provided, however, that the Board of Directors may, in its sole discretion, determine that the meeting shall not be held at any place, but may instead be held solely by means of remote communication as authorized by Section 211 of the Delaware General Corporations Law ("DGCL"). Meetings of stockholders for any other purpose may be held at such time and place, if any, within or without the State of Delaware, as shall be stated in the notice of the meeting or in a duly executed waiver of notice thereof, or a waiver by electronic transmission by the person entitled to notice.

2.2 **Timing**. Annual meetings of stockholders, commencing with the year 2015, shall be held at such date and time as shall be designated from time to time by the Board of Directors and stated in the notice of the meeting, at which they shall elect by a plurality vote a Board of Directors, and transact such other business as may properly be brought before the meeting.

2.3 **Notice of Meeting**. Written notice of any stockholder meeting stating the place, if any, date and hour of the meeting, the means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such meeting, shall be given to each stockholder entitled to vote at such meeting not fewer than ten (10) nor more than sixty (60) days before the date of the meeting.

2.4 **Stockholders' Records**. The officer who has charge of the stock ledger of the corporation shall prepare and make, at least ten (10) days before every meeting of stockholders, a complete list of the stockholders entitled to vote at the meeting, arranged in alphabetical order, and showing the address (but not the electronic address or other electronic contact information) of each stockholder and the number of shares registered in the name of each

stockholder. Such list shall be open to the examination of any stockholder, for any purpose germane to the meeting for a period of at least 10 days prior to the meeting: (i) on a reasonably accessible electronic network, provided that the information required to gain access to such list is provided with the notice of the meeting, or (ii) during ordinary business hours, at the principal place of business of the corporation. In the event that the corporation determines to make the list available on an electronic network, the corporation may take reasonable steps to ensure that such information is available only to stockholders of the corporation. If the meeting is to be held at a place, then the list shall be produced and kept at the time and place of the meeting during the whole time thereof, and may be inspected by any stockholder who is present. If the meeting is to be held solely by means of remote communication, then the list shall also be open to the examination of any stockholder during the whole time of the meeting on a reasonably accessible electronic network, and the information required to access such list shall be provided with the notice of the meeting.

2.5 **Special Meetings**. Special meetings of the stockholders, for any purpose or purposes, unless otherwise prescribed by statute or by the certificate of incorporation, may be called by (i) not less than two (2) members of the Board of Directors, (ii) by stockholders owning at least twenty percent (20%) of the corporation's outstanding Common Stock or (iii) by stockholders owning at least twenty percent (20%) of the corporation's outstanding Preferred Stock. Such request shall state the purpose or purposes of the proposed meeting.

2.6 **Notice of Meeting**. Written notice of a special meeting stating the place, date and hour of the meeting and the purpose or purposes for which the meeting is called, shall be given not fewer than ten (10) nor more than sixty (60) days before the date of the meeting, to each stockholder entitled to vote at such meeting. The means of remote communication, if any, by which stockholders and proxyholders may be deemed to be present in person and vote at such meeting shall also be provided in the notice.

2.7 **Business Transacted at Special Meeting**. Business transacted at any special meeting of stockholders shall be limited to the purposes stated in the notice.

2.8 **Quorum; Meeting Adjournment; Presence by Remote Means**.

(a) *Quorum; Meeting Adjournment*. The holders of a majority of the stock issued and outstanding and entitled to vote thereat, present in person or represented by proxy, shall constitute a quorum at all meetings of the stockholders for the transaction of business except as otherwise provided by statute or by the certificate of incorporation. If, however, such quorum shall not be present or represented at any meeting of the stockholders, the stockholders entitled to vote thereat, present in person or represented by proxy, shall have power to adjourn the meeting from time to time, without notice other than announcement at the meeting, until a quorum shall be present or represented. At such adjourned meeting at which a quorum shall be present or represented, any business may be transacted that might have been transacted at the meeting as originally notified. If the adjournment is for more than thirty (30) days, or if after the adjournment a new record date is fixed for the adjourned meeting, a notice of the adjourned meeting shall be given to each stockholder of record entitled to vote at the meeting.

(b) *Presence by Remote Means*. If authorized by the Board of Directors in its sole discretion, and subject to such guidelines and procedures as the Board of Directors may adopt, stockholders and proxyholders not physically present at a meeting of stockholders may, by means of remote communication:

(1) participate in a meeting of stockholders; and

(2) be deemed present in person and vote at a meeting of stockholders whether such meeting is to be held at a designated place or solely by means of remote communication, provided that (i) the corporation shall implement reasonable measures to verify that each person deemed present and permitted to vote at the meeting by means of remote communication is a stockholder or proxyholder, (ii) the corporation shall implement reasonable measures to provide such stockholders and proxyholders a reasonable opportunity to participate in the meeting and to vote on matters submitted to the stockholders, including an opportunity to read or hear the proceedings of the meeting substantially concurrently with such proceedings, and (iii) if any stockholder or proxyholder votes or takes other action at the meeting by means of remote communication, a record of such vote or other action shall be maintained by the corporation.

2.9 **Voting Thresholds**. When a quorum is present at any meeting, the vote of the holders of a majority of the stock having voting power present in person or represented by proxy shall decide any question brought before such meeting, unless the question is one upon which by express provision of the statutes or of the certificate of incorporation, a different vote is required, in which case such express provision shall govern and control the decision of such question.

2.10 **Number of Votes Per Share**. Unless otherwise provided in the certificate of incorporation, each stockholder shall at every meeting of the stockholders be entitled to one vote by such stockholder or by proxy for each share of the capital stock having voting power held by such stockholder, but no proxy shall be voted on after three years from its date, unless the proxy provides for a longer period.

2.11 **Action by Written Consent of Stockholders; Electronic Consent; Notice of Action**.

(a) *Action by Written Consent of Stockholders*. Unless otherwise provided by the certificate of incorporation, any action required or permitted to be taken at any annual or special meeting of the stockholders may be taken without a meeting, without prior notice and without a vote, if a consent in writing setting forth the action so taken, is signed in a manner permitted by law by the holders of outstanding stock having not less than the number of votes that would be necessary to authorize or take such action at a meeting at which all shares entitled to vote thereon were present and voted. Written stockholder consents shall bear the date of signature of each stockholder who signs the consent in the manner permitted by law and shall be delivered to the corporation as provided in subsection (b) below. No written consent shall be effective to take the action set forth therein unless, within sixty (60) days of the earliest dated consent delivered to the corporation in the manner provided above, written consents signed by a sufficient number of stockholders to take the action set forth therein are delivered to the corporation in the manner provided above.

(b) *Electronic Consent*. A telegram, cablegram or other electronic transmission consenting to an action to be taken and transmitted by a stockholder or proxyholder, or a person or persons authorized to act for a stockholder or proxyholder, shall be deemed to be written, signed and dated for the purposes of this section, provided that any such telegram, cablegram or other electronic transmission sets forth or is delivered with information from which the corporation can determine (1) that the telegram, cablegram or other electronic transmission was transmitted by the stockholder or proxyholder or by a person or persons authorized to act for the stockholder or proxyholder and (2) the date on which such stockholder or proxyholder or authorized person or persons transmitted such telegram, cablegram or electronic transmission. The date on which such telegram, cablegram or electronic transmission is transmitted shall be deemed to be the date on which such consent was signed. No consent given by telegram, cablegram or other electronic transmission shall be deemed to have been delivered until such consent is reproduced in paper form and until such paper form is delivered to the corporation by delivery to its registered office in the State of Delaware, its principal place of business or an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded. Delivery made to a corporation's registered office shall be made by hand or by certified or registered mail, return receipt requested. Notwithstanding the foregoing limitations on delivery, consents given by telegram, cablegram or other electronic transmission may be otherwise delivered to the principal place of business of the corporation or to an officer or agent of the corporation having custody of the book in which proceedings of meetings of stockholders are recorded if, to the extent and in the manner provided by resolution of the Board of Directors of the corporation.

(c) *Notice of Action*. Prompt notice of any action taken pursuant to this Section 2.11 shall be provided to the stockholders in accordance with Section 228(e) of the DGCL.

ARTICLE III
DIRECTORS

3.1 **Authorized Directors**. The number of directors that shall constitute the whole Board of Directors shall be determined by resolution of the Board of Directors or by the stockholders at the annual meeting of the stockholders, except as provided in Section 3.2 of this Article, and each director elected shall hold office until his or her successor is elected and qualified. Directors need not be stockholders.

3.2 **Vacancies**. Unless otherwise provided in the corporation's certificate of incorporation, as it may be amended, vacancies and newly created directorships resulting from any increase in the authorized number of directors may be filled by a majority of the directors then in office, though less than a quorum, or by a sole remaining director, and the directors so chosen shall hold office until the next annual election and until their successors are duly elected and shall qualify, unless sooner displaced. If there are no directors in office, then an election of directors may be held in the manner provided by statute. If, at the time of filling any vacancy or any newly created directorship, the directors then in office shall constitute less than a majority of

the whole Board of Directors (as constituted immediately prior to any such increase), the Court of Chancery may, upon application of any stockholder or stockholders holding at least ten percent (10%) of the total number of the shares at the time outstanding having the right to vote for such directors, summarily order an election to be held to fill any such vacancies or newly created directorships, or to replace the directors chosen by the directors then in office.

3.3 **Board Authority**. The business of the corporation shall be managed by or under the direction of its Board of Directors, which may exercise all such powers of the corporation and do all such lawful acts and things as are not by statute or by the certificate of incorporation or by these bylaws directed or required to be exercised or done by the stockholders.

3.4 **Location of Meetings**. The Board of Directors of the corporation may hold meetings, both regular and special, either within or without the State of Delaware.

3.5 **First Meeting**. The first meeting of each newly elected Board of Directors shall be held at such time and place as shall be fixed by the vote of the stockholders at the annual meeting and no notice of such meeting shall be necessary to the newly elected directors in order to legally constitute the meeting, provided a quorum shall be present. In the event of the failure of the stockholders to fix the time or place of such first meeting of the newly elected Board of Directors, or in the event such meeting is not held at the time and place so fixed by the stockholders, the meeting may be held at such time and place as shall be specified in a notice given as hereinafter provided for special meetings of the Board of Directors, or as shall be specified in a written waiver signed by all of the directors.

3.6 **Regular Meetings**. Regular meetings of the Board of Directors may be held without notice at such time and at such place as shall from time to time be determined by the Board of Directors.

3.7 **Special Meetings**. Special meetings of the Board of Directors may be called by the Chief Executive Officer or secretary upon at least one (1) business day's notice to each director and on like notice on the written request of two (2) directors unless the Board of Directors consists of only one director, in which case special meetings shall be called by the Chief Executive Officer or secretary in like manner and on like notice on the written request of the sole director. Notice of any special meeting shall be given to each director at his or her business or residence in writing, or by telegram, facsimile transmission, telephone communication or electronic transmission (provided, with respect to electronic transmission, that the director has consented to receive the form of transmission at the address to which it is directed). If mailed, such notice shall be deemed adequately delivered when deposited in the United States mails so addressed, with postage thereon prepaid, at least five (5) days before such meeting. If by telegram, such notice shall be deemed adequately delivered when the telegram is delivered to the telegraph company at least twenty-four (24) hours before such meeting. If by facsimile transmission or other electronic transmission, such notice shall be transmitted at least twenty-four (24) hours before such meeting. If by telephone, the notice shall be given at least twelve (12) hours prior to the time set for the meeting. Neither the business to be transacted at, nor the purpose of, any regular or special meeting of the Board of Directors need be specified in the notice of such meeting, except for amendments to these Bylaws as provided under Section 8.1 of Article VIII hereof. A meeting may be held at any time without notice if all the directors are present (except as otherwise provided by law) or if those not present waive notice of the meeting in writing, either before or after such meeting.

3.8 **Quorum**. At all meetings of the Board of Directors a majority of the directors shall constitute a quorum for the transaction of business and any act of a majority of the directors present at any meeting at which there is a quorum shall be an act of the Board of Directors, except as may be otherwise specifically provided by statute or by the certificate of incorporation. If a quorum is not present at any meeting of the Board of Directors, the directors present thereat may adjourn the meeting from time to time, without notice other than announcement at the meeting, until a quorum shall be present.

3.9 **Action Without a Meeting**. Unless otherwise restricted by the certificate of incorporation or these bylaws, any action required or permitted to be taken at any meeting of the Board of Directors or of any committee thereof may be taken without a meeting if all members of the Board of Directors or committee, as the case may be, consent thereto in writing or by electronic transmission, and the writing, electronic transmission or transmissions are filed with the minutes of proceedings of the Board of Directors or committee.

3.10 **Telephonic Meetings**. Unless otherwise restricted by the certificate of incorporation or these bylaws, members of the Board of Directors or any committee designated by the Board of Directors may participate in a meeting of the Board of Directors or any committee, by means of conference telephone or other means of communication by which all persons participating in the meeting can hear each other, and such participation shall constitute presence in person at the meeting.

3.11 **Committees**. The Board of Directors may designate one or more committees, each committee to consist of one or more of the directors of the corporation. The Board of Directors may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee. The directors nominated by the holders of such shares of Series A Preferred Stock, voting as a separate class under the certificate of incorporation, shall constitute the majority of the members of any such committee.

In the absence or disqualification of a member of a committee, the member or members thereof present at any meeting and not disqualified from voting, whether or not he or she or they constitute a quorum, may unanimously appoint another member of the Board of Directors to act at the meeting in the place of any such absent or disqualified member.

Any such committee, to the extent provided in the resolution of the Board of Directors, shall have and may exercise all the powers and authority of the Board of Directors in the management of the business and affairs of the corporation, and may authorize the seal of the corporation to be affixed to all papers which may require it, but no such committee shall have the power or authority in reference to the following matters: (i) approving or adopting, or recommending to the stockholders, any action or matter expressly required by the DGCL to be submitted to stockholders for approval or (ii) adopting, amending or repealing any provision of these bylaws.

3.12 **Minutes of Meetings**. Each committee shall keep regular minutes of its meetings and report the same to the Board of Directors when required.

3.13 **Compensation of Directors**. Unless otherwise restricted by the certificate of incorporation or these bylaws, the Board of Directors shall have the authority to fix the compensation of directors. The directors may be paid their expenses, if any, of attendance at each meeting of the Board of Directors and may be paid a fixed sum for attendance at each meeting of the Board of Directors or a stated salary as director. No such payment shall preclude any director from serving the corporation in any other capacity and receiving compensation therefor. Members of special or standing committees may be allowed like compensation for attending committee meetings.

3.14 **Removal of Directors**. Unless otherwise provided by the certificate of incorporation or these bylaws, any director or the entire Board of Directors may be removed, with or without cause, by the holders of a majority of shares entitled to vote at an election of directors.

ARTICLE IV
NOTICES

4.1 **Notice**. Unless otherwise provided in these bylaws, whenever, under the provisions of the statutes or of the certificate of incorporation or of these bylaws, notice is required to be given to any director or stockholder, it shall not be construed to mean personal notice, but such notice may be given in writing, by mail, addressed to such director or stockholder, at his or her address as it appears on the records of the corporation, with postage thereon prepaid, and such notice shall be deemed to be given at the time when the same shall be deposited in the United States mail. Notice to directors may also be given by telegram.

4.2 **Waiver of Notice**. Whenever any notice is required to be given under the provisions of the statutes or of the certificate of incorporation or of these bylaws, a waiver thereof in writing, signed by the person or persons entitled to said notice, whether before or after the time stated therein, shall be deemed equivalent thereto.

4.3 **Electronic Notice**.

(a) *Electronic Transmission*. Without limiting the manner by which notice otherwise may be given effectively to stockholders and directors, any notice to stockholders or directors given by the corporation under any provision of the DGCL, the certificate of incorporation or these bylaws shall be effective if given by a form of electronic transmission consented to by the stockholder or director to whom the notice is given. Any such consent shall be revocable by the stockholder or director by written notice to the corporation. Any such consent shall be deemed revoked if (1) the corporation is unable to deliver by electronic transmission two consecutive notices given by the corporation in accordance with such consent and (2) such inability becomes known to the secretary or an assistant secretary of the corporation or to the transfer agent, or other person responsible for the giving of notice; provided, however, the inadvertent failure to treat such inability as a revocation shall not invalidate any meeting or other action.

(b) *Effective Date of Notice*. Notice given pursuant to subsection (a) of this section shall be deemed given: (1) if by facsimile telecommunication, when directed to a number at which the stockholder or director has consented to receive notice; (2) if by electronic mail, when directed to an electronic mail address at which the stockholder or director has consented to receive notice; (3) if by a posting on an electronic network together with separate notice to the stockholder or director of such specific posting, upon the later of (i) such posting and (ii) the giving of such separate notice; and (4) if by any other form of electronic transmission, when directed to the stockholder or director. An affidavit of the secretary or an assistant secretary or of the transfer agent or other agent of the corporation that the notice has been given by a form of electronic transmission shall, in the absence of fraud, be prima facie evidence of the facts stated therein.

(c) *Form of Electronic Transmission*. For purposes of these bylaws, "electronic transmission" means any form of communication, not directly involving the physical transmission of paper, that creates a record that may be retained, retrieved, and reviewed by a recipient thereof, and that may be directly reproduced in paper form by such a recipient through an automated process.

ARTICLE V
OFFICERS

5.1 **Required and Permitted Officers**. The officers of the corporation shall be chosen by the Board of Directors and shall be a Chief Executive Officer and/or a president, a treasurer and a secretary. The Board of Directors may elect from among its members a Chairman of the Board and a Vice-Chairman of the Board. The Board of Directors may also choose one or more vice-presidents, assistant secretaries and assistant treasurers. Any number of offices may be held by the same person, unless the certificate of incorporation or these bylaws otherwise provide.

5.2 **Appointment of Required Officers**. The Board of Directors at its first meeting after each annual meeting of stockholders shall choose a Chief Executive Officer and/or a president , a president, a treasurer, and a secretary and may choose vice-presidents.

5.3 **Appointment of Permitted Officers**. The Board of Directors may appoint such other officers and agents as it shall deem necessary who shall hold their offices for such terms and shall exercise such powers and perform such duties as shall be determined from time to time by the Board of Directors.

5.4 **Officer Compensation**. The salaries of all officers and agents of the corporation shall be fixed by the Board of Directors.

5.5 **Term of Office; Vacancies**. The officers of the corporation shall hold office until their successors are chosen and qualify. Any officer elected or appointed by the Board of Directors may be removed at any time by the affirmative vote of a majority of the Board of Directors. Any vacancy occurring in any office of the corporation shall be filled by the Board of Directors.

THE CHAIRMAN OF THE BOARD

5.6 **Chairman Presides**. Unless the Board of Directors appoints a Chairman of the Board, the Chief Executive Officer shall be the Chairman of the Board, so long as the Chief Executive Officer is a director of the corporation. The Chairman of the Board shall preside at all meetings of the Board of Directors and of the stockholders at which he or she shall be present. He or she shall have and may exercise such powers as are, from time to time, assigned to him or her by the Board of Directors and as may be provided by law.

5.7 **Absence of Chairman**. In the absence of the Chairman of the Board, the Vice-Chairman of the Board, if any, shall preside at all meetings of the Board of Directors and of the stockholders at which he or she shall be present. He or she shall have and may exercise such powers as are, from time to time, assigned to him or her by the Board of Directors and as may be provided by law.

THE CHIEF EXECUTIVE OFFICER

5.8 **Powers of Chief Executive Officer**. The Chief Executive Officer shall have general and active management of the business of the corporation and shall see that all orders and resolutions of the Board of Directors are carried into effect.

5.9 **Chief Executive Officer's Signature Authority**. The Chief Executive Officer shall execute bonds, mortgages and other contracts requiring a seal, under the seal of the corporation, except where required or permitted by law to be otherwise signed and executed and except where the signing and execution thereof shall be expressly delegated by the Board of Directors to some other officer or agent of the corporation. The Chief Executive Officer may sign certificates for shares of stock of the corporation.

5.10 **Absence of Chief Executive Officer**. In the absence of the Chief Executive Officer or in the event of his or her inability or refusal to act, the president shall perform the duties of the Chief Executive Officer, and when so acting, shall have all the powers of and be subject to all the restrictions upon the Chief Executive Officer.

THE PRESIDENT AND VICE-PRESIDENTS

5.11 **Powers of President**. Unless the Board of Directors appoints a president of the corporation, the Chief Executive Officer shall be the president of the corporation. The president of the corporation shall have such powers as required by law and shall perform such other duties and have such other powers as the Board of Directors may from time to time prescribe.

5.12 **Absence of President**. In the absence of the president or in the event of his or her inability or refusal to act, the vice-president, if any, (or in the event there be more than one vice-president, the vice-presidents in the order designated by the directors, or in the absence of any designation, then in the order of their election) shall perform the duties of the president, and when so acting, shall have all the powers of and be subject to all the restrictions upon the president. The vice-presidents shall perform such other duties and have such other powers as the Board of Directors may from time to time prescribe.

THE SECRETARY AND ASSISTANT SECRETARY

5.13 **Duties of Secretary**. The secretary shall attend all meetings of the Board of Directors and all meetings of the stockholders and record all the proceedings of the meetings of the corporation and of the Board of Directors in a book to be kept for that purpose and shall perform like duties for the standing committees when required. He or she shall give, or cause to be given, notice of all meetings of the stockholders and special meetings of the Board of Directors, and shall perform such other duties as may be prescribed by the Board of Directors or the Chief Executive Officer, under whose supervision he or she shall be. He or she shall have custody of the corporate seal of the corporation and he or she, or an assistant secretary, shall have authority to affix the same to any instrument requiring it and when so affixed, it may be attested by his or her signature or by the signature of such assistant secretary. The Board of Directors may give general authority to any other officer to affix the seal of the corporation and to attest the affixing by his or her signature.

5.14 **Duties of Assistant Secretary**. The assistant secretary, or if there be more than one, the assistant secretaries in the order determined by the Board of Directors (or if there be no such determination, then in the order of their election) shall, in the absence of the secretary or in the event of his or her inability or refusal to act, perform the duties and exercise the powers of the secretary and shall perform such other duties and have such other powers as the Board of Directors may from time to time prescribe.

THE TREASURER AND ASSISTANT TREASURERS

5.15 **Duties of Treasurer**. The treasurer shall have the custody of the corporate funds and securities and shall keep full and accurate accounts of receipts and disbursements in books belonging to the corporation and shall deposit all moneys and other valuable effects in the name and to the credit of the corporation in such depositories as may be designated by the Board of Directors.

5.16 **Disbursements and Financial Reports**. He or she shall disburse the funds of the corporation as may be ordered by the Board of Directors, taking proper vouchers for such disbursements, and shall render to the Chief Executive Officer and the Board of Directors, at its regular meetings or when the Board of Directors so requires, an account of all his or her transactions as treasurer and of the financial condition of the corporation.

5.17 **Treasurer's Bond**. If required by the Board of Directors, the treasurer shall give the corporation a bond (which shall be renewed every six years) in such sum and with such surety or sureties as shall be satisfactory to the Board of Directors for the faithful performance of the duties of his or her office and for the restoration to the corporation, in case of his or her death, resignation, retirement or removal from office, of all books, papers, vouchers, money and other property of whatever kind in his or her possession or under his or her control belonging to the corporation.

5.18 **Duties of Assistant Treasurer**. The assistant treasurer, or if there shall be more than one, the assistant treasurers in the order determined by the Board of Directors (or if there be no such determination, then in the order of their election) shall, in the absence of the treasurer or in the event of the treasurer's inability or refusal to act, perform the duties and exercise the powers of the treasurer and shall perform such other duties and have such other powers as the Board of Directors may from time to time prescribe.

ARTICLE VI
CERTIFICATE OF STOCK

6.1 **Stock Certificates**. Every holder of stock in the corporation shall be entitled to have a certificate, signed by or in the name of the corporation by, the Chairman or Vice-Chairman of the Board of Directors, or the president or a vice-president and the treasurer or an assistant treasurer, or the secretary or an assistant secretary of the corporation, certifying the number of shares owned by him or her in the corporation.

Certificates may be issued for partly paid shares and in such case upon the face or back of the certificates issued to represent any such partly paid shares, the total amount of the consideration to be paid therefor, and the amount paid thereon shall be specified.

If the corporation shall be authorized to issue more than one class of stock or more than one series of any class, the powers, designations, preferences and relative participating, optional or other special rights of each class of stock or series thereof and the qualification, limitations or restrictions of such preferences and/or rights shall be set forth in full or summarized on the face or back of the certificate which the corporation shall issue to represent such class or series of stock, provided that, except as otherwise provided in Section 202 of the DGCL, in lieu of the foregoing requirements, there may be set forth on the face or back of the certificate which the corporation shall issue to represent such class or series of stock, a statement that the corporation will furnish without charge to each stockholder who so requests the powers, designations, preferences and relative participating, optional or other special rights of each class of stock or series thereof and the qualifications, limitations or restrictions of such preferences and/or rights.

6.2 **Facsimile Signatures**. Any or all of the signatures on the certificate may be facsimile. In the event that any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed upon a certificate shall have ceased to be such officer, transfer agent or registrar before such certificate is issued, the certificate may be issued by the corporation with the same effect as if such officer, transfer agent or registrar were still acting as such at the date of issue.

6.3 **Lost Certificates**. The Board of Directors may direct a new certificate or certificates to be issued in place of any certificate or certificates theretofore issued by the corporation alleged to have been lost, stolen or destroyed upon the making of an affidavit of that fact by the person claiming the certificate to be lost, stolen or destroyed. When authorizing such issuance of a new certificate or certificates, the Board of Directors may, in its discretion and as a condition precedent to the issuance, require the owner of such lost, stolen or destroyed certificate or certificates, or his or her legal representative, to advertise the same in such manner as it shall require and/or to give the corporation a bond in such sum as it may direct as indemnity against any claim that may be made against the corporation with respect to the certificate alleged to have been lost, stolen or destroyed.

6.4 **Transfer of Stock**. Upon surrender to the corporation or the transfer agent of the corporation of a certificate for shares duly endorsed or accompanied by proper evidence of succession, assignation or authority to transfer, it shall be the duty of the corporation to issue a new certificate to the person entitled thereto, cancel the old certificate and record the transaction upon its books.

6.5 **Fixing a Record Date**. In order that the corporation may determine the stockholders entitled to notice of or to vote at any meeting of stockholders or any adjournment thereof, or to express consent to corporate action in writing without a meeting, or entitled to receive payment of any dividend or other distribution or allotment of any rights, or entitled to exercise any rights in respect of any change, conversion or exchange of stock or for the purpose of any other lawful action, the Board of Directors may fix a record date which shall not be more than sixty (60) nor less than ten (10) days before the date of such meeting, nor more than sixty (60) days prior to any other action. A determination of stockholders of record entitled to notice of or to vote at a meeting of stockholders shall apply to any adjournment of the meeting; provided, however, that the Board of Directors may fix a new record date for the adjourned meeting.

6.6 **Registered Stockholders**. The corporation shall be entitled to recognize the exclusive right of a person registered on its books as the owner of shares to receive dividends, to vote as such owner, to hold liable for calls and assessments a person registered on its books as the owner of shares and shall not be bound to recognize any equitable or other claim to or interest in such share or shares on the part of any other person, whether or not it shall have express or other notice thereof, except as otherwise provided by the laws of Delaware.

ARTICLE VII
GENERAL PROVISIONS

7.1 **Dividends**. Dividends upon the capital stock of the corporation, if any, subject to the provisions of the certificate of incorporation, may be declared by the Board of Directors at any regular or special meeting, pursuant to law. Dividends may be paid in cash, in property or in shares of the capital stock, subject to the provisions of the certificate of incorporation.

7.2 **Reserve for Dividends**. Before payment of any dividend, there may be set aside out of any funds of the corporation available for dividends such sum or sums as the directors from time to time, in their sole discretion, think proper as a reserve or reserves to meet contingencies, or for equalizing dividends, or for repairing or maintaining any property of the corporation, or for such other purposes as the directors think conducive to the interests of the corporation, and the directors may modify or abolish any such reserve in the manner in which it was created.

7.3 **Checks**. All checks or demands for money and notes of the corporation shall be signed by such officer or officers or such other person or persons as the Board of Directors may from time to time designate.

7.4 **Fiscal Year**. The fiscal year of the corporation shall be fixed by resolution of the Board of Directors.

7.5 **Corporate Seal**. The Board of Directors may adopt a corporate seal having inscribed thereon the name of the corporation, the year of its organization and the words “Corporate Seal, Delaware.” The seal may be used by causing it or a facsimile thereof to be impressed or affixed or otherwise reproduced.

7.6 **Indemnification**. The corporation shall, to the fullest extent authorized under the laws of the State of Delaware, as those laws may be amended and supplemented from time to time, indemnify any director made, or threatened to be made, a party to an action or proceeding, whether criminal, civil, administrative or investigative, by reason of being a director of the corporation or a predecessor corporation or a director or officer of another corporation, if such person served in such position at the request of the corporation; provided, however, that the corporation shall indemnify any such director or officer in connection with a proceeding initiated by such director or officer only if such proceeding was authorized by the Board of Directors of the corporation. The indemnification provided for in this Section 7.6 shall: (i) not be deemed exclusive of any other rights to which those indemnified may be entitled under these bylaws, agreement or vote of stockholders or disinterested directors or otherwise, both as to action in their official capacities and as to action in another capacity while holding such office, (ii) continue as to a person who has ceased to be a director, and (iii) inure to the benefit of the heirs, executors and administrators of a person who has ceased to be a director. Except as otherwise agreed by the corporation, the corporation’s obligation to provide indemnification under this Section 7.6 shall be offset to the extent of any other source of indemnification or any otherwise applicable insurance coverage under a policy maintained by the corporation or any other person.

Expenses incurred by a director of the corporation in defending a civil or criminal action, suit or proceeding by reason of the fact that he or she is or was a director of the corporation (or was serving at the corporation’s request as a director or officer of another corporation) shall be paid by the corporation in advance of the final disposition of such action, suit or proceeding upon receipt of an undertaking by or on behalf of such director to repay such amount if it shall ultimately be determined that he or she is not entitled to be indemnified by the corporation as authorized by relevant sections of the DGCL. Notwithstanding the foregoing, the corporation shall not be required to advance such expenses to an agent who is a party to an action, suit or proceeding brought by the corporation and approved by a majority of the Board of Directors of the corporation that alleges willful misappropriation of corporate assets by such agent, disclosure of confidential information in violation of such agent’s fiduciary or contractual obligations to the corporation or any other willful and deliberate breach in bad faith of such agent’s duty to the corporation or its stockholders.

The foregoing provisions of this Section 7.6 shall be deemed to be a contract between the corporation and each director who serves in such capacity at any time while this bylaw is in effect, and any repeal or modification thereof shall not affect any rights or obligations then existing with respect to any state of facts then or theretofore existing or any action, suit or proceeding theretofore or thereafter brought based in whole or in part upon any such state of facts.

The Board of Directors in its sole discretion shall have power on behalf of the corporation to indemnify any person, other than a director, made a party to any action, suit or proceeding by reason of the fact that he or she, his or her testator or intestate, is or was an officer or employee of the corporation.

To assure indemnification under this Section 7.6 of all directors, officers and employees who are determined by the corporation or otherwise to be or to have been "fiduciaries" of any employee benefit plan of the corporation that may exist from time to time, Section 145 of the DGCL shall, for the purposes of this Section 7.6, be interpreted as follows: an "other enterprise" shall be deemed to include such an employee benefit plan, including without limitation, any plan of the corporation that is governed by the Act of Congress entitled "Employee Retirement Income Security Act of 1974," as amended from time to time; the corporation shall be deemed to have requested a person to serve the corporation for purposes of Section 145 of the DGCL, as administrator of an employee benefit plan where the performance by such person of his or her duties to the corporation also imposes duties on, or otherwise involves services by, such person to the plan or participants or beneficiaries of the plan; excise taxes assessed on a person with respect to an employee benefit plan pursuant to such Act of Congress shall be deemed "fines."

CERTIFICATE OF INCORPORATION GOVERNS

7.7 **Conflicts with Certificate of Incorporation**. In the event of any conflict between the provisions of the corporation's certificate of incorporation and these bylaws, the provisions of the certificate of incorporation shall govern.

ARTICLE VIII
AMENDMENTS

8.1 These bylaws may be altered, amended or repealed, or new bylaws may be adopted by the stockholders or by the Board of Directors, when such power is conferred upon the Board of Directors by the certificate of incorporation at any regular meeting of the stockholders or of the Board of Directors or at any special meeting of the stockholders or of the Board of Directors if notice of such alteration, amendment, repeal or adoption of new bylaws be contained in the notice of such special meeting. If the power to adopt, amend or repeal bylaws is conferred upon the Board of Directors by the certificate of incorporation, it shall not divest or limit the power of the stockholders to adopt, amend or repeal bylaws.

ARTICLE IX
LOANS TO OFFICERS

9.1 The corporation may lend money to, or guarantee any obligation of or otherwise assist any officer or other employee of the corporation or of its subsidiaries, including any officer or employee who is a director of the corporation or its subsidiaries, whenever, in the judgment of the Board of Directors, such loan, guarantee or assistance may reasonably be expected to benefit the corporation. The loan, guarantee or other assistance may be with or without interest and may be unsecured or secured in such manner as the Board of Directors shall approve, including, without limitation, a pledge of shares of stock of the corporation. Nothing in these bylaws shall be deemed to deny, limit or restrict the powers of guaranty or warranty of the corporation at common law or under any statute.

ARTICLE X
RECORDS AND REPORTS

10.1 The application and requirements of Section 1501 of the California General Corporation Law are hereby expressly waived to the fullest extent permitted thereunder.

CERTIFICATE OF SECRETARY OF

REGENXBIO INC.

The undersigned, Sara Berl, hereby certifies that he or she is the duly elected and acting Secretary of REGENXBIO Inc., a Delaware corporation (the "Corporation"), and that the Bylaws attached hereto constitute the Bylaws of said Corporation as duly adopted by Action by Written Consent in Lieu of Organizational Meeting by the Directors on September 16, 2014.

IN WITNESS WHEREOF, the undersigned has hereunto subscribed her name this 16th day of September, 2014.

/s/ Sara Berl
Sara Berl, Secretary

RESTATED
CERTIFICATE OF INCORPORATION

OF

REGENXBIO INC.

(Pursuant to Sections 242 and 245 of
the Delaware General Corporation Law)

REGENXBIO Inc., a corporation organized and existing under and by virtue of the provisions of the Delaware General Corporation Law,

DOES HEREBY CERTIFY:

FIRST: That the name of the corporation is REGENXBIO Inc. and that this corporation was originally incorporated pursuant to the Delaware General Corporation Law on September 16, 2014 under the name REGENXBIO Inc.

SECOND: That the Restated Certificate of Incorporation (the "Restated Certificate of Incorporation") was approved by the holders of the requisite number of shares of this corporation in accordance with Section 228 of the Delaware General Corporation Law.

THIRD: That the Restated Certificate of Incorporation, which restates and integrates and further amends the provisions of this corporation's heretofore existing Restated Certificate of Incorporation, has been duly adopted in accordance with Sections 242 and 245 of the Delaware General Corporation Law.

FOURTH: That the Restated Certificate of Incorporation of this corporation shall be amended and restated to read in full as follows:

ARTICLE I

The name of the corporation is REGENXBIO Inc. (the "Corporation").

ARTICLE II

The address of the registered office of the Corporation in the State of Delaware is 1209 Orange Street, in the City of Wilmington, County of New Castle, Zip Code 19801. The name of its registered agent at such address is The Corporation Trust Company.

ARTICLE III

The purpose of the Corporation is to engage in any lawful act or activity for which a corporation may be organized under the Delaware General Corporation Law.

ARTICLE IV

A. The total number of shares of all classes of stock which the Corporation shall have authority to issue is one hundred ten million (110,000,000), consisting of one hundred million (100,000,000) shares of Common Stock, par value $0.0001 per share (the "Common Stock"), and ten million (10,000,000) shares of Preferred Stock, par value $0.0001 per share (the "Preferred Stock").

B. The board of directors is authorized, without further stockholder approval and subject to any limitations prescribed by law, to provide for the issuance of shares of Preferred Stock in series, and by filing a certificate pursuant to the applicable law of the State of Delaware (such certificate being hereinafter referred to as a "Preferred Stock Designation"), to establish from time to time the number of shares to be included in each such series, and to fix the designation, powers, preferences, and rights of the shares of each such series and any qualifications, limitations or restrictions thereof. The number of authorized shares of Preferred Stock may be increased or decreased (but not below the number of shares thereof then outstanding) by the affirmative vote of the holders of a majority of the Common Stock, without a vote of the holders of the Preferred Stock, or of any series thereof, unless a vote of any such holders is required pursuant to the terms of any Preferred Stock Designation. In case the number of shares of any series shall be so decreased, the shares constituting such decrease shall resume the status that they had prior to the adoption of the resolution originally fixing the number of shares of such series.

C. Each outstanding share of Common Stock shall entitle the holder thereof to one vote on each matter properly submitted to the stockholders of the Corporation for their vote; provided, however, that, except as otherwise required by law, holders of Common Stock shall not be entitled to vote on any amendment to this Restated Certificate of Incorporation (including any Preferred Stock Designation) that relates solely to the terms of one or more outstanding series of Preferred Stock if the holders of such affected series are entitled, either separately or together as a class with the holders of one or more other such series, to vote thereon pursuant to this Restated Certificate of Incorporation (including any Preferred Stock Designation).

ARTICLE V

The following provisions are inserted for the management of the business and the conduct of the affairs of the Corporation, and for further definition, limitation and regulation of the powers of the Corporation and of its directors and stockholders:

A. The business and affairs of the Corporation shall be managed by or under the direction of the board of directors. In addition to the powers and authority expressly conferred upon them by statute or by this Restated Certificate of Incorporation or the bylaws of the Corporation, the directors are hereby empowered to exercise all such powers and do all such acts and things as may be exercised or done by the Corporation.

B. The directors of the Corporation need not be elected by written ballot unless the bylaws so provide.

C. Any action required or permitted to be taken by the stockholders of the Corporation must be effected at a duly called annual or special meeting of stockholders of the Corporation and may not be effected by any consent in writing by such stockholders.

D. Unless otherwise required by law, special meetings of stockholders of the Corporation may be called only by the Chairman of the board of directors or the Chief Executive Officer (or if there is no Chief Executive Officer, the President) or by the board of directors acting pursuant to a resolution adopted by a majority of the Whole Board. For purposes of this Restated Certificate of Incorporation, the term "Whole Board" shall mean the total number of authorized directors whether or not there exist any vacancies in previously authorized directorships.

E. Unless the Corporation consents in writing to the selection of an alternative forum, the Court of Chancery in the State of Delaware shall be the sole and exclusive forum for (i) any derivative action or proceeding brought on behalf of the Corporation, (ii) any action asserting a claim of breach of fiduciary duty owed by any director, officer or other employee of the Corporation to the Corporation or the Corporation's stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law or the Corporation's certificate of incorporation or bylaws or (iv) any action asserting a claim governed by the internal affairs doctrine.

ARTICLE VI

A. Subject to the rights of the holders of any series of Preferred Stock to elect additional directors under specified circumstances, the number of directors of the Corporation shall be fixed from time to time exclusively by the board of directors pursuant to a resolution adopted by a majority of the Whole Board and may not be fixed by any other person(s).

B. The board of directors, other than those who may be elected by the holders of any series of Preferred Stock under specified circumstances, shall be divided into three classes: Class I, Class II, and Class III. Such classes shall be as nearly equal in number of directors as reasonably possible. Each director shall serve for a term ending on the third annual meeting of stockholders following the annual meeting of stockholders at which such director was elected; provided, however, that the directors first elected or appointed to Class I shall serve for a term ending on the Corporation's first annual meeting of stockholders following the effectiveness of this Restated Certificate of Incorporation, the directors first elected or appointed to Class II shall serve for a term ending on the Corporation's second annual meeting of stockholders following the effectiveness of this Restated Certificate of Incorporation, and the directors first elected or appointed to Class III shall serve for a term ending on the Corporation's third annual meeting of stockholders following the effectiveness of this Restated Certificate of Incorporation. The board of directors is authorized to assign members of the board of directors already in office to such classes as it may determine at the time the classification of the board of directors becomes effective. The foregoing notwithstanding, each director shall serve until such director's successor shall have been duly elected and qualified, or until such director's prior death, resignation, retirement, disqualification or other removal.

C. At each annual election, directors chosen to succeed those whose terms then expire shall be of the same class as the directors they succeed unless, by reason of any intervening changes in the authorized number of directors, the board of directors shall designate one or more directorships whose term then expires as directorships of another class in order more nearly to achieve equality of number of directors among the classes.

D. Notwithstanding the rule that the three classes shall be as nearly equal in number of directors as reasonably possible, in the event of any change in the authorized number of directors, each director then continuing to serve as such shall nevertheless continue as a director of the class of which such director is a member until the expiration of such director's current term, or such director's prior death, resignation, retirement, disqualification or other removal. If any newly created directorship may, consistently with the rule that the three classes shall be as nearly equal in number of directors as reasonably possible, be allocated to more than one class, the board of directors shall allocate it to that of the available class whose term of office is due to expire at the earliest date following such allocation.

E. Subject to the rights of the holders of any series of Preferred Stock then outstanding, newly created directorships resulting from any increase in the authorized number of directors or any vacancies in the board of directors resulting from death, resignation, retirement, disqualification, removal from office or other cause shall, unless otherwise required by law or by resolution of the board of directors, be filled only by a majority vote of the directors then in office, though less than a quorum (and not by stockholders), and directors so chosen shall hold office for a term expiring at the annual meeting of stockholders at which the term of office of the class to which they have been chosen expires or until such director's successor shall have been duly elected and qualified. No decrease in the authorized number of directors shall shorten the term of any incumbent director.

F. Advance notice of stockholder nominations for the election of directors and of business to be brought by stockholders before any meeting of the stockholders of the Corporation shall be given in the manner provided in the bylaws of the Corporation.

G. Subject to the rights of the holders of any series of Preferred Stock then outstanding, any director, or the entire board of directors, may be removed from office at any time, but only for cause and only by the affirmative vote of the holders of at least two-thirds of the voting power of all of the then-outstanding shares of capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class.

ARTICLE VII

In furtherance and not in limitation of the powers conferred by statute, the board of directors is expressly empowered to adopt, amend or repeal bylaws of the Corporation. Any adoption, amendment or repeal of the bylaws of the Corporation by the board of directors shall require the approval of a majority of the Whole Board. The stockholders shall also have the power to adopt, amend or repeal the bylaws of the Corporation as prescribed by law; provided, however, that, in addition to any vote of the holders of any class or series of stock of the Corporation required by law or by this Restated Certificate of Incorporation (including any Preferred Stock Designation), the affirmative vote of the holders of at least two-thirds of the voting power of all of the then-outstanding shares of the capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class, shall be required to adopt, amend or repeal any provision of the bylaws of the Corporation.

ARTICLE VIII

A director of the Corporation shall not be personally liable to the Corporation or its stockholders for monetary damages for breach of fiduciary duty as a director, except for liability (i) for any breach of the director's duty of loyalty to the Corporation or its stockholders, (ii) for

acts or omissions not in good faith or which involve intentional misconduct or a knowing violation of law, (iii) under Section 174 of the Delaware General Corporation Law, or (iv) for any transaction from which the director derived any improper personal benefit. If the Delaware General Corporation Law is amended after approval by the stockholders of this Article VIII to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of a director of the Corporation shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

The Corporation may indemnify to the fullest extent permitted by law any person made or threatened to be made a party to an action or proceeding, whether criminal, civil, administrative or investigative, by reason of the fact that he, she, his or her testator or intestate is or was a director, officer, employee or agent at the request of the Corporation or any predecessor to the Corporation or serves or served at any other enterprise as a director, officer, employee or agent at the request of the Corporation or any predecessor to the Corporation.

Any repeal or modification of the foregoing provisions of this Article VIII by the stockholders of the Corporation shall not adversely affect any right or protection of a director of the Corporation existing at the time of, or increase the liability of any director of the Corporation with respect to any acts or omissions of such director occurring prior to, such repeal or modification.

ARTICLE IX

The Corporation reserves the right to amend or repeal any provision contained in this Restated Certificate of Incorporation in the manner prescribed by the laws of the State of Delaware and all rights conferred upon stockholders are granted subject to this reservation; provided, however, that, notwithstanding any other provision of this Restated Certificate of Incorporation or any provision of law that might otherwise permit a lesser vote or no vote, but in addition to any vote of the holders of any class or series of the stock of this Corporation required by law or by this Restated Certificate of Incorporation (including any Preferred Stock Designation), the affirmative vote of the holders of a majority of the voting power of all of the then-outstanding shares of the capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class, shall be required to amend or repeal the provisions of this Restated Certificate of Incorporation; provided, however, that any amendment or repeal of Sections C or D or E of Article V, or any provision of Article VI, Article VII, Article VIII or this Article IX shall require the affirmative vote of the holders of at least two-thirds of the voting power of all of the then-outstanding shares of the capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class.

* * * *

IN WITNESS WHEREOF, this Restated Certificate of Incorporation has been executed by a duly authorized officer of the Corporation this ____ day of _____, 2015.

Kenneth T. Mills
President and Chief Executive Officer

SIGNATURE PAGE TO RESTATED CERTIFICATE OF INCORPORATION OF REGENXBIO INC.

AMENDED AND RESTATED

BYLAWS OF

REGENXBIO INC.

A DELAWARE CORPORATION

EFFECTIVE: _____, 2015

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ARTICLE I

OFFICES AND RECORDS

Section 1.1 Delaware Office. The registered office of the Corporation in the State of Delaware shall be located in the City of Wilmington, County of New Castle.

Section 1.2 Other Offices. The Corporation may have such other offices, either within or without the State of Delaware, as the Board of Directors may designate or as the business of the Corporation may from time to time require.

Section 1.3 Books and Records. The books and records of the Corporation may be kept at the Corporation's headquarters in Rockville, Maryland or at such other locations outside the State of Delaware as may from time to time be designated by the Board of Directors.

ARTICLE II

STOCKHOLDERS

Section 2.1 Annual Meeting. The annual meeting of the stockholders of the Corporation shall be held at such date, place and/or time as may be fixed by resolution of the Board of Directors.

Section 2.2 Special Meeting. Special meetings of stockholders of the Corporation may be called only by the Chairman of the Board or the President or by the Board of Directors acting pursuant to a resolution adopted by a majority of the Whole Board. For purposes of these Amended and Restated Bylaws, the term "Whole Board" shall mean the total number of authorized directors whether or not there exist any vacancies in previously authorized directorships.

Section 2.3 Place of Meeting. The Board of Directors may designate the place of meeting for any meeting of the stockholders or the means of remote communications by which any meeting shall be held. If no designation is made by the Board of Directors, the place of meeting shall be the principal office of the Corporation.

Section 2.4 Notice of Meeting. Except as otherwise required by law, written, printed or electronic notice stating the place, if any, date and time of the meeting, the means of remote communications, if any, by which the stockholders and proxy holders may be deemed to be present in person and vote at such meeting, and in the case of a special meeting, the purposes for which the meeting is called shall be prepared and delivered by the Corporation not less than ten (10) days nor more than sixty (60) days before the date of the meeting, either personally, by mail, or in the case of stockholders who have consented to such delivery, by electronic transmission (as such term is defined in the Delaware General Corporation Law), to each stockholder of record entitled to vote at such meeting. If mailed, such notice shall be deemed to be delivered when deposited in the U.S. mail with postage thereon prepaid, addressed to the stockholder at his address as it appears on the stock transfer books of the Corporation. Notice given by electronic transmission shall be effective (A) if by facsimile, when faxed to a number where the stockholder has consented to receive notice; (B) if by electronic mail, when mailed

electronically to an electronic mail address at which the stockholder has consented to receive such notice; (C) if by posting on an electronic network together with a separate notice of such posting, upon the later to occur of (1) the posting or (2) the giving of separate notice of the posting; or (D) if by other form of electronic communication, when directed to the stockholder in the manner consented to by the stockholder. Meetings may be held without notice if all stockholders entitled to vote are present (except as otherwise provided by law), or if notice is waived by those not present. Any previously scheduled meeting of the stockholders may be postponed and (unless the Corporation's Amended and Restated Certificate of Incorporation (the "Certificate of Incorporation") otherwise provides) any special meeting of the stockholders may be cancelled, by resolution of the Board of Directors upon public notice given prior to the time previously scheduled for such meeting of stockholders.

Section 2.5 Quorum and Adjournment. Except as otherwise provided by law or by the Certificate of Incorporation, the holders of a majority of the voting power of the outstanding shares of the Corporation entitled to vote generally in the election of directors (the "Voting Stock"), represented in person or by proxy, shall constitute a quorum at a meeting of stockholders, except that when specified business is to be voted on by a class or series voting separately as a class or series, the holders of a majority of the voting power of the shares of such class or series shall constitute a quorum for the transaction of such business for the purposes of taking action on such business. If a quorum shall fail to attend any meeting, the chairman of the meeting may adjourn the meeting to another place, if any, date or time. No notice of an adjourned meeting need be given if the time, place, if any, and the means of remote communication, if any, by which stockholders and proxy holders may be deemed to be present and vote at such adjourned meeting are announced at the meeting at which the adjournment is taken; provided such adjournment is for not more than thirty (30) days and further provided that no new record date is fixed for the adjourned meeting.

Section 2.6 Proxies. At all meetings of stockholders, a stockholder may vote by proxy executed in writing by the stockholder or as may be permitted by law, or by his duly authorized attorney-in-fact. Such proxy must be filed with the Secretary of the Corporation or his representative, or otherwise delivered telephonically or electronically as set forth in the applicable proxy statement, at or before the time of the meeting.

Section 2.7 Notice of Stockholder Business and Nominations.

A. Nominations of persons for election to the Board of Directors and the proposal of business to be transacted by the stockholders may be made at an annual meeting of stockholders (1) pursuant to the Corporation's notice with respect to such meeting, (2) by or at the direction of the Board of Directors or (3) by any stockholder of record of the Corporation who was a stockholder of record at the time of the giving of the notice provided for in the following paragraph, who is entitled to vote at the meeting and who has complied with the notice procedures set forth in this Section 2.7.

B. For nominations or other business to be properly brought before an annual meeting by a stockholder pursuant to paragraph (A)(3) of this Section 2.7, (1) the stockholder must have given timely notice thereof in writing to the Secretary of the Corporation, (2) such business must be a proper matter for stockholder action under the Delaware General

Corporation Law, (3) if the stockholder, or the beneficial owner on whose behalf any such proposal or nomination is made, has provided the Corporation with a Solicitation Notice, as that term is defined in subclause (c)(iii) of this paragraph, such stockholder or beneficial owner must, in the case of a proposal, have delivered prior to the meeting a proxy statement and form of proxy to holders of at least the percentage of the Corporation's voting shares required under applicable law to carry any such proposal, or, in the case of a nomination or nominations, have delivered prior to the meeting a proxy statement and form of proxy to holders of a percentage of the Corporation's voting shares reasonably believed by such stockholder or beneficial holder to be sufficient to elect the nominee or nominees proposed to be nominated by such stockholder, and must, in either case, have included in such materials the Solicitation Notice and (4) if no Solicitation Notice relating thereto has been timely provided pursuant to this section, the stockholder or beneficial owner proposing such business or nomination must not have solicited a number of proxies sufficient to have required the delivery of such a Solicitation Notice under this section. To be timely, a stockholder's notice shall be delivered to the Secretary at the principal executive offices of the Corporation not less than forty-five (45) or more than seventy-five (75) days prior to the first anniversary (the "Anniversary") of the date on which the Corporation first mailed its proxy materials for the preceding year's annual meeting of stockholders; provided, however, that if no proxy materials were mailed by the Corporation in connection with the preceding year's annual meeting, or if the date of the annual meeting is advanced more than thirty (30) days prior to or delayed by more than thirty (30) days after the anniversary of the preceding year's annual meeting, notice by the stockholder to be timely must be so delivered not later than the close of business on the later of (x) the 90th day prior to such annual meeting or (y) the 10th day following the day on which public announcement of the date of such meeting is first made. Such stockholder's notice shall set forth (a) as to each person whom the stockholder proposes to nominate for election or reelection as a director all information relating to such person as would be required to be disclosed in solicitations of proxies for the election of such nominees as directors pursuant to Regulation 14A under the Securities Exchange Act of 1934, as amended (the "Exchange Act"), and such person's written consent to serve as a director if elected; (b) as to any other business that the stockholder proposes to bring before the meeting, a brief description of such business, the reasons for conducting such business at the meeting and any material interest in such business of such stockholder and the beneficial owner, if any, on whose behalf the proposal is made; and (c) as to the stockholder giving the notice and the beneficial owner, if any, on whose behalf the nomination or proposal is made (i) the name and address of such stockholder, as they appear on the Corporation's books, and of such beneficial owner, (ii) the class and number of shares of the Corporation that are owned beneficially and of record by such stockholder and such beneficial owner, and (iii) whether either such stockholder or beneficial owner intends to deliver a proxy statement and form of proxy to holders of, in the case of a proposal, at least the percentage of the Corporation's voting shares required under applicable law to carry the proposal or, in the case of a nomination or nominations, a sufficient number of holders of the Corporation's voting shares to elect such nominee or nominees (an affirmative statement of such intent, a "Solicitation Notice").

C. Notwithstanding anything in the second sentence of paragraph (B) of this Section 2.7 to the contrary, in the event that the number of directors to be elected to the Board of Directors is increased and there is no public announcement naming all of the nominees for director or specifying the size of the increased Board of Directors made by the Corporation at least fifty-five (55) days prior to the Anniversary, a stockholder's notice required by this Bylaw

shall also be considered timely, but only with respect to nominees for any new positions created by such increase, if it shall be delivered to the Secretary at the principal executive offices of the Corporation not later than the close of business on the 10th day following the day on which such public announcement is first made by the Corporation.

D. Only persons nominated in accordance with the procedures set forth in this Section 2.7 shall be eligible to serve as directors and only such business shall be conducted at an annual meeting of stockholders as shall have been brought before the meeting in accordance with the procedures set forth in this Section 2.7. The chair of the meeting shall have the power and the duty to determine whether a nomination or any business proposed to be brought before the meeting has been made in accordance with the procedures set forth in these Bylaws and, if any proposed nomination or business is not in compliance with these Bylaws, to declare that such defective proposed business or nomination shall not be presented for stockholder action at the meeting and shall be disregarded.

E. Only such business shall be conducted at a special meeting of stockholders as shall have been brought before the meeting pursuant to the Corporation's notice of meeting. Nominations of persons for election to the Board of Directors may be made at a special meeting of stockholders at which directors are to be elected pursuant to the Corporation's notice of meeting (1) by or at the direction of the Board of Directors or (2) by any stockholder of record of the Corporation who is a stockholder of record at the time of giving of notice provided for in this paragraph, who shall be entitled to vote at the meeting and who complies with the notice procedures set forth in this Section 2.7. Nominations by stockholders of persons for election to the Board of Directors may be made at such a special meeting of stockholders if the stockholder's notice required by paragraph (B) of this Section 2.7 shall be delivered to the Secretary at the principal executive offices of the Corporation not later than the close of business on the later of the 90th day prior to such special meeting or the 10th day following the day on which public announcement is first made of the date of the special meeting and of the nominees proposed by the Board of Directors to be elected at such meeting.

F. For purposes of this Section 2.7, "public announcement" shall mean disclosure in a press release reported by the Dow Jones News Service, Associated Press or a comparable national news service or in a document publicly filed by the Corporation with the Securities and Exchange Commission pursuant to Section 13, 14 or 15(d) of the Exchange Act.

G. Notwithstanding the foregoing provisions of this Section 2.7, a stockholder shall also comply with all applicable requirements of the Exchange Act and the rules and regulations thereunder with respect to matters set forth in this Section 2.7. Nothing in this Section 2.7 shall be deemed to affect any rights of stockholders to request inclusion of proposals in the Corporation's proxy statement pursuant to Rule 14a-8 under the Exchange Act.

Section 2.8 Procedure for Election of Directors. Election of directors at all meetings of the stockholders at which directors are to be elected shall be by written ballot, and, except as otherwise set forth in the Certificate of Incorporation with respect to the right of the holders of any series of Preferred Stock or any other series or class of stock to elect additional directors under specified circumstances, a plurality of the votes cast thereat shall elect directors. Except as otherwise provided by law, the Certificate of Incorporation or these Bylaws, all

matters other than the election of directors submitted to the stockholders at any meeting shall be decided by the affirmative vote of a majority of the voting power of the outstanding Voting Stock present in person or represented by proxy at the meeting and entitled to vote thereon.

Section 2.9 Inspectors of Elections. The Board of Directors by resolution may, and to the extent required by law, shall appoint one or more inspectors, which inspector or inspectors may include individuals who serve the Corporation in other capacities, including, without limitation, as officers, employees, agents or representatives of the Corporation, to act at the meeting and make a written report thereof. One or more persons may be designated as alternate inspectors to replace any inspector who fails to act. If no inspector or alternate has been appointed to act, or if all inspectors or alternates who have been appointed are unable to act, at a meeting of stockholders, the chairman of the meeting may, and to the extent required by law, shall appoint one or more inspectors to act at the meeting. Each inspector, before discharging his or her duties, shall take and sign an oath faithfully to execute the duties of inspector with strict impartiality and according to the best of his or her ability. The inspectors shall have the duties prescribed by the Delaware General Corporation Law.

Section 2.10 Conduct of Meetings.

A. The President and Chief Executive Officer shall preside at all meetings of the stockholders. In the absence of the President and Chief Executive Officer, the Chairman of the Board shall preside at a meeting of the stockholders. In the absence of both the President and Chief Executive Officer and the Chairman of the Board, the Secretary shall preside at a meeting of the stockholders. In the anticipated absence of all officers designated to preside over the meetings of stockholders, the Board of Directors may designate an individual to preside over a meeting of the stockholders.

B. The chairman of the meeting shall fix and announce at the meeting the date and time of the opening and the closing of the polls for each matter upon which the stockholders will vote at a meeting. The chairman shall have the power to adjourn the meeting to another place, if any, date and time.

C. The Board of Directors may, to the extent not prohibited by law, adopt by resolution such rules and regulations for the conduct of the meeting of stockholders as it shall deem appropriate. Except to the extent inconsistent with such rules and regulations as adopted by the Board of Directors, the chairman of any meeting of stockholders shall have the right and authority to prescribe such rules, regulations and procedures and to do all such acts as, in the judgment of such chairman, are appropriate for the proper conduct of the meeting. Such rules, regulations or procedures, whether adopted by the Board of Directors or prescribed by the chairman of the meeting, may to the extent not prohibited by law include, without limitation, the following: (i) the establishment of an agenda or order of business for the meeting; (ii) rules and procedures for maintaining order at the meeting and the safety of those present; (iii) limitations on attendance at or participation in the meeting to stockholders of record of the Corporation, their duly authorized and constituted proxies or such other persons as the chairman of the meeting shall determine; (iv) restrictions on entry to the meeting after the time fixed for the commencement thereof and (v) limitations on the time allotted to questions or comments by participants. Unless, and to the extent, determined by the Board of Directors or the chairman of the meeting, meetings of stockholders shall not be required to be held in accordance with the rules of parliamentary procedure.

Section 2.11 No Consent of Stockholders in Lieu of Meeting. Subject to the rights of the holders of any series of Preferred Stock, any action required or permitted to be taken by the stockholders of the Corporation must be effected at a duly called annual or special meeting of stockholders of the Corporation and may not be effected by any consent in writing by such stockholders.

ARTICLE III

BOARD OF DIRECTORS

Section 3.1 General Powers. The business and affairs of the Corporation shall be managed by or under the direction of the Board of Directors. In addition to the powers and authority expressly conferred upon them by statute or by the Certificate of Incorporation or by these Bylaws, the directors are hereby empowered to exercise all such powers and do all such acts and things as may be exercised or done by the Corporation.

Section 3.2 Number, Tenure and Qualifications. Subject to the rights of the holders of any series of Preferred Stock to elect additional directors under specified circumstances, the number of directors shall be fixed from time to time exclusively by the Board of Directors pursuant to a resolution adopted by a majority of the Whole Board. The directors, other than those who may be elected by the holders of any series of Preferred Stock under specified circumstances, shall be divided into three classes pursuant to the Certificate of Incorporation. At each annual meeting of stockholders, directors elected to succeed those directors whose terms expire shall be elected for a term of office to expire at the third succeeding annual meeting of stockholders after their election. The foregoing notwithstanding, each director shall serve until such director's successor shall have been duly elected and qualified, or until such director's prior death, resignation, retirement, disqualification or other removal. The Board of Directors is authorized to assign members of the Board of Directors already in office to such classes as it may determine at the time the classification of the Board of Directors becomes effective.

Section 3.3 Regular Meetings. The Board of Directors may, by resolution, provide the time and place for the holding of regular meetings of the Board of Directors. A notice of each regular meeting shall not be required.

Section 3.4 Special Meetings. Special meetings of the Board of Directors shall be called at the request of the Chairman of the Board, the Chief Executive Officer or a majority of the Board of Directors. The person or persons authorized to call special meetings of the Board of Directors may fix the place and time of the meetings, and the writing or transmission shall be filed with the minutes of proceedings of the Board of Directors.

Section 3.5 Action By Unanimous Consent of Directors. The Board of Directors may take action without the necessity of a meeting by unanimous consent of directors. Such consent may be in writing or given by electronic transmission, as such term is defined in the Delaware General Corporation Law.

Section 3.6 Notice. Notice of any special meeting shall be given to each director at his business or residence in writing, or by telegram, facsimile transmission, telephone communication or electronic transmission (provided, with respect to electronic transmission, that the director has consented to receive the form of transmission at the address to which it is directed). If mailed, such notice shall be deemed adequately delivered when deposited in the United States mails so addressed, with postage thereon prepaid, at least five (5) days before such meeting. If by telegram, such notice shall be deemed adequately delivered when the telegram is delivered to the telegraph company at least twenty-four (24) hours before such meeting. If by facsimile transmission or other electronic transmission, such notice shall be transmitted at least twenty-four (24) hours before such meeting. If by telephone, the notice shall be given at least twelve (12) hours prior to the time set for the meeting. Neither the business to be transacted at, nor the purpose of, any regular or special meeting of the Board of Directors need be specified in the notice of such meeting, except for amendments to these Bylaws as provided under Section 8.1 of Article VIII hereof. A meeting may be held at any time without notice if all the directors are present (except as otherwise provided by law) or if those not present waive notice of the meeting in writing or by electronic transmission, either before or after such meeting.

Section 3.7 Conference Telephone Meetings. Members of the Board of Directors, or any committee thereof, may participate in a meeting of the Board of Directors or such committee by means of conference telephone or similar communications equipment by means of which all persons participating in the meeting can hear each other, and such participation in a meeting shall constitute presence in person at such meeting.

Section 3.8 Quorum. A whole number of directors equal to at least a majority of the Whole Board shall constitute a quorum for the transaction of business, but if at any meeting of the Board of Directors there shall be less than a quorum present, a majority of the directors present may adjourn the meeting from time to time without further notice. The act of the majority of the directors present at a meeting at which a quorum is present shall be the act of the Board of Directors.

Section 3.9 Vacancies. Subject to the rights of the holders of any series of Preferred Stock then outstanding, newly created directorships resulting from any increase in the authorized number of directors or any vacancies in the Board of Directors resulting from death, resignation, retirement, disqualification, removal from office or other cause shall, unless otherwise provided by law or by resolution of the Board of Directors, be filled only by a majority vote of the directors then in office, though less than a quorum (and not by stockholders), and directors so chosen shall hold office for a term expiring at the annual meeting of stockholders at which the term of office of the class to which they have been chosen expires or until such director's successor has been duly elected and qualified. No decrease in the authorized number of directors shall shorten the term of any incumbent director.

Section 3.10 Committees.

A. The Board of Directors may designate one or more committees, each committee to consist of one or more of the directors of the Corporation. The Board of Directors may designate one or more directors as alternate members of any committee, who may replace any absent or disqualified member at any meeting of the committee. In the absence or disqualification of a member of the committee, the member or members thereof present at any meeting and not disqualified from voting, whether or not he or they constitute a quorum, may unanimously appoint another member of the Board of Directors to act at the meeting in place of any such absent or disqualified member. Any such committee, to the extent permitted by law and to the extent provided in the resolution of the Board of Directors, shall have and may exercise all the powers and authority of the Board of Directors in the management of the business and affairs of the Corporation, and may authorize the seal of the Corporation to be affixed to all papers which may require it; provided, however, that no committee shall have power or authority in reference to the following matters: (1) approving, adopting or recommending to stockholders any action or matter required by law to be submitted to stockholders for approval or (2) adopting, amending or repealing any bylaw.

B. Unless the Board of Directors otherwise provides, each committee designated by the Board of Directors may make, alter and repeal rules for the conduct of its business. In the absence of such rules each committee shall conduct its business in the same manner as the Board of Directors conducts its business pursuant to these Bylaws.

Section 3.11 Removal. Subject to the rights of the holders of any series of Preferred Stock then outstanding, any director, or the entire Board of Directors, may be removed from office at any time, but only for cause and only by the affirmative vote of the holders of at least two-thirds of the voting power of all of the then-outstanding shares of capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class.

ARTICLE IV

OFFICERS

Section 4.1 Elected Officers. The elected officers of the Corporation shall be a Chairman of the Board, a President, a Secretary, a Treasurer, and such other officers as the Board of Directors from time to time may deem proper. The Chairman of the Board shall be chosen from the directors. All officers chosen by the Board of Directors shall each have such powers and duties as generally pertain to their respective offices, subject to the specific provisions of this Article IV. Such officers shall also have powers and duties as from time to time may be conferred by the Board of Directors or by any committee thereof.

Section 4.2 Election and Term of Office. The elected officers of the Corporation shall be elected annually by the Board of Directors at the regular meeting of the Board of Directors held after each annual meeting of the stockholders. If the election of officers shall not be held at such meeting, such election shall be held as soon thereafter as convenient. Subject to Section 4.7 of these Bylaws, each officer shall hold office until his successor shall have been duly elected and shall have qualified or until his death or until he shall resign.

Section 4.3 Chairman of the Board. The Chairman of the Board shall preside at all meetings of the Board of Directors.

Section 4.4 President and Chief Executive Officer. The President and Chief Executive Officer shall be the general manager of the Corporation, subject to the control of the Board of Directors, and as such shall, subject to Section 2.10(A) hereof, preside at all meetings of stockholders, shall have general supervision of the affairs of the Corporation, shall sign or countersign or authorize another officer to sign all certificates, contracts, and other instruments of the Corporation as authorized by the Board of Directors, shall make reports to the Board of Directors and stockholders, and shall perform all such other duties as are incident to such office or are properly required by the Board of Directors. If the Board of Directors creates the office of Chief Executive Officer as a separate office from President, the President shall be the chief operating officer of the corporation and shall be subject to the general supervision, direction, and control of the Chief Executive Officer unless the Board of Directors provides otherwise.

Section 4.5 Secretary. The Secretary shall give, or cause to be given, notice of all meetings of stockholders and directors and all other notices required by law or by these Bylaws, and in case of his absence or refusal or neglect so to do, any such notice may be given by any person thereunto directed by the Chairman of the Board or the President, or by the Board of Directors, upon whose request the meeting is called as provided in these Bylaws. The Secretary shall record all the proceedings of the meetings of the Board of Directors, any committees thereof and the stockholders of the Corporation in a book to be kept for that purpose, and shall perform such other duties as may be assigned to the Secretary by the Board of Directors, the Chairman of the Board or the President. The Secretary shall have custody of the seal of the Corporation and shall affix the same to all instruments requiring it, when authorized by the Board of Directors, the Chairman of the Board or the President, and attest to the same.

Section 4.6 Treasurer. The Treasurer shall have the custody of the corporate funds and securities and shall keep full and accurate receipts and disbursements in books belonging to the Corporation. The Treasurer shall deposit all moneys and other valuables in the name and to the credit of the Corporation in such depositaries as may be designated by the Board of Directors. The Treasurer shall disburse the funds of the Corporation as may be ordered by the Board of Directors the Chairman of the Board, or the President, taking proper vouchers for such disbursements. The Treasurer shall render to the Chairman of the Board, the President and the Board of Directors, whenever requested, an account of all his transactions as Treasurer and of the financial condition of the Corporation. If required by the Board of Directors, the Treasurer shall give the Corporation a bond for the faithful discharge of his duties in such amount and with such surety as the Board of Directors shall prescribe.

Section 4.7 Removal. Any officer elected by the Board of Directors may be removed by the Board of Directors at any time, with or without cause. No elected officer shall have any contractual rights against the Corporation for compensation by virtue of such election beyond the date of the election of his successor, his death, his resignation or his removal, whichever event shall first occur, except as otherwise provided in an employment contract or an employee plan.

Section 4.8 Vacancies. A newly created office and a vacancy in any office because of death, resignation, or removal may be filled by the Board of Directors for the unexpired portion of the term at any meeting of the Board of Directors.

ARTICLE V

STOCK CERTIFICATES AND TRANSFERS

Section 5.1 Stock Certificates and Transfers.

A. Unless the Board of Directors has determined by resolution that some or all of any or all classes or series of stock shall be uncertificated shares, the interest of each stockholder of the Corporation shall be evidenced by certificates for shares of stock in such form as the appropriate officers of the Corporation may from time to time prescribe. The shares of the stock of the Corporation shall be transferred on the books of the Corporation by the holder thereof in person or by his attorney, upon surrender for cancellation of certificates for the same number of shares, with an assignment and power of transfer endorsed thereon or attached thereto, duly executed, and with such proof of the authenticity of the signature as the Corporation or its agents may reasonably require.

B. Every holder of stock represented by certificates shall be entitled to have a certificate signed, countersigned and registered in such manner as the Board of Directors may by resolution prescribe, which resolution may permit all or any of the signatures on such certificates to be in facsimile. In case any officer, transfer agent or registrar who has signed or whose facsimile signature has been placed upon a certificate has ceased to be such officer, transfer agent or registrar before such certificate is issued, it may be issued by the Corporation with the same effect as if he were such officer, transfer agent or registrar at the date of issue.

ARTICLE VI

INDEMNIFICATION

Section 6.1 Right to Indemnification. Each person who was or is made a party or is threatened to be made a party to or is otherwise involved in any action, suit or proceeding, whether civil, criminal, administrative or investigative (hereinafter a “proceeding”), by reason of the fact that he or she is or was a director or officer of the Corporation or, while a director or officer of the Corporation, is or was serving at the request of the Corporation as a director, officer, employee, trustee or agent of another corporation or of a partnership, joint venture, trust or other enterprise, including service with respect to an employee benefit plan (hereinafter an “indemnitee”), where the basis of such proceeding is alleged action in an official capacity as a director, officer, employee, trustee or agent or in any other capacity while serving as a director, officer, trustee or agent, shall be indemnified and held harmless by the Corporation to the fullest extent authorized by the Delaware General Corporation Law, as the same exists or may hereafter be amended (but, in the case of any such amendment, only to the extent that such amendment

permits the Corporation to provide broader indemnification rights than permitted prior thereto), against all expense, liability and loss (including attorneys' fees, judgments, fines, ERISA excise taxes or penalties and amounts paid in settlement) reasonably incurred or suffered by such indemnitee in connection therewith and such indemnification shall continue as to an indemnitee who has ceased to be a director, officer, employee, trustee or agent and shall inure to the benefit of the indemnitee's heirs, executors and administrators; provided, however, that, except as provided in Section 6.3 hereof with respect to proceedings to enforce rights to indemnification, the Corporation shall indemnify any such indemnitee in connection with a proceeding (or part thereof) initiated by such indemnitee only if such proceeding (or part thereof) was authorized by the Board of Directors of the Corporation.

Section 6.2 Right to Advancement of Expenses. The right to indemnification conferred in Section 6.1 shall include the right to be paid by the Corporation the expenses (including attorney's fees) incurred in defending any proceeding for which such right to indemnification is applicable in advance of its final disposition (hereinafter an "advancement of expenses"); provided, however, that, if the Delaware General Corporation Law requires, an advancement of expenses incurred by an indemnitee in his or her capacity as a director or officer (and not in any other capacity in which service was or is rendered by such indemnitee, including, without limitation, service to an employee benefit plan) shall be made only upon delivery to the Corporation of an undertaking (hereinafter an "undertaking"), by or on behalf of such indemnitee, to repay all amounts so advanced if it shall ultimately be determined by final judicial decision from which there is no further right to appeal (hereinafter a "final adjudication") that such indemnitee is not entitled to be indemnified for such expenses under this Section or otherwise.

Section 6.3 Right of Indemnitee to Bring Suit. The rights to indemnification and to the advancement of expenses conferred in Section 6.1 and Section 6.2, respectively, shall be contract rights. If a claim under Section 6.1 or Section 6.2 is not paid in full by the Corporation within sixty days after a written claim has been received by the Corporation, except in the case of a claim for an advancement of expenses, in which case the applicable period shall be twenty days, the indemnitee may at any time thereafter bring suit against the Corporation to recover the unpaid amount of the claim. If successful in whole or in part in any such suit, or in a suit brought by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the indemnitee shall be entitled to be paid also the expense of prosecuting or defending such suit. In (A) any suit brought by the indemnitee to enforce a right to indemnification hereunder (but not in a suit brought by the indemnitee to enforce a right to an advancement of expenses) it shall be a defense that, and (B) in any suit by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking the Corporation shall be entitled to recover such expenses upon a final adjudication that, the indemnitee has not met any applicable standard for indemnification set forth in the Delaware General Corporation Law. Neither the failure of the Corporation (including its Board of Directors, independent legal counsel, or its stockholders) to have made a determination prior to the commencement of such suit that indemnification of the indemnitee is proper in the circumstances because the indemnitee has met the applicable standard of conduct set forth in the Delaware General Corporation Law, nor an actual determination by the Corporation (including its Board of Directors, independent legal counsel, or its stockholders) that the indemnitee has not met such applicable standard of conduct, shall create a presumption that the indemnitee has not met the applicable standard of

conduct or, in the case of such a suit brought by the indemnitee, be a defense to such suit. In any suit brought by the indemnitee to enforce a right to indemnification or to an advancement of expenses hereunder, or by the Corporation to recover an advancement of expenses pursuant to the terms of an undertaking, the burden of proving that the indemnitee is not entitled to be indemnified, or to such advancement of expenses, under this Section or otherwise shall be on the Corporation.

Section 6.4 Non-Exclusivity of Rights. The rights to indemnification and to the advancement of expenses conferred in this Article VI shall not be exclusive of any other right which any person may have or hereafter acquire under the Certificate of Incorporation, these Amended and Restated Bylaws, or any statute, agreement, vote of stockholders or disinterested directors or otherwise.

Section 6.5 Insurance. The Corporation may maintain insurance, at its expense, to protect itself and any director, officer, employee or agent of the Corporation or another corporation, partnership, joint venture, trust or other enterprise against any expense, liability or loss, whether or not the Corporation would have the power to indemnify such person against such expense, liability or loss under the Delaware General Corporation Law.

Section 6.6 Amendment of Rights. Any amendment, alteration or repeal of this Article VI that adversely affects any right of an indemnitee or its successors shall be prospective only and shall not limit or eliminate any such right with respect to any proceeding involving any occurrence or alleged occurrence of any action or omission to act that took place prior to such amendment or repeal.

Section 6.7 Indemnification of Employees and Agents of the Corporation. The Corporation may, to the extent authorized from time to time by the Board of Directors, grant rights to indemnification, and to the advancement of expenses, to any employee or agent of the Corporation to the fullest extent of the provisions of this Section with respect to the indemnification and advancement of expenses of directors and officers of the Corporation.

ARTICLE VII

MISCELLANEOUS PROVISIONS

Section 7.1 Fiscal Year. The fiscal year of the Corporation shall begin on the first day of January and end on the thirty-first day of December of each year.

Section 7.2 Dividends. The Board of Directors may from time to time declare, and the Corporation may pay, dividends on its outstanding shares in the manner and upon the terms and conditions provided by law and its Certificate of Incorporation.

Section 7.3 Seal. The corporate seal shall have inscribed the name of the Corporation thereon and shall be in such form as may be approved from time to time by the Board of Directors.

Section 7.4 Waiver of Notice. Whenever any notice is required to be given to any stockholder or director of the Corporation under the provisions of the Delaware General Corporation Law, the Certificate of Incorporation or the Bylaws, a waiver thereof in writing, signed by the person or persons entitled to such notice, or a waiver by electronic transmission, whether before or after the time of the event for which notice is to be given, shall be deemed equivalent to the giving of such notice. Neither the business to be transacted at, nor the purpose of, any annual or special meeting of the stockholders or the Board of Directors need be specified in any waiver of notice of such meeting. Attendance of a person at a meeting shall constitute a waiver of notice of such meeting, except when the person attends a meeting for the express purpose of objecting at the beginning of the meeting to the transaction of any business because the meeting is not lawfully called or convened.

Section 7.5 Audits. The accounts, books and records of the Corporation shall be audited upon the conclusion of each fiscal year by an independent certified public accountant selected by the Board of Directors, and it shall be the duty of the Board of Directors to cause such audit to be made annually.

Section 7.6 Resignations. Any director or any officer, whether elected or appointed, may resign at any time by serving written notice of such resignation on the Chairman of the Board, the Chief Executive Officer or the Secretary, or by submitting such resignation by electronic transmission (as such term is defined in the Delaware General Corporation Law), and such resignation shall be deemed to be effective as of the close of business on the date said notice is received by the Chairman of the Board, the Chief Executive Officer, or the Secretary or at such later date as is stated therein. No formal action shall be required of the Board of Directors or the stockholders to make any such resignation effective.

Section 7.7 Contracts. Except as otherwise required by law, the Certificate of Incorporation or these Bylaws, any contracts or other instruments may be executed and delivered in the name and on the behalf of the Corporation by such officer or officers of the Corporation as the Board of Directors may from time to time direct. Such authority may be general or confined to specific instances as the Board of Directors may determine. The Chairman of the Board, the Chief Executive Officer, the President or any Vice President may execute bonds, contracts, deeds, leases and other instruments to be made or executed for or on behalf of the Corporation. Subject to any restrictions imposed by the Board of Directors or the Chairman of the Board, the Chief Executive Officer, the President or any Vice President of the Corporation may delegate contractual powers to others under his jurisdiction, it being understood, however, that any such delegation of power shall not relieve such officer of responsibility with respect to the exercise of such delegated power.

Section 7.8 Proxies. Unless otherwise provided by resolution adopted by the Board of Directors, the Chairman of the Board, the Chief Executive Officer, the President or any Vice President may from time to time appoint any attorney or attorneys or agent or agents of the Corporation, in the name and on behalf of the Corporation, to cast the votes which the Corporation may be entitled to cast as the holder of stock or other securities in any other corporation or other entity, any of whose stock or other securities may be held by the Corporation, at meetings of the holders of the stock and other securities of such other corporation or other entity, or to consent in writing, in the name of the Corporation as such holder, to any action by such other corporation or other entity, and may instruct the person or persons so appointed as to the manner of casting such votes or giving such consent, and may execute or cause to be executed in the name and on behalf of the Corporation and under its corporate seal or otherwise, all such written proxies or other instruments as he may deem necessary or proper in the premises.

ARTICLE VIII

AMENDMENTS

Section 8.1 Amendments. Subject to the provisions of the Certificate of Incorporation (including the rights of the holders of any series of Preferred Stock then outstanding), these Bylaws may be adopted, amended or repealed at any meeting of the Board of Directors by a resolution adopted by a majority of the Whole Board, provided notice of the proposed change was given in the notice of the meeting in a notice given no less than twenty-four (24) hours prior to the meeting. Subject to the provisions of the Certificate of Incorporation (including the rights of the holders of any series of Preferred Stock then outstanding), the stockholders shall also have power to adopt, amend or repeal these Bylaws, provided that notice of the proposed change was given in the notice of the meeting and provided further that, in addition to any vote of the holders of any class or series of stock of the Corporation required by law or by the Certificate of Incorporation (including the rights of the holders of any series of Preferred Stock then outstanding), the affirmative vote of the holders of at least two-thirds of the voting power of all of the then-outstanding shares of the capital stock of the Corporation entitled to vote generally in the election of directors, voting together as a single class, shall be required to adopt, amend or repeal any provision of these Bylaws.

CERTIFICATE OF SECRETARY OF

REGENXBIO INC.

The undersigned, Sara Garon Berl, hereby certifies that she is the duly elected and acting Secretary of REGENXBIO Inc., a Delaware corporation (the "Corporation"), and that the Bylaws attached hereto constitute the Bylaws of said Corporation as duly adopted by the Directors on _____, 2015.

IN WITNESS WHEREOF, the undersigned has hereunto subscribed her name this _____ day of _____, 2015.

_____

Sara Garon Berl, Secretary

ZQ|CERT#|COY|CLS|RGSTRY|ACCT#|TRANSTYPE|RUN#|TRANS#

COMMON STOCK

PAR VALUE $0.0001

COMMON STOCK

THIS CERTIFICATE IS TRANSFERABLE IN CANTON, MA; JERSEY CITY, NJ AND COLLEGE STATION, TX

REGENXBIO

Certificate Number

ZQ00000000

Shares

REGENXBIO INC.

INCORPORATED UNDER THE LAWS OF THE STATE OF DELAWARE

THIS CERTIFIES THAT

MR. SAMPLE & MRS. SAMPLE & MR. SAMPLE & MRS. SAMPLE

CUSIP 75901B 10 7

SEE REVERSE FOR CERTAIN DEFINITIONS

is the owner of

ZERO HUNDRED THOUSAND ZERO HUNDRED AND ZERO

FULLY-PAID AND NON-ASSESSABLE SHARES OF COMMON STOCK OF

REGENXBIO Inc. (hereinafter called the "Company"), transferable on the books of the Company in person or by duly authorized attorney, upon surrender of this Certificate properly endorsed. This Certificate and the shares represented hereby, are issued and shall be held subject to all of the provisions of the Certificate of Incorporation, as amended, and the By-Laws, as amended, of the Company (copies of which are on file with the Company and with the Transfer Agent), to all of which each holder, by acceptance hereof, assents. This Certificate is not valid unless countersigned and registered by the Transfer Agent and Registrar.

Witness the facsimile seal of the Company and the facsimile signatures of its duly authorized officers.

President

Secretary

SEAL

DATED DD-MMM-YYYY

COUNTERSIGNED AND REGISTERED:
COMPUTERSHARE TRUST COMPANY, N.A.
TRANSFER AGENT AND REGISTRAR,

By _____
AUTHORIZED SIGNATURE

1234567

REGENXBIO

PO BOX 43064, Providence, RI 02940-3064

MR A SAMPLE
DESIGNATION (IF ANY)
ADD 1
ADD 2
ADD 3
ADD 4

CUSIP XXXXXX XX X
Holder ID XXXXXXXXXX
Insurance Value 1,000,000.00
Number of Shares 123456
DTC 12345678 1234567890123456

Certificate Numbers	Num/No.	Denom.	Total
1234567890123456789	1	1	1
1234567890123456789	2	2	2
1234567890123456789	3	3	3
1234567890123456789	4	4	4
1234567890123456789	5	5	5
1234567890123456789	6	6	6
1234567890123456789			7

Total Transaction

REGENXBIO INC.

THE COMPANY WILL FURNISH WITHOUT CHARGE TO EACH SHAREHOLDER WHO SO REQUESTS, A SUMMARY OF THE POWERS, DESIGNATIONS, PREFERENCES AND RELATIVE, PARTICIPATING, OPTIONAL OR OTHER SPECIAL RIGHTS OF EACH CLASS OF STOCK OF THE COMPANY AND THE QUALIFICATIONS, LIMITATIONS OR RESTRICTIONS OF SUCH PREFERENCES AND RIGHTS, AND THE VARIATIONS IN RIGHTS, PREFERENCES AND LIMITATIONS DETERMINED FOR EACH SERIES, WHICH ARE FIXED BY THE CERTIFICATE OF INCORPORATION OF THE COMPANY, AS AMENDED, AND THE RESOLUTIONS OF THE BOARD OF DIRECTORS OF THE COMPANY, AND THE AUTHORITY OF THE BOARD OF DIRECTORS TO DETERMINE VARIATIONS FOR FUTURE SERIES. SUCH REQUEST MAY BE MADE TO THE OFFICE OF THE SECRETARY OF THE COMPANY OR TO THE TRANSFER AGENT. THE BOARD OF DIRECTORS MAY REQUIRE THE OWNER OF A LOST OR DESTROYED STOCK CERTIFICATE, OR HIS LEGAL REPRESENTATIVES, TO GIVE THE COMPANY A BOND TO INDEMNIFY IT AND ITS TRANSFER AGENTS AND REGISTRARS AGAINST ANY CLAIM THAT MAY BE MADE AGAINST THEM ON ACCOUNT OF THE ALLEGED LOSS OR DESTRUCTION OF ANY SUCH CERTIFICATE.

The following abbreviations, when used in the inscription on the face of this certificate, shall be construed as though they were written out in full according to applicable laws or regulations:

TEN COM	- as tenants in common	UNIF GIFT MIN ACT	-.....(Cust).....Custodian.....(Minor)..... under Uniform Gifts to Minors Act.....(State).....
TEN ENT	- as tenants by the entireties		
JT TEN	- as joint tenants with right of survivorship and not as tenants in common	UNIF TRF MIN ACT	-.....(Cust).....Custodian (until age.....) .....(Minor).....under Uniform Transfers to Minors Act.....(State).....

Additional abbreviations may also be used though not in the above list.

PLEASE INSERT SOCIAL SECURITY OR OTHER IDENTIFYING NUMBER OF ASSIGNEE

For value received, _____ hereby sell, assign and transfer unto

_____

(PLEASE PRINT OR TYPEWRITE NAME AND ADDRESS, INCLUDING POSTAL ZIP CODE, OF ASSIGNEE)

_____

_____

_____ Shares

of the common stock represented by the within Certificate, and do hereby irrevocably constitute and appoint

_____ Attorney

to transfer the said stock on the books of the within-named Company with full power of substitution in the premises.

Dated: _____ 20____

Signature: _____

Signature: _____

Notice: The signature to this assignment must correspond with the name as written upon the face of the certificate, in every particular, without alteration or enlargement, or any change whatever.

Signature(s) Guaranteed: Medallion Guarantee Stamp

THE SIGNATURE(S) SHOULD BE GUARANTEED BY AN ELIGIBLE GUARANTOR INSTITUTION (Banks, Stockbrokers, Savings and Loan Associations and Credit Unions) WITH MEMBERSHIP IN AN APPROVED SIGNATURE GUARANTEE MEDALLION PROGRAM, PURSUANT TO S.E.C. RULE 17Ad-15.

SECURITY INSTRUCTIONS

THIS IS WATERMARKED PAPER. DO NOT ACCEPT WITHOUT NOTING WATERMARK. HOLD TO LIGHT TO VERIFY WATERMARK.

The IRS requires that we report the cost basis of certain shares acquired after January 1, 2011. If your shares were covered by the legislation and you have sold or transferred the shares and requested a specific cost basis calculation method, we have processed as requested. If you did not specify a cost basis calculation method, we have defaulted to the first in, first out (FIFO) method. Please visit our website or consult your tax advisor if you need additional information about cost basis.

If you do not keep in contact with us or do not have any activity in your account for the time periods specified by state law, your property could become subject to state unclaimed property laws and transferred to the appropriate state.

153457

AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT

This AMENDED AND RESTATED INVESTORS' RIGHTS AGREEMENT (the "Agreement") is made as of the 15th day of May, 2015, by and among REGENXBIO Inc., a Delaware corporation (the "Company"), the investors listed on Schedule A hereto (the "Investors"), and the holders of Common Stock (as defined below) listed on Schedule B hereto (the "Common Holders" and together with the Investors, the "Holders").

RECITALS

WHEREAS, certain of the Investors (the "Existing Investors") hold shares of the Company's Series A Preferred Stock, par value $0.0001 per share (the "Series A Preferred Stock"), Series B Preferred Stock, par value $0.0001 per share (the "Series B Preferred Stock"), Series C Preferred Stock, par value $0.0001 per share (the "Series C Preferred Stock"), and/or shares of Common Stock issued upon conversion thereof and possess registration rights, information rights, rights of first offer and other rights pursuant to that certain Investors' Rights Agreement dated as of January 13, 2015 by and among the Company, certain holders of Common Stock (the "Existing Common Holders" and together with the Existing Investors, the "Existing Holders"), par value $0.0001 per share (the "Common Stock"), and such Existing Investors (the "Prior Agreement");

WHEREAS, the Prior Agreement may be amended, and any provision therein waived, with the consent of the Company and the Holders (as such term is defined in the Prior Agreement) of at least fifty-five percent (55%) of the then outstanding capital stock held by the Holders and the consent of two of the Lead Investors (as defined in the Prior Agreement);

WHEREAS, the Existing Holders as holders of at least fifty-five percent (55%) of the then outstanding capital stock held by the Holders (as such term is defined in the Prior Agreement) desire to terminate the Prior Agreement and to accept the rights created pursuant hereto in lieu of the rights granted to them under the Prior Agreement; and

WHEREAS, certain Investors are parties to that certain Series D Preferred Stock Purchase Agreement of even date herewith by and among the Company and certain of the Investors (the "Purchase Agreement"), which provides that as a condition to the closing of the sale of the Series D Preferred Stock, par value $0.0001 per share (the "Series D Preferred Stock" and collectively with the Series A Preferred Stock, the Series B Preferred Stock and Series C Preferred Stock, the "Preferred Stock"), this Agreement must be executed and delivered by such Investors, Existing Holders holding at least fifty-five percent (55%) of the then outstanding capital stock held by the Holders (as such term is defined in the Prior Agreement), and the Company.

NOW, THEREFORE, in consideration of the mutual promises and covenants set forth herein, the Company and the Existing Holders hereby agree that the Prior Agreement shall be superseded and replaced in its entirety by this Agreement, and the parties hereto further agree as follows:

1. Definitions. For purposes of this Agreement:

    (a) The term "1934 Act" means the Securities Exchange Act of 1934, as amended.

(b) The term "Advisory Holder" means a Holder that is a mutual fund, pension fund, pooled investment vehicle or separate account advised by an investment advisor registered under the Investment Advisers Act of 1940, as amended.

(c) The term "Affiliate" means, with respect to any Person, any other Person who or which, directly or indirectly, controls, is controlled by, or is under common control with such specified Person, including, without limitation, any general partner, managing member, limited partner, member, manager, officer or director of such Person and any venture capital fund now or hereafter existing that is controlled by one or more general partners or managing members of, or is under common investment management with, such Person. For purposes of this definition, the term "control" when used with respect to any Person means the power to direct the management or policies of such Person, directly or indirectly, whether through ownership of voting securities, by contract or otherwise, and the terms "controlling" and "controlled" shall have meanings correlative to the foregoing.

(d) The term "API" means ARIAD Pharmaceuticals, Inc.

(e) The term "API Dilution Threshold" shall have the meaning set forth in the Certificate.

(f) The term "Board" means the Company's Board of Directors, as constituted from time to time.

(g) The term "Certificate" means the Company's Restated Certificate of Incorporation, as amended and/or restated from time to time.

(h) The term "Common Stock" means shares of the Company's common stock, par value $0.0001 per share.

(i) The term "Form S-3" means such form under the Securities Act as in effect on the date hereof or any registration form under the Securities Act subsequently adopted by the SEC that permits inclusion or incorporation of substantial information by reference to other documents filed by the Company with the SEC.

(j) The term "Free Writing Prospectus" means a free-writing prospectus, as defined in Rule 405 under the Securities Act ("Rule 405").

(k) The term "GSK" means GlaxoSmithKline LLC.

(l) The term "GSK Dilution Threshold" shall have the meaning set forth in the Certificate.

(m) The term "Incentive Plan" means the Company's 2014 Stock Plan.

(n) The term "Initial Offering" means the Company's first firm commitment underwritten public offering of its Common Stock under the Securities Act.

(o) The term "Investment Advisor" means an investment advisor registered under the Investment Advisers Act of 1940, as amended

(p) The term "Lead Investors" means (i) Vivo Capital Fund VIII, L.P. and its Affiliates (collectively, "Vivo"), (ii) Venrock Healthcare Capital Partners II, L.P. and Venrock Associates VII, L.P. and its Affiliates (collectively, "Venrock"), (iii) Brookside Capital Partners Fund, L.P. ("Brookside"), (iv) BlackRock Health Sciences Trust and its affiliates (collectively, "BlackRock"), (v) Janus Global Life Sciences Fund, (vi) Perceptive Life Sciences Master Fund LTD and its Affiliates (collectively, "Perceptive") and (vii) funds advised by T. Rowe Price Associates, Inc. (collectively "T. Rowe"); for the avoidance of doubt, Venrock, BlackRock, Vivo, Perceptive and T. Rowe shall each be treated as a single Lead Investor.

(q) The term "Lead Observers" means (i) Vivo, (ii) Venrock and (iii) Brookside; for the avoidance of doubt, Venrock and Vivo shall each be treated as a single Lead Observer.

(r) The term "Major Investor" means any Investor that, together with its Affiliates, holds at least 100,000 shares of Series B Preferred Stock, Series C Preferred Stock and/or Series D Preferred Stock (as appropriately adjusted for any stock split, dividend, combination or other recapitalization).

(s) The term "Major Investor's Proportional Share" means (A) the aggregate number of Shares then owned by the applicable Major Investor, divided by (B) the aggregate number of Shares then outstanding, in each case assuming the exercise or conversion of all securities exercisable for or convertible into Shares.

(t) The term "New Securities" means, collectively, equity securities of the Company, whether or not currently authorized, as well as rights, options, or warrants to purchase such equity securities, or securities of any type whatsoever that are, or may become, convertible or exchangeable into or exercisable for such equity securities.

(u) The term "Person" means any individual, corporation, partnership, trust, limited liability company, association or other entity.

(v) The term "Proportional Share" means, (i) with respect to API, (A) the aggregate number of Shares then owned by API, divided by (B) the aggregate number of Shares then outstanding or reserved for issuance under the Incentive Plan, in each case assuming the exercise or conversion of all securities exercisable for or convertible into Shares, including without limitation securities reserved for issuance under the Incentive Plan, and (ii) with respect to GSK, (A) the aggregate number of Shares then owned by GSK, divided by (B) the aggregate number of Shares then outstanding, in each case assuming the exercise or conversion of all securities exercisable for or convertible into Shares.

(w) The terms "register," "registered," and "registration" refer to a registration effected by preparing and filing a registration statement or similar document in compliance with the Securities Act, and the declaration or ordering of effectiveness of such registration statement or document.

(x) The terms "Securities" and "Shares" each means (i) the Common Stock issuable or issued upon conversion of the Preferred Stock; (ii) the Common Stock issued to the Common Holders and (iii) any Common Stock of the Company issued as (or issuable upon the conversion or exercise of any warrant, right or other security that is issued as) a dividend or other distribution with respect to, or in exchange for, or in replacement of, the shares referenced in (i) and (ii) above, excluding in all cases, however, any Securities sold by a Person in a transaction in which his rights under Section 2 of this Agreement are not assigned. In addition, the number of shares of Securities outstanding shall equal the aggregate of the number of shares of Common Stock outstanding that are, and the number of shares of Common Stock issuable pursuant to then exercisable or convertible securities that are, Securities.

(y) The term "Securities Act" means the Securities Act of 1933, as amended.

(z) The term "Rule 144" means Rule 144 under the Securities Act.

(aa) The term "Rule 144(b)(1)(i)" means subsection (b)(1)(i) of Rule 144 under the Securities Act as it applies to Persons who have held shares for more than one (1) year.

(bb) The term "SEC" means the Securities and Exchange Commission.

(cc) The term "University" means The Trustees of the University of Pennsylvania.

2. Registration Rights. The Company covenants and agrees as follows:

2.1 Request for Registration.

(a) Subject to the conditions of this Section 2.1, if the Company shall receive at any time after the earlier of (i) five (5) years after the date of this Agreement or (ii) six (6) months after the effective date of the Initial Offering, a written request from the Holders of at least fifty percent (50%) of the Securities then outstanding (for purposes of this Section 2.1, the "Initiating Holders") that the Company file a registration statement under the Securities Act covering the registration of Securities with an anticipated aggregate offering price of at least $30,000,000, then the Company shall, within twenty (20) days of the receipt thereof, give written notice of such request to all Holders, and subject to the limitations of this Section 2.1, use its commercially reasonable efforts to effect, as soon as practicable, the registration under the Securities Act of all Securities that the Holders request to be registered in a written request received by the Company within twenty (20) days of the mailing of the Company's notice pursuant to this Section 2.1(a).

(b) If the Initiating Holders intend to distribute the Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to this Section 2.1, and the Company shall include such information in the

written notice referred to in Section 2.1(a). In such event the right of any Holder to include its Securities in such registration shall be conditioned upon such Holder's participation in such underwriting and the inclusion of such Holder's Securities in the underwriting (unless otherwise mutually agreed by a majority in interest of the Initiating Holders and such Holder) to the extent provided herein. All Holders proposing to distribute their securities through such underwriting shall enter into an underwriting agreement in customary form with the underwriter or underwriters selected for such underwriting by the Company (which underwriter or underwriters shall be reasonably acceptable to those Initiating Holders holding a majority of the Securities then held by all Initiating Holders). Notwithstanding any other provision of this Section 2.1, if the underwriter advises the Company that marketing factors require a limitation on the number of securities underwritten (including Securities), then the Company shall so advise all Holders of Securities that would otherwise be underwritten pursuant hereto, and the number of shares that may be included in the underwriting shall be allocated to the Holders of such Securities pro rata based on the number of Securities held by all such Holders (including the Initiating Holders). In no event shall any Securities be excluded from such underwriting unless all other securities are first excluded. Any Securities excluded or withdrawn from such underwriting shall be withdrawn from the registration.

(c) Notwithstanding the foregoing, the Company shall not be required to effect a registration pursuant to this Section 2.1:

(i) in any particular jurisdiction in which the Company would be required to execute a general consent to service of process in effecting such registration, unless the Company is already subject to service in such jurisdiction and except as may be required under the Securities Act; or

(ii) after the Company has effected two (2) registrations pursuant to this Section 2.1, and such registrations have been declared or ordered effective; or

(iii) during the period starting with the date sixty (60) days prior to the Company's good faith estimate of the date of the filing of and ending on a date one hundred eighty (180) days following the effective date of a Company-initiated registration subject to Section 2.2 below, provided that the Company is actively employing in good faith its commercially reasonable efforts to cause such registration statement to become effective; or

(iv) if the Initiating Holders propose to dispose of Securities that may be registered on Form S-3 pursuant to Section 2.3 hereof; or

(v) if the Company shall furnish to Holders requesting a registration statement pursuant to this Section 2.1 a certificate signed by the Company's Chief Executive Officer or Chairman of the Board of Directors stating that in the good faith judgment of the Board, it would be seriously detrimental to the Company and its stockholders for such registration statement to be effected at such time, in which event the Company shall have the right to defer such filing for a period of not more than ninety (90) days after receipt of the request of the Initiating Holders; provided that such right shall be exercised by the Company not more than twice in any twelve (12) month period.

2.2 Company Registration.

(a) If (but without any obligation to do so) the Company proposes to register (including for this purpose a registration effected by the Company for stockholders other than the Holders) any of its stock or other securities under the Securities Act in connection with the public offering of such securities (other than (i) a registration relating to a demand pursuant to Section 2.1 of this Agreement or (ii) a registration relating solely to the sale of securities of participants in a Company stock plan, a registration relating to a corporate reorganization or transaction under Rule 145 of the Securities Act, a registration on any form that does not include substantially the same information as would be required to be included in a registration statement covering the sale of the Securities, or a registration in which the only Common Stock being registered is Common Stock issuable upon conversion of debt securities that are also being registered), the Company shall, at such time, promptly give each Holder written notice of such registration. Upon the written request of each Holder given within twenty (20) days after mailing of such notice by the Company in accordance with Section 4.5 of this Agreement, the Company shall, subject to the provisions of Section 2.2(c) of this Agreement, use its commercially reasonable efforts to cause to be registered under the Securities Act all of the Securities that each such Holder requests to be registered.

(b) Right to Terminate Registration. The Company shall have the right to terminate or withdraw any registration initiated by it under this Section 2.2 prior to the effectiveness of such registration whether or not any Holder has elected to include securities in such registration. The expenses of such withdrawn registration shall be borne by the Company in accordance with Section 2.6 hereof.

(c) Underwriting Requirements. In connection with any offering involving an underwriting of shares of the Company's capital stock, the Company shall not be required under this Section 2.2 to include any of the Holders' securities in such underwriting unless they accept the terms of the underwriting as agreed upon between the Company and the underwriters selected by the Company (or by other Persons entitled to select the underwriters) and enter into an underwriting agreement in customary form with such underwriters, and then only in such quantity as the underwriters determine in their sole discretion will not jeopardize the success of the offering by the Company. If the total amount of securities, including Securities, requested by stockholders to be included in such offering exceeds the amount of securities sold other than by the Company that the underwriters determine in their sole discretion is compatible with the success of the offering, then the Company shall be required to include in the offering only that number of such securities, including Securities, that the underwriters determine in their sole discretion will not jeopardize the success of the offering. In the event that the underwriters determine that less than all of the Securities requested to be registered can be included in such offering, then the Securities that are included in such offering shall be apportioned pro rata among the selling Holders based on the number of Securities held by all selling Holders or in such other proportions as shall mutually be agreed to by all such selling Holders. Notwithstanding the foregoing, in no event shall (i) any Securities be excluded from such offering unless all other stockholders' securities have been first excluded from the offering and (ii) the amount of securities of the selling Holders included in the offering be reduced below twenty percent (20%) of the total amount of securities included in such offering, unless such offering is the Initial Offering, in which case the selling Holders may be excluded if the

underwriters make the determination described above and no other stockholder's securities are included in such offering. For purposes of the preceding sentence concerning apportionment, for any selling stockholder that is a Holder of Securities and that is a venture capital fund, partnership or corporation, the affiliated venture capital funds, partners, members, retired partners and stockholders of such Holder, or the estates and family members of any such partners, members and retired partners and any trusts for the benefit of any of the foregoing Persons shall be deemed to be a single "selling Holder," and any pro rata reduction with respect to such "selling Holder" shall be based upon the aggregate amount of Securities owned by all such related entities and individuals.

2.3 Form S-3 Registration. In case the Company shall receive from the Holders of at least thirty percent (30%) of the Securities (for purposes of this Section 2.3, the "S-3 Initiating Holders") a written request or requests that the Company effect a registration on Form S-3 and any related qualification or compliance with respect to all or a part of the Securities owned by such Holder or Holders, the Company shall:

(a) promptly give written notice of the proposed registration, and any related qualification or compliance, to all other Holders; and

(b) use its commercially reasonable efforts to effect, as soon as practicable, such registration and all such qualifications and compliances as may be so requested and as would permit or facilitate the sale and distribution of all or such portion of such Holders' Securities as are specified in such request, together with all or such portion of the Securities of any other Holders joining in such request as are specified in a written request given within fifteen (15) days after receipt of such written notice from the Company; provided, however, that the Company shall not be obligated to effect any such registration, qualification or compliance, pursuant to this Section 2.3:

(i) if Form S-3 is not available for such offering by the Holders;

(ii) if the Holders, together with the holders of any other securities of the Company entitled to inclusion in such registration, propose to sell Securities and such other securities (if any) at an aggregate price to the public (net of any underwriters' discounts or commissions) of less than $10,000,000;

(iii) if the Company shall furnish to all Holders requesting a registration statement pursuant to this Section 2.3 a certificate signed by the Company's Chief Executive Officer or Chairman of the Board of Directors stating that in the good faith judgment of the Board, it would be seriously detrimental to the Company and its stockholders for such registration statement to be effected at such time, in which event the Company shall have the right to defer such filing for a period of not more than ninety (90) days after receipt of the request of the S-3 Initiating Holders; provided that such right shall be exercised by the Company not more than twice in any twelve (12) month period;

(iv) if the Company has, within the twelve (12) month period preceding the date of such request, already effected two (2) registrations on Form S-3 pursuant to this Section 2.3;

(v) in any particular jurisdiction in which the Company would be required to qualify to do business or to execute a general consent to service of process in effecting such registration, qualification or compliance;

(vi) if the Company, within thirty (30) days of receipt of the request of such S-3 Initiating Holders, gives notice of its bona fide intention to effect the filing of a registration statement with the SEC within one hundred twenty (120) days of receipt of such request (other than a registration effected solely to qualify an employee benefit plan or to effect a business combination pursuant to Rule 145), provided that the Company is actively employing in good faith its commercially reasonable efforts to cause such registration statement to become effective; or

(vii) during the period starting with the date thirty (30) days prior to the Company's good faith estimate of the date of the filing of and ending on a date ninety (90) days following the effective date of a Company-initiated registration subject to Section 2.2 of this Agreement, provided that the Company is actively employing in good faith its commercially reasonable efforts to cause such registration statement to become effective.

(c) If the S-3 Initiating Holders intend to distribute the Securities covered by their request by means of an underwriting, they shall so advise the Company as a part of their request made pursuant to this Section 2.3 and the Company shall include such information in the written notice referred to in Section 2.3(a). The provisions of Section 2.1(b) of this Agreement shall be applicable to such request (with the substitution of Section 2.3 for references to Section 2.1).

(d) Subject to the foregoing, the Company shall file a registration statement covering the Securities and other securities so requested to be registered as soon as practicable after receipt of the request or requests of the S-3 Initiating Holders. Registrations effected pursuant to this Section 2.3 shall not be counted as requests for registration effected pursuant to Section 2.1 of this Agreement.

2.4 Obligations of the Company. Whenever required under this Section 2 to effect the registration of any Securities, the Company shall, as expeditiously as reasonably possible:

(a) prepare and file with the SEC a registration statement with respect to such Securities and use its commercially reasonable efforts to cause such registration statement to become effective, and, upon the request of the Holders of a majority of the Securities registered thereunder, keep such registration statement effective for a period of up to one hundred twenty (120) days or, if earlier, until the distribution contemplated in the Registration Statement has been completed;

(b) prepare and file with the SEC such amendments and supplements to such registration statement and the prospectus used in connection with such registration statement as may be necessary to comply with the provisions of the Securities Act with respect to the disposition of all securities covered by such registration statement;

(c) furnish to the Holders such number of copies of a prospectus, including a preliminary prospectus and any Free Writing Prospectus, in conformity with the requirements of the Securities Act, and such other documents as they may reasonably request in order to facilitate the disposition of Securities owned by them;

(d) use its commercially reasonable efforts to register and qualify the securities covered by such registration statement under such other securities or Blue Sky laws of such jurisdictions as shall be reasonably requested by the Holders, provided that the Company shall not be required in connection therewith or as a condition thereto to qualify to do business or to file a general consent to service of process in any such states or jurisdictions;

(e) in the event of any underwritten public offering, enter into and perform its obligations under an underwriting agreement, in usual and customary form, with the managing underwriter of such offering;

(f) notify each Holder of Securities covered by such registration statement at any time when a prospectus or Free Writing Prospectus (to the extent prepared by or on behalf of the Company) relating thereto is required to be delivered under the Securities Act of the happening of any event as a result of which the prospectus included in such registration statement, as then in effect, includes an untrue statement of a material fact or omits to state a material fact required to be stated therein or necessary to make the statements therein not misleading in the light of the circumstances then existing, and, at the request of any such Holder, the Company will, as soon as reasonably practicable, file and furnish to all such Holders a supplement or amendment to such prospectus or Free Writing Prospectus (to the extent prepared by or on behalf of the Company) so that, as thereafter delivered to the purchasers of such Securities, such prospectus will not contain an untrue statement of a material fact or omit to state any fact necessary to make the statements therein not misleading in light of the circumstances under which they were made;

(g) cause all such Securities registered pursuant to this Section 2 to be listed on a national exchange or trading system and on each securities exchange and trading system on which similar securities issued by the Company are then listed; and

(h) provide a transfer agent and registrar for all Securities registered pursuant to this Agreement and a CUSIP number for all such Securities, in each case not later than the effective date of such registration.

Notwithstanding the provisions of this Section 2, the Company shall be entitled to postpone or suspend, for a reasonable period of time, the filing, effectiveness or use of, or trading under, any registration statement if the Company shall determine that any such filing or the sale of any securities pursuant to such registration statement would in the good faith judgment of the Board:

(i) materially impede, delay or interfere with any material pending or proposed financing, acquisition, corporate reorganization or other similar transaction involving the Company for which the Board has authorized negotiations;

(ii) materially and adversely impair the consummation of any pending or proposed material offering or sale of any class of securities by the Company; or

(iii) require disclosure of material nonpublic information that, if disclosed at such time, would be materially harmful to the interests of the Company and its stockholders; provided, however, that during any such period all executive officers and directors of the Company are also prohibited from selling securities of the Company (or any security of any of the Company's subsidiaries or affiliates).

In the event of the suspension of effectiveness of any registration statement pursuant to this Section 2.4, the applicable time period during which such registration statement is to remain effective shall be extended by that number of days equal to the number of days the effectiveness of such registration statement was suspended.

2.5 Information from Holder. It shall be a condition precedent to the obligations of the Company to take any action pursuant to this Section 2 with respect to the Securities of any selling Holder that such Holder shall furnish to the Company such information regarding itself, the Securities held by it, and the intended method of disposition of such securities as shall be reasonably required to effect the registration of such Holder's Securities.

2.6 Expenses of Registration. All expenses other than underwriting discounts and commissions incurred in connection with registrations, filings or qualifications pursuant to Sections 2.1 and 2.2 of this Agreement, including, without limitation, all registration, filing and qualification fees, printers' and accounting fees, fees and disbursements of counsel for the Company and the reasonable fees and disbursements of one counsel for the selling Holders (not to exceed $50,000) shall be borne by the Company. Notwithstanding the foregoing, the Company shall not be required to pay for any expenses of any registration proceeding begun pursuant to Section 2.1 of this Agreement if the registration request is subsequently withdrawn at the request of the Holders of a majority of the Securities to be registered (in which case all participating Holders shall bear such expenses pro rata based upon the number of Securities that were to be included in the withdrawn registration) unless, in the case of a registration requested under Section 2.1 of this Agreement, the Holders of a majority of the Securities agree to forfeit their right to one demand registration pursuant to Section 2.1 of this Agreement; provided, however, that if at the time of such withdrawal, the Holders have learned of a material adverse change in the condition, business or prospects of the Company from that known to the Holders at the time of their request and have withdrawn the request with reasonable promptness following disclosure by the Company of such material adverse change, then the Holders shall not be required to pay any of such expenses and shall retain their rights pursuant to Section 2.1 of this Agreement. All expenses incurred in connection with a registration requested pursuant to Section 2.3 of this Agreement, including, without limitation, all registration, filing, qualification, printer's and accounting fees and the reasonable fees and disbursements of counsel for the selling Holder or Holders and counsel for the Company, shall be borne pro rata by the Holder or Holders participating in such registration effected pursuant to Section 2.3 of this Agreement.

2.7 Delay of Registration. No Holder shall have any right to obtain or seek an injunction restraining or otherwise delaying any such registration as the result of any controversy that might arise with respect to the interpretation or implementation of this Section 2.

2.8 Indemnification. In the event any Securities are included in a registration statement under this Section 2:

(a) To the extent permitted by law, the Company will indemnify and hold harmless each Holder, the partners, members, officers, directors and stockholders of each Holder, legal counsel and accountants for each Holder, any underwriter (as defined in the Securities Act) for such Holder and each Person, if any, who controls such Holder or underwriter within the meaning of the Securities Act or the 1934 Act, against any losses, claims, damages or liabilities (joint or several) to which they may become subject under the Securities Act, the 1934 Act, any state securities laws or any rule or regulation promulgated under the Securities Act, the 1934 Act or any state securities laws, insofar as such losses, claims, damages, or liabilities (or actions or proceedings, whether commenced or threatened, in respect thereof) arise out of or are based upon any of the following statements, omissions or violations (collectively, a "Violation"): (i) any untrue or alleged untrue statement of a material fact contained in such registration statement, including any preliminary prospectus, final prospectus, or Free Writing Prospectus contained therein or any amendments or supplements thereto, any issuer information (as defined in Rule 433 of the Securities Act) filed or required to be filed pursuant to Rule 433(d) under the Securities Act or any other document incident to such registration prepared by or on behalf of the Company or used or referred to by the Company, (ii) the omission or alleged omission of a material fact required to be stated in such registration statement, or necessary to make the statements therein not misleading or (iii) any violation or alleged violation by the Company of the Securities Act, the 1934 Act, any state securities laws or any rule or regulation promulgated under the Securities Act, the 1934 Act or any state securities laws, and the Company will reimburse each such Holder, underwriter, controlling Person or other aforementioned Person for any legal or other expenses reasonably incurred by them in connection with investigating or defending any such loss, claim, damage, liability, action or proceeding as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(a) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability, action or proceeding if such settlement is effected without the consent of the Company (which consent shall not be unreasonably withheld), nor shall the Company be liable in any such case for any such loss, claim, damage, liability, action or proceeding to the extent that it arises out of or is based upon a Violation that occurs in reliance upon, and in conformity with, written information furnished expressly for use in connection with such registration by any such Holder, underwriter, controlling Person or other aforementioned Person.

(b) To the extent permitted by law, each selling Holder, severally and not jointly, will indemnify and hold harmless the Company, each of its directors, each of its officers who has signed the registration statement, each Person, if any, who controls the Company within the meaning of the Securities Act, legal counsel and accountants for the Company, any underwriter, any other Holder selling securities in such registration statement and any controlling Person of any such underwriter or other Holder, against any losses, claims, damages or liabilities (joint or several) to which any of the foregoing Persons may become subject, under the Securities Act, the 1934 Act, any state securities laws or any rule or regulation

promulgated under the Securities Act, the 1934 Act or any state securities laws, insofar as such losses, claims, damages or liabilities (or actions or proceedings, whether commenced or threatened, in respect thereof) arise out of or are based upon any Violation, in each case to the extent (and only to the extent) that such Violation occurs in reliance upon and in conformity with written information furnished by such Holder expressly for use in connection with such registration; and each such Holder will reimburse any Person intended to be indemnified pursuant to this Section 2.8(b) for any legal or other expenses reasonably incurred by such Person in connection with investigating or defending any such loss, claim, damage, liability, action or proceeding as such expenses are incurred; provided, however, that the indemnity agreement contained in this Section 2.8(b) shall not apply to amounts paid in settlement of any such loss, claim, damage, liability, action or proceeding if such settlement is effected without the consent of the Holder (which consent shall not be unreasonably withheld), and provided that in no event shall any indemnity under this Section 2.8(b) exceed the gross proceeds from the offering received by such Holder.

(c) Promptly after receipt by an indemnified party under this Section 2.8 of notice of the commencement of any action or proceeding (including any governmental action or proceeding) for which a party may be entitled to indemnification, such indemnified party will, if a claim in respect thereof is to be made against any indemnifying party under this Section 2.8, deliver to the indemnifying party a written notice of the commencement thereof and the indemnifying party shall have the right to participate in and, to the extent the indemnifying party so desires, jointly with any other indemnifying party similarly noticed, to assume the defense thereof with counsel mutually satisfactory to the parties; provided, however, that an indemnified party (together with all other indemnified parties that may be represented without conflict by one counsel) shall have the right to retain one (1) separate counsel, with the fees and expenses to be paid by the indemnifying party, if representation of such indemnified party by the counsel retained by the indemnifying party would be inappropriate due to actual or potential differing interests between such indemnified party and any other party represented by such counsel in such proceeding. The failure to deliver written notice to the indemnifying party within a reasonable time of the commencement of any such action or proceeding, if prejudicial to its ability to defend such action or proceeding, shall relieve such indemnifying party of liability to the indemnified party under this Section 2.8 to the extent of such prejudice, but the omission to so deliver written notice to the indemnifying party will not relieve such indemnifying party of any liability that it may have to any indemnified party otherwise than under this Section 2.8.

(d) If the indemnification provided for in this Section 2.8 is held by a court of competent jurisdiction to be unavailable to an indemnified party with respect to any loss, liability, claim, damage or expense referred to herein, then the indemnifying party, in lieu of indemnifying such indemnified party hereunder, shall contribute to the amount paid or payable by such indemnified party as a result of such loss, liability, claim, damage or expense in such proportion as is appropriate to reflect the relative fault of the indemnifying party on the one hand and the indemnified party on the other hand in connection with the statements or omissions that resulted in such loss, liability, claim, damage or expense, as well as any other relevant equitable considerations; provided, however, that (i) no contribution by any Holder, when combined with any amounts paid by such Holder pursuant to Section 2.8(b), shall exceed the gross proceeds from the offering received by such Holder and (ii) no Person guilty of fraudulent misrepresentation (within the meaning of Section 11(f) of the Securities Act) will be entitled to

contribution from any Person who was not guilty of such fraudulent misrepresentation; and provided further that in no event shall a Holder's liability pursuant to this Section 2.8(d), when combined with the amounts paid or payable by such Holder pursuant to Section 2.8(b), exceed the proceeds from the offering received by such Holder (net of any expenses paid by such Holder). The relative fault of the indemnifying party and the indemnified party shall be determined by reference to, among other things, whether the untrue or alleged untrue statement of a material fact or the omission or alleged omission to state a material fact relates to information supplied by the indemnifying party or by the indemnified party and the parties' relative intent, knowledge, access to information and opportunity to correct or prevent such statement or omission.

(e) Notwithstanding the foregoing, to the extent that the provisions on indemnification and contribution contained in the underwriting agreement entered into in connection with the underwritten public offering are in conflict with the foregoing provisions, the provisions in the underwriting agreement shall control.

(f) The obligations of the Company and Holders under this Section 2.8 shall survive the completion of any offering of Securities in a registration statement under this Section 2 and otherwise

2.9 Reports Under the 1934 Act. With a view to making available to the Holders the benefits of Rule 144 and any other rule or regulation of the SEC that may at any time permit a Holder to sell securities of the Company to the public without registration or pursuant to a registration on Form S-3, the Company agrees to:

(a) make and keep public information available, as those terms are understood and defined in Rule 144, at all times after the effective date of the Initial Offering;

(b) file with the SEC in a timely manner all reports and other documents required of the Company under the Securities Act and the 1934 Act; and

(c) furnish to any Holder, so long as the Holder owns any Securities, forthwith upon request (i) a written statement by the Company that it has complied with the reporting requirements of Rule 144 (at any time after ninety (90) days after the effective date of the first registration statement filed by the Company), the Securities Act and the 1934 Act (at any time after it has become subject to such reporting requirements), or that it qualifies as a registrant whose securities may be resold pursuant to Form S-3 (at any time after it so qualifies), (ii) a copy of the most recent annual or quarterly report of the Company and such other reports and documents so filed by the Company and (iii) such other information as may be reasonably requested to avail any Holder of any rule or regulation of the SEC that permits the selling of any such securities without registration or pursuant to such form.

2.10 Assignment of Registration Rights. The rights to cause the Company to register Securities pursuant to this Section 2 may be assigned (but only with all related obligations) by a Holder to a transferee or assignee of such securities that (a) is an Affiliate, subsidiary, parent, partner, limited partner, retired partner, member or stockholder of a Holder, or a family member or trust for the benefit of any of the foregoing, (b) is a Holder's family member

or trust for the benefit of an individual Holder or (c) in the case of Beacon Bioventures Fund III LP, up to an aggregate of fifteen (15) employees of Beacon Bioventures Fund III LP or its Affiliates; provided: (i) the Company is, within a reasonable time after such transfer, furnished with written notice of the name and address of such transferee or assignee and the securities with respect to which such registration rights are being assigned; (ii) such transferee or assignee agrees in writing to be bound by and subject to the terms and conditions of this Agreement, including, without limitation, the provisions of Section 2.12 of this Agreement; and (iii) such assignment shall be effective only if immediately following such transfer the further disposition of such securities by the transferee or assignee is restricted under the Securities Act.

2.11 Limitations on Subsequent Registration Rights. From and after the date of this Agreement, the Company shall not, without the prior written consent of the Holders holding at least fifty-five percent (55%) of the Securities then held by all Holders, enter into any agreement with any holder or prospective holder of any securities of the Company that would allow such holder or prospective holder (a) to include any of such securities in any registration filed under Section 2.1, Section 2.2 or Section 2.3 of this Agreement, unless under the terms of such agreement, such holder or prospective holder may include such securities in any such registration only to the extent that the inclusion of such securities will not reduce the amount of the Securities of the Holders that are included or (b) to demand registration of their securities.

2.12 "Market Stand-Off" Agreement.

(a) Each Holder hereby agrees that it will not, without the prior written consent of the managing underwriter, during the period commencing on the date of the final prospectus relating to the Initial Offering and ending on the date specified by the Company and the managing underwriter (such period not to exceed one hundred eighty (180) days) (i) lend, offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, or otherwise transfer or dispose of, directly or indirectly, any shares of Common Stock or any securities convertible into or exercisable or exchangeable for Common Stock held immediately before the effective date of the Registration Statement for such offering, or (ii) enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the Common Stock, whether any such transaction described in clause (i) or (ii) above is to be settled by delivery of Common Stock or other securities, in cash or otherwise. The foregoing provisions of this Section 2.12 shall apply only to the Initial Offering, shall not apply to the sale of any shares to an underwriter pursuant to an underwriting agreement, and shall only be applicable to the Holders if all officers, directors and greater than one percent (1%) stockholders of the Company enter into similar agreements. The underwriters in connection with the Initial Offering are intended third-party beneficiaries of this Section 2.12 and shall have the right, power and authority to enforce the provisions hereof as though they were a party hereto. Each Holder further agrees to execute such agreements as may be reasonably requested by the underwriters in the Initial Offering that are consistent with this Section 2.12 or that are necessary to give further effect thereto. Any discretionary waiver or termination of the restrictions of any or all of such agreements by the Company or the underwriters shall apply to all Holders subject to such agreements pro rata based on the number of shares subject to such agreements.

(b) In order to enforce the foregoing covenant, the Company may impose stop-transfer instructions with respect to the Securities of each Holder (and the shares or securities of every other Person subject to the foregoing restriction) until the end of such period.

(c) Each Holder agrees that a legend reading substantially as follows shall be placed on all certificates representing all shares or securities of the Company of each Holder (and the shares or securities of every other Person subject to the restriction contained in this Section 2.12):

THE SECURITIES REPRESENTED BY THIS CERTIFICATE ARE SUBJECT TO A LOCK-UP PERIOD AFTER THE EFFECTIVE DATE OF THE ISSUER'S REGISTRATION STATEMENT FILED UNDER THE SECURITIES ACT, AS AMENDED, AS SET FORTH IN AN AGREEMENT BETWEEN THE COMPANY AND THE ORIGINAL HOLDER OF THESE SECURITIES, A COPY OF WHICH MAY BE OBTAINED AT THE ISSUER'S PRINCIPAL OFFICE. SUCH LOCK-UP PERIOD IS BINDING ON TRANSFEREES OF THESE SHARES.

2.13 Termination of Registration Rights. No Holder shall be entitled to exercise any right provided for in this Section 2: (a) after five (5) years following the consummation of the Initial Offering, (b) as to any Holder, such earlier time after the Initial Offering at which such Holder (i) can sell all shares held by it in compliance with Rule 144(b)(1)(i) or (ii) holds one percent (1%) or less of the Company's outstanding Common Stock and all Securities held by such Holder (together with any Affiliate of the Holder with whom such Holder must aggregate its sales under Rule 144) can be sold in any three (3) month period without registration in compliance with Rule 144 or (c) after the consummation of a Liquidation Event, as that term is defined in the Certificate.

3. Covenants of the Company.

3.1 Delivery of Financial Statements.

(a) The Company shall deliver to each Holder holding at least 100,000 Securities (appropriately adjusted for any stock split, dividend, combination or other recapitalization):

(i) as soon as practicable, but in any event within one hundred twenty (120) days after the end of each fiscal year of the Company, an unaudited income statement for such fiscal year, an unaudited balance sheet of the Company and statement of stockholders' equity as of the end of such year, and an unaudited statement of cash flows for such year, such year-end financial reports to be in reasonable detail, prepared in accordance with generally accepted accounting principles ("GAAP"); provided, however, that if the Company has such financial statements audited by independent certified public accountants, the Company shall provide to each Holder a copy of such audited financial statements, together with any report on such financial statements prepared by the independent certified public accountants that conducted the audit and audited and certified by independent public accountants of nationally recognized standing selected by the Company;

(ii) as soon as practicable, but in any event within sixty (60) days after the end of each of the first three (3) quarters of each fiscal year of the Company, an unaudited income statement and statement of cash flows for such fiscal quarter and an unaudited balance sheet and a statement of stockholders' equity as of the end of such fiscal quarter, all prepared in accordance with GAAP (except that such financial statements may (A) be subject to normal year-end audit adjustments and (B) not contain all notes thereto that may be required in accordance with GAAP);

(iii) within thirty (30) days of being approved by the Board, copies of any business plans, development plans or periodic internal reports, including reports of financial condition; and

(iv) within thirty (30) days of any change in capital ownership of the Company, an updated capitalization schedule.

(b) Notwithstanding anything else in this Section 3.1 to the contrary, the Company may cease providing the information set forth in this Section 3.1 during the period starting with the date thirty (30) days before the Company's good-faith estimate of the date of filing of a registration statement if it reasonably concludes it must do so to comply with the SEC rules applicable to such registration statement and related offering; provided that the Company's covenants under this Section 3.1 shall be reinstated at such time as the Company is no longer actively employing its commercially reasonable efforts to cause such registration statement to become effective.

3.2 Termination of Information Covenants. The covenants set forth in Section 3.1 shall terminate and be of no further force or effect upon the earlier to occur of (a) the consummation of the sale of securities pursuant to a registration statement filed by the Company under the Securities Act in connection with the firm commitment underwritten offering of its securities to the general public, (b) when the Company first becomes subject to the periodic reporting requirements of Sections 12(g) or 15(d) of the 1934 Act, whichever event shall first occur and (c) the consummation of a Liquidation Event, as that term is defined in the Certificate.

3.3 GSK and API Pre-emptive Rights.

(a) Until such time as (i) with respect to GSK, the GSK Dilution Threshold (as defined in the Certificate) or (ii) with respect to API, the API Dilution Threshold (as defined in the Certificate) has been exceeded, in the event that the Company issues New Securities (other than issuances of equity securities as compensation or as part of an incentive arrangement, including without limitation the issuance of options to purchase Common Stock under the Incentive Plan, or in connection with a split, dividend or similar event), the Company shall first offer to sell to GSK or API, as applicable pursuant to clause (i) or (ii) above, a portion of the New Securities being issued equal to each such Investor's Proportional Share. If such Investor elects to participate in the contemplated issuance, as set forth in Section 3.3(c) and (d) below, then such Investor's rights under Article V, Section B(4)(l) or B(4)(m), as applicable, of the Certificate shall cease and such Investor shall thereafter only have pre-emptive rights as set forth in this Section 3.3(a) and in Section 3.3(b) below.

(b) Any time after the transaction pursuant to which the GSK Dilution Threshold or API Dilution Threshold, as the case may be, is exceeded, if the Company authorizes the issuance and sale of New Securities, the Company shall first offer to sell to such Investor a portion of the New Securities being issued equal to such Investor's Proportional Share. If such Investor has not exercised its rights under Section 3.3(a) above prior to first exceeding the GSK Dilution Threshold or the API Dilution Threshold, as applicable, and, in a single transaction, the Company issues New Securities in connection with the transaction that causes the GSK Dilution Threshold or the API Dilution Threshold, as the case may be, to be reached and exceeded, then such transaction shall be bifurcated and treated as two separate transactions for purposes of this Section 3.3(b) (i.e., one transaction in which the Company issues New Securities in connection with a transaction that causes the GSK Dilution Threshold or the API Dilution Threshold, as the case may be, to be reached and a second transaction subject to this Section 3.3(b) in which the Company issues additional New Securities).

(c) To implement the foregoing, at least twenty (20) days prior to the date of any issuance by the Company of any New Securities that triggers GSK's or API's rights under Sections 3.3(a) or (b) above (the "Proposed Issuance Date"), the Company shall deliver a written notice (the "Issuance Notice") to such Investor specifying in reasonable detail the number and type of New Securities to be issued and the terms and conditions of the issuance. Such Investor may elect to participate in the contemplated issuance at the same price per New Securities (however denominated) and on the same terms by delivering written notice to the Company at least five (5) days prior to the Proposed Issuance Date specifying the number of New Securities such Investor desires to purchase, up to such Investor's Proportional Share.

(d) Upon the delivery of the Issuance Notice and subject to the provisions hereof, no more than 30 days after the delivery of the Issuance Notice, the Company shall sell, and such Investor, if it so elects, shall purchase, the amount of New Securities determined pursuant to Sections 3.3(a) or (b) above at a mutually agreeable time and place (the "Issuance Closing"). At the Issuance Closing, the Company shall deliver to such Investor the certificates or other instruments representing the issued New Securities (if certificated), free and clear of all liens and encumbrances, and such Investor shall deliver to the Company the purchase price therefore by cashier's or certified check payable to the Company or by wire transfer of immediately available funds to an account designated by the Company.

(e) The preemptive rights in this Section 3.3 shall not be applicable to (i) Exempted Securities (as defined in the Certificate), (ii) shares of Common Stock issued in the Initial Offering and (iii) the issuance of shares of Series D Preferred Stock pursuant to the Purchase Agreement.

3.4 Preferred Stock Pre-emptive Rights.

(a) In the event that the Company issues New Securities the Company shall first offer to sell to each Major Investor, such Major Investor's Proportional Share.

(b) To implement the foregoing, at least twenty (20) days prior to the date of any issuance by the Company of any New Securities that triggers any Major Investor's rights under Section 3.4(a) above (the "Major Investor Proposed Issuance Date"), the Company

shall deliver an Issuance Notice to each Major Investor specifying in reasonable detail the number and type of New Securities to be issued and the terms and conditions of the issuance. Each Major Investor may elect to participate in the contemplated issuance at the same price per share of New Securities (however denominated) and on the same terms by delivering written notice to the Company at least five days (5) prior to the Major Investor Proposed Issuance Date specifying the amount of New Securities each such Major Investor desires to purchase, up to such Major Investor's Proportional Share.

(c) Upon the delivery of the Issuance Notice and subject to the provisions hereof, no more than thirty (30) days after the delivery of the Issuance Notice, the Company shall sell, and each Major Investor, if such Major Investor so elects, shall purchase, the amount of New Securities determined pursuant to Section 3.4(a) above at a mutually agreeable time and place (the "Major Investor Issuance Closing"). At the Major Investor Issuance Closing, the Company shall deliver to each applicable Major Investor the certificates or other instruments representing the issued New Securities (if certificated), free and clear of all liens and encumbrances, and each applicable Major Investor shall deliver to the Company the purchase price therefore by cashier's or certified check payable to the Company or by wire transfer of immediately available funds to an account designated by the Company.

(d) The preemptive rights in this Section 3.4 shall not be applicable to Exempted Securities (as that term is defined in the Certificate).

3.5 Board Matters and Observer Rights.

(a) With respect to University, API or GSK, for so long as each such Investor owns not less than 100,000 shares of Common Stock (appropriately adjusted for any stock split, dividend, combination or other recapitalization), the Company shall invite a representative of such respective Investor that continues to hold such number of shares of Common Stock to attend all meetings of the Board in a nonvoting observer capacity and, in this respect, shall give each such representative copies of all notices, minutes, consents, and other materials that it provides to its directors; provided, however, that each such representative shall agree to hold in confidence and trust and to act in a fiduciary manner with respect to all information so provided or otherwise made available to such representative; and provided further, that the Company reserves the right to withhold any information and to exclude each such representative from any meeting or portion thereof if access to such information or attendance at such meeting could adversely affect the attorney-client privilege between the Company and its counsel or result in disclosure of trade secrets or a conflict of interest, or if University, API or GSK or its or their representative is or becomes an officer, director or security holder of a competitor of the Company.

(b) With respect to any Lead Observer, so long as such Lead Observer does not have a designee on the Board pursuant to that certain Amended and Restated Voting Agreement, dated as of May 15, 2015, and as may be amended, by among the Company and certain of its stockholders (the "Voting Agreement"), the Company shall invite a representative of such Lead Observer to attend all meetings of the Board in a nonvoting observer capacity and, in this respect, shall give each such representative copies of all notices, minutes, consents, and other materials that it provides to its directors; provided, however, that each such representative

shall agree to hold in confidence and trust and to act in a fiduciary manner with respect to all information so provided or otherwise made available to such representative; and provided further, that the Company reserves the right to withhold any information and to exclude each such representative from any meeting or portion thereof if access to such information or attendance at such meeting could adversely affect the attorney-client privilege between the Company and its counsel or result in disclosure of trade secrets or a conflict of interest, or if such Lead Observer or its representative is or becomes an officer, director or security holder of a competitor of the Company.

(c) With respect to Deerfield Private Design Fund III, L.P. ("Deerfield"), so long as Deerfield does not have a designee on the Board pursuant to the Voting Agreement, the Company shall invite a representative of Deerfield to attend all meetings of the Board in a nonvoting observer capacity and, in this respect, shall give each such representative copies of all notices, minutes, consents, and other materials that it provides to its directors; provided, however, that each such representative shall agree to hold in confidence and trust and to act in a fiduciary manner with respect to all information so provided or otherwise made available to such representative; and provided further, that the Company reserves the right to withhold any information and to exclude each such representative from any meeting or portion thereof if access to such information or attendance at such meeting could adversely affect the attorney-client privilege between the Company and its counsel or result in disclosure of trade secrets or a conflict of interest, or Deerfield or its representative is or becomes an officer, director or security holder of a competitor of the Company.

3.6 Indemnification Matters. The Company hereby acknowledges that one (1) or more of the directors nominated to serve on the Board by the Investors (each a "Fund Director") may have certain rights to indemnification, advancement of expenses and/or insurance provided by one or more of the Investors and certain of their affiliates (collectively, the "Fund Indemnitors"). The Company hereby agrees (a) that it is the indemnitor of first resort (i.e., its obligations to any such Fund Director are primary and any obligation of the Fund Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by such Fund Director are secondary), (b) that it shall be required to advance the full amount of expenses incurred by such Fund Director and shall be liable for the full amount of all expenses, judgments, penalties, fines and amounts paid in settlement by or on behalf of any such Fund Director to the extent legally permitted and as required by the Certificate or Bylaws of the Company (or any agreement between the Company and such Fund Director), without regard to any rights such Fund Director may have against the Fund Indemnitors, and, (c) that it irrevocably waives, relinquishes and releases the Fund Indemnitors from any and all claims against the Fund Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Fund Indemnitors on behalf of any such Fund Director with respect to any claim for which such Fund Director has sought indemnification from the Company shall affect the foregoing and the Fund Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of such Fund Director against the Company.

3.7 Confidentiality.

(a) Each Holder agrees, severally and not jointly, to use the same degree of care as such Holder uses to protect its own confidential information for any information obtained pursuant to this Agreement or otherwise as a stockholder of the Company including but not limited to confidential information of the Company and its subsidiaries regarding identifiable, specific and discrete business opportunities being pursued by the Company or its subsidiaries, and such Holder acknowledges that it will not, unless otherwise required by law or the rules of any national securities exchange, association or marketplace, disclose, take commercial or proprietary advantage of or profit from any such information without the prior written consent of the Company except such information that (i) was in the public domain prior to the time it was furnished to such Holder, (ii) is or becomes (through no willful improper action or inaction by such Holder) generally available to the public, (iii) was in its possession or known by such Holder without restriction prior to receipt from the Company, (iv) was rightfully disclosed to such Holder by a third party without restriction, (v) was independently developed without any use of the Company's confidential information, (vi) was to authorized representatives and employees of the Company or its subsidiaries and as otherwise may be proper in the course of performing such Holder's obligations, or enforcing such Holder's rights, under this Agreement, (vii) was part of a Holder's normal reporting or review procedure, or in connection with a Holder's or such Holder's Affiliates' or Investment Advisors', auditors, directors, officers, employees, professional consultants, attorneys or other agents or rating agencies, or to any regulatory authority having jurisdiction over such Holder, (viii) was to any bona fide prospective purchaser of the equity or assets of such Holder's or such Holder's Affiliates or the Shares held by such Holder or prospective merger partner of such Holder or such Holder's Affiliates, provided that such purchaser or merger partner agrees to be bound by the provisions of this Section 3.7, or (ix) as is required to be disclosed by order of a court of competent jurisdiction or other compulsory legal process, provided that (unless prohibited by law or final court order) the Holder required to make such disclosure shall provide to the Board prompt notice of such disclosure.

(b) Notwithstanding the foregoing, each Holder that is a limited partnership, limited liability company or Advisory Holder may disclose such proprietary or confidential information to any former partners or members who retained an economic interest in such Holder, current or prospective partner of the partnership or any subsequent partnership under common investment management, limited partner, general partner, member or management company of such Holder (or any employee or representative of any of the foregoing) (each of the foregoing Persons, a "Permitted Disclosee") or legal counsel, accountants or representatives for such Holder. Furthermore, nothing contained herein shall prevent any Holder or any Permitted Disclosee from (i) entering into any business, entering into any agreement with a third party, or investing in or engaging in investment discussions with any other company (whether or not competitive with the Company), provided that such Holder or Permitted Disclosee does not, except as permitted in accordance with this Section 3.7, disclose or otherwise make use of any proprietary or confidential information of the Company in connection with such activities, or (ii) making any disclosures required by law, rule, regulation or court or other governmental order.

(c) Without intending to limit the remedies available to the Company, each Holder acknowledges that a breach of any of the covenants contained in this Section 3.7 may result in material irreparable injury to the Company or its Affiliates for which there is no adequate remedy at law, that it will not be possible to measure damages for such injuries precisely and that, in the event of such a breach or threat thereof, to the fullest extent permitted by law, the Company shall be entitled to seek a temporary restraining order and/or a preliminary or permanent injunction restraining the Holder and/or such Holder's Affiliates from engaging in activities prohibited hereby or such other relief as may be required to specifically enforce any of the covenants contained herein, and to the fullest extent permitted by law, such Holder agrees not to oppose the granting of such injunctive relief on the basis that monetary damages are an adequate remedy. Each Holder hereby agrees and consents that such injunctive relief may be sought in the courts in the State of Delaware, or in any other court having competent jurisdiction. Each Holder agrees that he, she or it shall advise each Person to whom such Holder provides information pursuant to this Section 3.7 of the confidentiality and non-disclosure restrictions contained herein. Each Holder shall be liable and responsible for any breaches of this Section 3.7 by Persons to whom such Holder has provided information in compliance with this Section 3.7.

3.8 Right to Conduct Activities.

(a) The Company hereby agrees and acknowledges that each of the Investors (together with their respective Affiliates) is a professional investment fund and as such, such Investors and their respective Affiliates invest in numerous portfolio companies, some of which may be deemed competitive with the Company's business (as currently conducted or as currently proposed to be conducted). The Company hereby agrees that, to the extent permitted under applicable law, that the Investors and their respective Affiliates shall not be liable to the Company for any claim arising out of, or based upon, (i) the investment by such Investor or its Affiliates in any entity competitive with the Company, or (ii) actions taken by any partner, officer or other representative of such Investor or its Affiliates to assist any such competitive company, whether or not such action was taken as a member of the board of directors of such competitive company or otherwise, and whether or not such action has a detrimental effect on the Company; provided, however, that the foregoing shall not relieve (x) the Investors or their respective Affiliates from liability associated with the unauthorized disclosure of the Company's confidential information, or (y) any director or officer of the Company from any liability associated with his or her fiduciary duties to the Company.

3.9 Related Party Transactions.

(a) Following the date hereof, the Company agrees that it shall not enter into any material transaction with any Holder or Affiliate of the Company (other than any Excepted Transaction (as defined below), or revise the terms of an existing material transaction with any Holder or Affiliate of the Company unless:

(i) the terms of the transaction are not materially less favorable to the Company than those that could have been negotiated in a comparable arm's length transaction with an independent unrelated third party, or

(ii) such transaction is approved by a majority of the disinterested members of the Board, which majority must include either the Series C Director or the Series D Director (as such terms are defined in the Voting Agreement), to the extent such Series C Director or Series D Director is disinterested.

(b) For purposes hereof, an "Excepted Transaction" includes any of the following:

(i) any transaction providing for reasonable and customary arm's length fees and compensation paid to, or indemnity provided on behalf of, officers, directors, employees, consultants or advisors of the Company, as determined in good faith by the Board of Directors (it being understood that no fees may be paid by the Company to FoxKiser LLP or any Affiliate of FoxKiser LLP under this clause (i));

(ii) any transaction between or among the Company and any of its subsidiaries or between or among such subsidiaries; and

(iii) any transaction contemplated or permitted by this Agreement, the Amended and Restated Certificate of Incorporation of the Company and/or the Purchase Agreement (as defined in the Purchase Agreement).

3.10 Termination of Certain Covenants. The covenants set forth in Sections 3.3, 3.4, 3.5 and 3.9 shall terminate and be of no further force or effect immediately prior to the consummation of (a) the Company's sale of its Common Stock or other securities pursuant to Registration Statement under the Securities Act (other than a registration statement relating either to the sale of securities to employees of the Company pursuant to its stock option, stock purchase or similar plan or a transaction under Rule 145 of the Securities Act) or (b) a Liquidation Event, as that term is defined in the Certificate, whichever event occurs first.

4. Miscellaneous.

4.1 Successors and Assigns. Except as otherwise provided herein, the terms and conditions of this Agreement shall inure to the benefit of and be binding upon the respective successors and assigns of the parties (including transferees of any shares of Securities). Nothing in this Agreement, express or implied, is intended to confer upon any party other than the parties hereto or their respective successors and assigns any rights, remedies, obligations or liabilities under or by reason of this Agreement, except as expressly provided in this Agreement.

4.2 Governing Law. This Agreement shall be governed by and construed under the laws of the State of Delaware as applied to agreements among Delaware residents entered into and to be performed entirely within Delaware.

4.3 Counterparts; Facsimile. This Agreement may be executed by electronic signature and in two (2) or more counterparts, each of which shall be deemed an original, but all of which together shall constitute one (1) and the same instrument. Counterparts may be delivered by facsimile, electronic mail (including pdf) or other transmission method and any counterpart so delivered shall be deemed to have been duly and validly delivered and be valid and effective for all purposes.

4.4 Titles and Subtitles. The titles and subtitles used in this Agreement are used for convenience only and are not to be considered in construing or interpreting this Agreement.

4.5 Notices. All notices and other communications given or made pursuant hereto shall be in writing and shall be deemed effectively given upon the earlier to occur of actual receipt or: (a) upon personal delivery to the party to be notified, (b) when sent by confirmed electronic mail or facsimile if sent during normal business hours of the recipient; if not, then on the next business day, (c) five (5) days after having been sent by registered or certified mail, return receipt requested, postage prepaid, or (d) one (1) day after deposit with a nationally recognized overnight courier, specifying next day delivery, with written verification of receipt. All notices and other communications shall be sent to the Company at 9712 Medical Center Drive, Suite 100, Rockville, MD 20850, Attention: Chief Executive Officer and to the other parties at the addresses on record with the Company (or at such other addresses as shall be specified by notice given in accordance with this Section 4.5).

4.6 Expenses. If any action at law or in equity is necessary to enforce or interpret the terms of this Agreement, the prevailing party shall be entitled to reasonable attorneys' fees, costs and necessary disbursements in addition to any other relief to which such party may be entitled.

4.7 Entire Agreement; Amendments. This Agreement (including the Exhibits hereto, if any) constitutes the full and entire understanding and agreement among the parties with regard to the subjects hereof and thereof. Any term of this Agreement (other than Section 2.12, Section 3.1, Section 3.2, Section 3.3, Section 3.4, Section 3.5, Section 3.6, Section 3.9 and Section 3.10) may be amended and the observance of any term of this Agreement may be waived (either generally or in a particular instance and either retroactively or prospectively) only with the written consent of the Company and the Holders of at least fifty-five percent (55%) of the then outstanding capital stock held by the Holders. The provisions of Section 2.12, Section 3.9 and Section 3.10 may be amended or waived (either generally or in a particular instance and either retroactively or prospectively) only with the written consent of the Company and the Holders of at least fifty-five percent (55%) of the then outstanding capital stock held by the Holders, which must include at least three of the Lead Investors. The provisions of Section 3.1, Section 3.2, Section 3.3, Section 3.4, Section 3.5 and Section 3.6 may be amended or waived (either generally or in a particular instance and either retroactively or prospectively) only with the written consent of the Company and the Investors whose rights and/or obligations under such Sections would be directly affected by such amendment or waiver. Notwithstanding the foregoing, if an amendment, modification or waiver of this Agreement would have an adverse effect on the express rights or obligations hereunder of a Holder (each, an "Adverse Party") in a manner that differs from the express rights or obligations hereunder of the other Holders, such amendment, modification or waiver shall also require the written consent of the Adverse Parties holding a majority of the outstanding shares of Preferred Stock and Common Stock (voting together as a class and not as separate series and on an as-converted basis) then held by all Adverse Parties; provided that in the event Adverse Parties are holders of Series D Preferred Stock, such majority must include at least three of the Lead Investors. Notwithstanding anything to the contrary contained herein, no rights to information held by an Advisory Holder shall be waived, limited or amended without the consent of the Advisory Holders holding at least a majority of the capital stock of the Company held by Advisory Holders. Any amendment or waiver effected in accordance with this paragraph shall be binding upon each holder of any Securities, each future holder of all such Securities and the Company.

4.8 Severability. Whenever possible, each provision of this Agreement shall be interpreted in such manner as to be effective and valid under applicable law, but if any provision of this Agreement shall be held to be prohibited by or invalid under applicable law, such provision shall be ineffective only to the extent of such prohibition or invalidity, without invalidating the remainder of such provision or the remaining provisions of this Agreement.

4.9 Aggregation of Stock. All shares of Securities held or acquired by affiliated entities (including affiliated venture capital funds or venture capital funds under common investment management or Advisory Holders with the same or affiliated Investment Advisor) or Persons shall be aggregated together for the purpose of determining the availability of any rights under this Agreement.

[*Remainder of page intentionally left blank*]

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

REGENXBIO INC.

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President and Chief Executive Officer

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Vivo Capital Fund VIII, L.P.
By: Vivo Capital VIII, LLC
Its: General Partner

By: /s/ Edgar Engleman
Name: Edgar Engleman
Title: Managing Partner

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Vivo Capital Surplus Fund VIII, L.P.
By: Vivo Capital VIII, LLC
Its: General Partner

By: /s/ Edgar Engleman
Name: Edgar Engleman
Title: Managing Partner

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Venrock Healthcare Capital Partners II, L.P.
By: VHCP Management II, LLC
Its: General Partner

VHCP CO-Investment Holdings II, LLC
By: VHCP Management II, LLC
Its: Manager

By: /s/ David L. Stepp
Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Venrock Associates VII, L.P.
By: Venrock Management VII, LLC
Its: General Partner

Venrock Partners VII, L.P.
By: Venrock Partners Management VII, LLC
Its: General Partner

By: /s/ David L. Stepp
Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

BEACON BIOVENTURES FUND III LIMITED PARTNERSHIP

By: Beacon Bioventures Advisors Fund III Limited Partnership, its sole General Partner

By: Impresa Management LLC, its sole General Partner

By: /s/ Mary Bevelock Pendergast
Name: Mary Bevelock Pendergast
Title: Vice President

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

T. Rowe Price Health Sciences Fund, Inc.
TD Mutual Funds – TD Health Sciences Fund
VALIC Company I – Health Sciences Fund
T. Rowe Price Health Sciences Portfolio
John Hancock Variable Insurance Trust – Health Sciences Trust
John Hancock Fund II – Health Sciences Fund
Each fund, severally and not jointly

By: T. Rowe Price Associates, Inc., Investment Advisor or Subadvisor, as applicable

By: /s/ Taymour Tamaddon
Name: Taymour Tamaddon
Title: Vice President

T. Rowe Price New Horizons Fund, Inc.
T. Rowe Price New Horizons Trust
T. Rowe Price U.S. Equities Trust
Each fund, severally not jointly

By: T. Rowe Price Associates, Inc., Investment Advisor

By: /s/ Henry Ellenbogen
Name: Henry Ellenbogen
Title: Vice President

Address:
T. Rowe Price Associates, Inc.
100 East Pratt Street
Baltimore, MD 21202
Attn: Andrew Baek, Vice President
Phone: 410-345-2090
E-mail: andrew_baek@troweprice.com

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

BlackRock Health Sciences Trust
By: BlackRock Advisors, LLC
Its: Investment Advisor

By: /s/ Erin Xie
Authorized Signatory

BlackRock Health Sciences Opportunities Portfolio, a series of BlackRock Funds
By: BlackRock Advisors, LLC
Its: Investment Advisor

By: /s/ Erin Xie
Authorized Signatory

BlackRock Health Sciences Master Unit Trust
By: BlackRock Capital Management, Inc.
Its: Investment Advisor

By: /s/ Erin Xie
Authorized Signatory

c/o BlackRock Advisors, LLC
Fundamental Equity – Global Opportunities
Health & Sciences Team
60 State Street, 19th/20th Floors
Boston, MA 02109
Attn: Erin Xie, Chian Jiang
Email: erin.xie@blackrock.com,
chian.jiang@blackrock.com

With a copy (which shall constitute notice) to:

c/o BlackRock, Inc.
Office of the General Counsel
40 East 52nd Street
New York, NY 10022
Attn: David Maryles and Vincent Taurassi
Email: legaltransactions@blackrock.com

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

THE ALLAN M. FOX TRUST (U/A/D APRIL 21, 2015)

By: /s/ John Daniel Kiser
Name: John Daniel Kiser
Title: Trustee

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

THE ALLAN M. FOX REVOCABLE TRUST

By: /s/ Allan M. Fox
Name: Allan M. Fox
Title: Trustee

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

THE KISER 2012 GIFT TRUST

By: /s/ Allan M. Fox
Name: Allan M. Fox
Title: Trustee

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

FOXKISER LLP

By: /s/ Allan M. Fox
Name: Allan M. Fox
Title: Partner

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Brookside Capital Partners Fund, L.P.
By: Matthew McPherron
Its: General Partner

By: /s/ Matthew McPherron
Name: Matthew McPherron
Title: General Partner

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

DEERFIELD PRIVATE DESIGN FUND III, L.P.
By: Deerfield Mgmt III, L.P.
General Partner

By: J.E. Flynn Capital III, LLC
General Partner

By: /s/ David J. Clark
Name: David J. Clark
Title: Authorized Signatory

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

GFO II, LLC
By: GFO, LLC, its Manager

By: /s/ George P. Levendis
Name: George P. Levendis
Title: Manager

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Kerry S. Propper

By: /s/ Kerry S. Propper
Name: Kerry S. Propper

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

David B. Schmickel

By: /s/ David B. Schmickel
Name: David B. Schmickel

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

William Edward Holtz

By: /s/ William Edward Holtz
Name: William Edward Holtz

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

Matthew M. Rosini

By: /s/ Matthew M. Rosini
Name: Matthew M. Rosini

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

G&H PARTNERS

By: /s/ Stefan J. Palmer, Jr.
Name: Stefan J. Palmer, Jr.
Title: General Partner

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

SOUTH OCEAN CAPITAL, LLC

By: /s/ Steven Oliveira
Name: Steven Oliveira
Title: Manager

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

RTW MASTER FUND, LTD.

By: /s/ Roderick Wong
Name: Roderick Wong
Title: Director

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

PERCEPTIVE LIFE SCIENCES MASTER FUND LTD.

By: /s/ James Mannix
Name: James Mannix
Title: Chief Operating Officer

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

CORNIX ADVISORS, LLC

By: /s/ Steven Urbach
Name: Steven Urbach
Title: President

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

FORESITE CAPITAL FUND II, LP

By: /s/ Dennis D. Ryan
Dennis D. Ryan CFO of
Name: Foresite Capital Management II, LLC
General Partner of
Title: Foresite Capital Fund II, LP

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

FOURTH AVENUE CAPITAL PARTNERS LP

By: Fourth Avenue Capital Partners GP LLC
Its General Partner

By: /s/ Tracy Fu
Name: Tracy Fu
Title: Managing Member

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

QVT FUND IV LP

By: QVT Associates GP LLC
Its General Partner

By: /s/ Tracy Fu
Name: Tracy Fu
Title: Managing Member

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

QUINTESSENCE FUND L.P.

By: QVT Associates GP LLC
Its General Partner

By: /s/ Tracy Fu
Name: Tracy Fu
Title: Managing Member

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

TITAL PERC, LTD

By: /s/ Darren Ross
Name: Darren Ross
Title: Director

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

TOURBILLON GLOBAL VENTURES, LLC

By: /s/ Jason Karp
Name: Jason Karp
Title: Managing Member of JHK Global Ventures, LLC, as Management Member of Tourbillon Global Ventures, LLC

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

CORMORANT GLOBAL HEALTHCARE MASTER FUND, LP

By: /s/ Bihua Chen
Name: Bihua Chen
Title: Managing Member of GP

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

NEW EMERGING MEDICAL OPPORTUNITIES FUND II, L.P.

By: Sectoral Asset Management
Its General Partner

By: /s/ Michael Sjostrom
Name: Michael Sjostrom
Title: Chief Investment Officer

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

JANUS GLOBAL LIFE SCIENCES FUND

By: /s/ Andrew Acker
Name: Andrew Acker
Title: Vice President

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTORS:

JENNISON GLOBAL HEALTHCARE MASTER FUND, LTD.

By: Jennison Associates LLC, as the Investment Manager of Jennison Global Healthcare Master Fund, Ltd.

By: /s/ David Chan
Name: David Chan
Title: Managing Director

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR AND COMMON HOLDER:

Kenneth T. Mills

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR AND COMMON HOLDER:

Vittal Vasista

By: /s/ Vittal Vasista
Name: Vittal Vasista

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

INVESTOR AND COMMON HOLDER:

Donald J. Hayden, Jr.

By: /s/ Donald J. Hayden, Jr.
Name: Donald J. Hayden, Jr.

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

REGENX HOLDINGS, LLC

By: /s/ John Daniel Kiser
Name: John Daniel Kiser
Title: Director

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

James M. Wilson

By: /s/ James M. Wilson
Name: James M. Wilson

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ John S. Swartley
Name: John S. Swartely
Title: Associate Vice Provost for Research
Executive Director, PCI

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

GLAXOSMITHKLINE LLC

By: /s/ William J. Mosher
Name: William J. Mosher
Title: Vice President & Secretary

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

ARIAD PHARMACEUTICALS, INC.

By: /s/ Thomas J. DesRosier
Name: Thomas J. DesRosier
Title: Executive Vice President – Chief Legal and Administrative Officer

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

Sara Garon Berl

By: /s/ Sara Garon Berl
Name: Sara Garon Berl

IN WITNESS WHEREOF, the parties have executed this Agreement as of the date first above written.

COMMON HOLDER:

Stephen Yoo

By: /s/ Stephen Yoo
Name: Stephen Yoo

SCHEDULE A

SCHEDULE OF INVESTORS

Investor

Vivo Capital Fund VIII, L.P.
Vivo Capital Surplus Fund VIII, L.P.
T. Rowe Price Health Sciences Fund, Inc.
TD Mutual Funds – TD Health Sciences Fund
VALIC Company I – Health Sciences Fund
T. Rowe Price Health Sciences Portfolio
John Hancock Variable Insurance Trust – Health
John Hancock Funds II – Health Sciences Fund
T. Rowe Price New Horizons Fund, Inc.
T. Rowe Price New Horizons Trust
T. Rowe Price U.S. Equities Trust
BlackRock Health Sciences Trust
BlackRock Health Sciences Master Unit Trust
BlackRock Health Sciences Opportunities Portfolio
Janus Global Life Sciences Fund
Perceptive Life Sciences Master Fund LTD
Titan-Perc LTD
Foresite Capital Fund II, LP
QVT Fund IV LP
Quintessence Fund L.P.
Fourth Avenue Capital Partners LP
New Emerging Medical Opportunities Fund II, L.P.
Tourbillon Global Ventures
Jennison Global Healthcare Master Fund, Ltd
Cormorant Global Healthcare Master Fund, LP
RTW Master Fund, Ltd.
Cornix Advisors, LLC
South Ocean Capital, LLC
The Allan M. Fox Trust (U/A/D April 21, 2015)
The Allan M. Fox Revocable Trust
The Kiser 2012 Gift Trust
FoxKiser LLP
Beacon Bioventures Fund III LP
Venrock Healthcare Capital Partners II, L.P.
VHCP CO-Investment Holdings II, LLC
Venrock Associates VII, L.P.
Venrock Partners VII, L.P.
Brookside Capital Partners Fund, L.P.
Deerfield Private Design Fund III, L.P.
GFO II, LLC
Kerry S. Propper

Kenneth T. Mills
David B. Schmickel
William Edward Holtz
Vittal Vasista
Donald J. Hayden, Jr.
Matthew M. Rosini
G&H Partners

SCHEDULE B

SCHEDULE OF COMMON HOLDERS

Common Holder

ReGenX Holdings, LLC
James M. Wilson
The Trustees of the University of Pennsylvania
GlaxoSmithKline LLC
ARIAD Pharmaceuticals
Kenneth T. Mills
Vittal Vasista
Sara Berl
Donald J. Hayden, Jr.
Stephen Yoo

INDEMNITY AGREEMENT

THIS INDEMNITY AGREEMENT (this "Agreement") dated as of _____, 2015, is made by and between REGENXBIO Inc., a Delaware corporation (the "Company"), and _____ ("Indemnitee").

RECITALS:

A. The Company desires to attract and retain the services of highly qualified individuals as directors, officers, employees and agents.

B. The Company's bylaws (the "Bylaws") require that the Company indemnify its directors, and empowers the Company to indemnify its officers, employees and agents, as authorized by the Delaware General Corporation Law, as amended (the "Code"), under which the Company is organized and such Bylaws expressly provide that the indemnification provided therein is not exclusive and contemplates that the Company may enter into separate agreements with its directors, officers and other persons to set forth specific indemnification provisions.

C. Indemnitee does not regard the protection currently provided by applicable law, the Company's governing documents and available insurance as adequate under the present circumstances, and the Company has determined that Indemnitee and other directors, officers, employees and agents of the Company may not be willing to serve or continue to serve in such capacities without additional protection.

D. The Company desires and has requested Indemnitee to serve or continue to serve as a director, officer, employee or agent of the Company, as the case may be, and has proffered this Agreement to Indemnitee as an additional inducement to serve in such capacity.

E. Indemnitee is willing to serve, or to continue to serve, as a director, officer, employee or agent of the Company, as the case may be, if Indemnitee is furnished the indemnity provided for herein by the Company.

F. [Indemnitee has certain rights to indemnification and/or insurance provided by _____ ("[Venture Fund]") which Indemnitee and [Venture Fund] intend to be secondary to the primary obligation of the Company to indemnify Indemnitee as provided herein, with the Company's acknowledgement and agreement to the foregoing being a material condition to Indemnitee's willingness to serve on the Company's Board of Directors].

AGREEMENT:

NOW THEREFORE, in consideration of the mutual covenants and agreements set forth herein, the parties hereto, intending to be legally bound, hereby agree as follows:

1. Definitions.

(a) Agent. For purposes of this Agreement, the term "agent" of the Company means any person who: (i) is or was a director, officer, employee or other fiduciary of the

Company or a subsidiary of the Company; or (ii) is or was serving at the request or for the convenience of, or representing the interests of, the Company or a subsidiary of the Company, as a director, officer, employee or other fiduciary of a foreign or domestic corporation, partnership, joint venture, trust or other enterprise.

(b) Expenses. For purposes of this Agreement, the term "expenses" shall be broadly construed and shall include, without limitation, all direct and indirect costs of any type or nature whatsoever (including, without limitation, all attorneys', witness, or other professional fees and related disbursements, and other out-of-pocket costs of whatever nature), actually and reasonably incurred by Indemnitee in connection with the investigation, defense or appeal of a proceeding or establishing or enforcing a right to indemnification under this Agreement, the Code or otherwise, and amounts paid in settlement by or on behalf of Indemnitee, but shall not include any judgments, fines or penalties actually levied against Indemnitee for such individual's violations of law. The term "expenses" shall also include reasonable compensation for time spent by Indemnitee for which he is not compensated by the Company or any subsidiary or third party (i) for any period during which Indemnitee is not an agent, in the employment of, or providing services for compensation to, the Company or any subsidiary; and (ii) if the rate of compensation and estimated time involved is approved by the directors of the Company who are not parties to any action with respect to which expenses are incurred, for Indemnitee while an agent of, employed by, or providing services for compensation to, the Company or any subsidiary.

(c) Proceedings. For purposes of this Agreement, the term "proceeding" shall be broadly construed and shall include, without limitation, any threatened, pending, or completed action, suit, arbitration, alternate dispute resolution mechanism, investigation, inquiry, administrative hearing or any other actual, threatened or completed proceeding, whether brought in the right of the Company or otherwise and whether of a civil, criminal, administrative or investigative nature, and whether formal or informal in any case, in which Indemnitee was, is or will be involved as a party or otherwise by reason of: (i) the fact that Indemnitee is or was a director or officer of the Company; (ii) the fact that any action taken by Indemnitee or of any action on Indemnitee's part while acting as director, officer, employee or agent of the Company; or (iii) the fact that Indemnitee is or was serving at the request of the Company as a director, officer, employee or agent of another corporation, partnership, joint venture, trust, employee benefit plan or other enterprise, and in any such case described above, whether or not serving in any such capacity at the time any liability or expense is incurred for which indemnification, reimbursement, or advancement of expenses may be provided under this Agreement.

(d) Subsidiary. For purposes of this Agreement, the term "subsidiary" means any corporation or limited liability company of which more than 50% of the outstanding voting securities or equity interests are owned, directly or indirectly, by the Company and one or more of its subsidiaries, and any other corporation, limited liability company, partnership, joint venture, trust, employee benefit plan or other enterprise of which Indemnitee is or was serving at the request of the Company as a director, officer, employee, agent or fiduciary.

(e) Independent Counsel. For purposes of this Agreement, the term "independent counsel" means a law firm, or a partner (or, if applicable, member) of such a law firm, that is experienced in matters of corporation law and neither presently is, nor in the past

five (5) years has been, retained to represent: (i) the Company or Indemnitee in any matter material to either such party, or (ii) any other party to the proceeding giving rise to a claim for indemnification hereunder. Notwithstanding the foregoing, the term "independent counsel" shall not include any person who, under the applicable standards of professional conduct then prevailing, would have a conflict of interest in representing either the Company or Indemnitee in an action to determine Indemnitee's rights under this Agreement.

2. Agreement to Serve. Indemnitee will serve, or continue to serve, as a director, officer, employee or agent of the Company or any subsidiary, as the case may be, faithfully and to the best of his or her ability, at the will of such corporation (or under separate agreement, if such agreement exists), in the capacity Indemnitee currently serves as an agent of such corporation, so long as Indemnitee is duly appointed or elected and qualified in accordance with the applicable provisions of the Bylaws or other applicable charter documents of such corporation, or until such time as Indemnitee tenders his or her resignation in writing; provided, however, that nothing contained in this Agreement is intended as an employment agreement between Indemnitee and the Company or any of its subsidiaries or to create any right to continued employment of Indemnitee with the Company or any of its subsidiaries in any capacity.

The Company acknowledges that it has entered into this Agreement and assumes the obligations imposed on it hereby, in addition to and separate from its obligations to Indemnitee under the Bylaws, to induce Indemnitee to serve, or continue to serve, as a director, officer, employee or agent of the Company, and the Company acknowledges that Indemnitee is relying upon this Agreement in serving as a director, officer, employee or agent of the Company.

3. Indemnification.

(a) Indemnification in Third Party Proceedings. Subject to Section 10 below, the Company shall indemnify Indemnitee to the fullest extent permitted by the Code, as the same may be amended from time to time (but, only to the extent that such amendment permits Indemnitee to broader indemnification rights than the Code permitted prior to adoption of such amendment), if Indemnitee is a party to or threatened to be made a party to or otherwise involved in any proceeding, for any and all expenses, actually and reasonably incurred by Indemnitee in connection with the investigation, defense, settlement or appeal of such proceeding.

(b) Indemnification in Derivative Actions and Direct Actions by the Company. Subject to Section 10 below, the Company shall indemnify Indemnitee to the fullest extent permitted by the Code, as the same may be amended from time to time (but, only to the extent that such amendment permits Indemnitee to broader indemnification rights than the Code permitted prior to adoption of such amendment), if Indemnitee is a party to or threatened to be made a party to or otherwise involved in any proceeding by or in the right of the Company to procure a judgment in its favor, against any and all expenses actually and reasonably incurred by Indemnitee in connection with the investigation, defense, settlement, or appeal of such proceedings.

4. Indemnification of Expenses of Successful Party. Notwithstanding any other provision of this Agreement, to the extent that Indemnitee has been successful on the merits or otherwise in defense of any proceeding or in defense of any claim, issue or matter therein, including the dismissal of any action without prejudice, the Company shall indemnify Indemnitee against all expenses actually and reasonably incurred in connection with the investigation, defense or appeal of such proceeding.

5. Partial Indemnification. If Indemnitee is entitled under any provision of this Agreement to indemnification by the Company for some or a portion of any expenses actually and reasonably incurred by Indemnitee in the investigation, defense, settlement or appeal of a proceeding, but is precluded by applicable law or the specific terms of this Agreement to indemnification for the total amount thereof, the Company shall nevertheless indemnify Indemnitee for the portion thereof to which Indemnitee is entitled.

6. Advancement of Expenses. To the extent not prohibited by law, the Company shall advance the expenses incurred by Indemnitee in connection with any proceeding, and such advancement shall be made within twenty (20) days after the receipt by the Company of a statement or statements requesting such advances (which shall include invoices received by Indemnitee in connection with such expenses but, in the case of invoices in connection with legal services, any references to legal work performed or to expenditures made that would cause Indemnitee to waive any privilege accorded by applicable law shall not be included with the invoice) and upon request of the Company, an undertaking to repay the advancement of expenses if and to the extent that it is ultimately determined by a court of competent jurisdiction in a final judgment, not subject to appeal, that Indemnitee is not entitled to be indemnified by the Company. Advances shall be unsecured, interest free and without regard to Indemnitee's ability to repay the expenses. Advances shall include any and all expenses actually and reasonably incurred by Indemnitee pursuing an action to enforce Indemnitee's right to indemnification under this Agreement, or otherwise and this right of advancement, including expenses incurred preparing and forwarding statements to the Company to support the advances claimed. Indemnitee acknowledges that the execution and delivery of this Agreement shall constitute an undertaking providing that Indemnitee shall, to the fullest extent required by law, repay the advance if and to the extent that it is ultimately determined by a court of competent jurisdiction in a final judgment, not subject to appeal, that Indemnitee is not entitled to be indemnified by the Company. The right to advances under this Section shall continue until final disposition of any proceeding, including any appeal therein. This Section 6 shall not apply to any claim made by Indemnitee for which indemnity is excluded pursuant to Section 10(b).

7. Notice and Other Indemnification Procedures.

(a) Notification of Proceeding. Indemnitee will notify the Company in writing promptly upon being served with any summons, citation, subpoena, complaint, indictment, information or other document relating to any proceeding or matter which may be subject to indemnification or advancement of expenses covered hereunder. The failure of Indemnitee to so notify the Company shall not relieve the Company of any obligation which it may have to Indemnitee under this Agreement or otherwise.

(b) Request for Indemnification and Indemnification Payments. Indemnitee shall notify the Company promptly in writing upon receiving notice of nay demand, judgment or other requirement for payment that Indemnitee reasonably believes to the subject to

indemnification under the terms of this Agreement, and shall request payment thereof by the Company. Indemnification payments requested by Indemnitee under Section 3 hereof shall be made by the Company no later than sixty (60) days after receipt of the written request of Indemnitee. Claims for advancement of expenses shall be made under the provisions of Section 6 herein.

(c) Application for Enforcement. In the event the Company fails to make timely payments as set forth in Sections 6 or 7(b) above, Indemnitee shall have the right to apply to any court of competent jurisdiction for the purpose of enforcing Indemnitee's right to indemnification or advancement of expenses pursuant to this Agreement. In such an enforcement hearing or proceeding, the burden of proof shall be on the Company to prove by that indemnification or advancement of expenses to Indemnitee is not required under this Agreement or permitted by applicable law. Any determination by the Company (including its Board of Directors, stockholders or independent counsel) that Indemnitee is not entitled to indemnification hereunder, shall not be a defense by the Company to the action nor create any presumption that Indemnitee is not entitled to indemnification or advancement of expenses hereunder.

(d) Indemnification of Certain Expenses. The Company shall indemnify Indemnitee against all expenses incurred in connection with any hearing or proceeding under this Section 7 unless the Company prevails in such hearing or proceeding on the merits in all material respects.

8. Assumption of Defense. In the event the Company shall be requested by Indemnitee to pay the expenses of any proceeding, the Company, if appropriate, shall be entitled to assume the defense of such proceeding, or to participate to the extent permissible in such proceeding, with counsel reasonably acceptable to Indemnitee. Upon assumption of the defense by the Company and the retention of such counsel by the Company, the Company shall not be liable to Indemnitee under this Agreement for any fees of counsel subsequently incurred by Indemnitee with respect to the same proceeding, provided that Indemnitee shall have the right to employ separate counsel in such proceeding at Indemnitee's sole cost and expense. Notwithstanding the foregoing, if Indemnitee's counsel delivers a written notice to the Company stating that such counsel has reasonably concluded that there may be a conflict of interest between the Company and Indemnitee in the conduct of any such defense or the Company shall not, in fact, have employed counsel or otherwise actively pursued the defense of such proceeding within a reasonable time, then in any such event the fees and expenses of Indemnitee's counsel to defend such proceeding shall be subject to the indemnification and advancement of expenses provisions of this Agreement.

9. Insurance. To the extent that the Company maintains an insurance policy or policies providing liability insurance for directors, officers, employees, or agents of the Company or of any subsidiary ("D&O Insurance"), Indemnitee shall be covered by such policy or policies in accordance with its or their terms to the maximum extent of the coverage available for any such director, officer, employee or agent under such policy or policies. If, at the time of the receipt of a notice of a claim pursuant to the terms hereof, the Company has D&O Insurance in effect, the Company shall give prompt notice of the commencement of such proceeding to the insurers in accordance with the procedures set forth in the respective policies. The Company shall thereafter take all necessary or desirable action to cause such insurers to pay, on behalf of Indemnitee, all amounts payable as a result of such proceeding in accordance with the terms of such policies.

10. Exceptions.

(a) Certain Matters. Any provision herein to the contrary notwithstanding, the Company shall not be obligated pursuant to the terms of this Agreement to indemnify Indemnitee on account of any proceeding with respect to (i) remuneration paid to Indemnitee if it is determined by final judgment or other final adjudication that such remuneration was in violation of law (and, in this respect, both the Company and Indemnitee have been advised that the Securities and Exchange Commission believes that indemnification for liabilities arising under the federal securities laws is against public policy and is, therefore, unenforceable and that claims for indemnification should be submitted to appropriate courts for adjudication, as indicated in Section 10(d) below); (ii) a final judgment rendered against Indemnitee for an accounting, disgorgement or repayment of profits made from the purchase or sale by Indemnitee of securities of the Company against Indemnitee or in connection with a settlement by or on behalf of Indemnitee to the extent it is acknowledged by Indemnitee and the Company that such amount paid in settlement resulted from Indemnitee's conduct from which Indemnitee received monetary personal profit pursuant to the provisions of Section 16(b) of the Securities Exchange Act of 1934, as amended, or other provisions of any federal, state or local statute or rules and regulations thereunder; (iii) a final judgment or other final adjudication that Indemnitee's conduct was in bad faith, knowingly fraudulent or deliberately dishonest or constituted willful misconduct (but only to the extent of such specific determination); or (iv) on account of conduct that is established by a final judgment as constituting a breach of Indemnitee's duty of loyalty to the Company or resulting in any personal profit or advantage to which Indemnitee is not legally entitled. For purposes of the foregoing sentence, a final judgment or other adjudication may be reached in either the underlying proceeding or action in connection with which indemnification is sought or a separate proceeding or action to establish rights and liabilities under this Agreement.

(b) Claims Initiated by Indemnitee. Any provision herein to the contrary notwithstanding, the Company shall not be obligated to indemnify or advance expenses to Indemnitee with respect to proceedings or claims initiated or brought by Indemnitee against the Company or its directors, officers, employees or other agents and not by way of defense, except (i) with respect to proceedings brought to establish or enforce a right to indemnification under this Agreement or under any other agreement, provision in the Bylaws or Certificate of Incorporation or applicable law, or (ii) with respect to any other proceeding initiated by Indemnitee that is either approved by the Board of Directors or Indemnitee's participation is required by applicable law. However, indemnification or advancement of expenses may be provided by the Company in specific cases if the Board of Directors determines it to be appropriate.

(c) Unauthorized Settlements. Any provision herein to the contrary notwithstanding, the Company shall not be obligated pursuant to the terms of this Agreement to indemnify Indemnitee under this Agreement for any amounts paid in settlement of a proceeding effected without the Company's written consent. Neither the Company nor Indemnitee shall

unreasonably withhold consent to any proposed settlement; provided, however, that the Company may in any event decline to consent to (or to otherwise admit or agree to any liability for indemnification hereunder in respect of) any proposed settlement if the Company is also a party in such proceeding and determines in good faith that such settlement is not in the best interests of the Company and its stockholders.

(d) Securities Act Liabilities. Any provision herein to the contrary notwithstanding, the Company shall not be obligated pursuant to the terms of this Agreement to indemnify Indemnitee or otherwise act in violation of any undertaking appearing in and required by the rules and regulations promulgated under the Securities Act of 1933, as amended (the "Act"), or in any registration statement filed with the SEC under the Act. Indemnitee acknowledges that paragraph (h) of Item 512 of Regulation S-K currently generally requires the Company to undertake in connection with any registration statement filed under the Act to submit the issue of the enforceability of Indemnitee's rights under this Agreement in connection with any liability under the Act on public policy grounds to a court of appropriate jurisdiction and to be governed by any final adjudication of such issue. Indemnitee specifically agrees that any such undertaking shall supersede the provisions of this Agreement and to be bound by any such undertaking.

11. Nonexclusivity; Priority of Payment and Survival of Rights.

(a) The provisions for indemnification and advancement of expenses set forth in this Agreement shall not be deemed exclusive of any other rights which Indemnitee may at any time be entitled under any provision of applicable law, the Company's Certificate of Incorporation, Bylaws or other agreements, both as to action in Indemnitee's official capacity and Indemnitee's action as an agent of the Company, in any court in which a proceeding is brought, and Indemnitee's rights hereunder shall continue after Indemnitee has ceased acting as an agent of the Company and shall inure to the benefit of the heirs, executors, administrators and assigns of Indemnitee. The obligations and duties of the Company to Indemnitee under this Agreement shall be binding on the Company and its successors and assigns until terminated in accordance with its terms. The Company shall require any successor (whether direct or indirect, by purchase, merger, consolidation or otherwise) to all or substantially all of the business or assets of the Company, expressly to assume and agree to perform this Agreement in the same manner and to the same extent that the Company would be required to perform if no such succession had taken place.

(b) The Company hereby acknowledges that Indemnitee has certain rights to indemnification, advancement of expenses and/or insurance provided by [Venture Fund] and certain of its affiliates (collectively, the "Fund Indemnitors"). The Company hereby agrees (i) that it is the indemnitor of first resort (i.e., its obligations to Indemnitee are primary and any obligation of the Fund Indemnitors to advance expenses or to provide indemnification for the same expenses or liabilities incurred by Indemnitee are secondary), (ii) that it shall be required to advance the full amount of expenses incurred by Indemnitee and shall be liable for the full amount of all Expenses, judgments, penalties, fines and amounts paid in settlement to the extent legally permitted and as required by the terms of this Agreement and the Certificate of Incorporation or Bylaws of the Company (or any other agreement between the Company and Indemnitee), without regard to any rights Indemnitee may have against the Fund Indemnitors,

and, (iii) that it irrevocably waives, relinquishes and releases the Fund Indemnitors from any and all claims against the Fund Indemnitors for contribution, subrogation or any other recovery of any kind in respect thereof. The Company further agrees that no advancement or payment by the Fund Indemnitors on behalf of Indemnitee with respect to any claim for which Indemnitee has sought indemnification from the Company shall affect the foregoing and the Fund Indemnitors shall have a right of contribution and/or be subrogated to the extent of such advancement or payment to all of the rights of recovery of Indemnitee against the Company. The Company and Indemnitee agree that the Fund Indemnitors are express third party beneficiaries of the terms of this Section 11(b).

(c) No amendment, alteration or repeal of this Agreement or of any provision hereof shall limit or restrict any right of Indemnitee under this Agreement in respect of any action taken or omitted by such Indemnitee in his or her corporate status prior to such amendment, alteration or repeal. To the extent that a change in the Code, whether by statute or judicial decision, permits greater indemnification or advancement of expenses than would be afforded currently under the Company's Certificate of Incorporation, Bylaws and this Agreement, it is the intent of the parties hereto that Indemnitee shall enjoy by this Agreement the greater benefits so afforded by such change. No right or remedy herein conferred is intended to be exclusive of any other right or remedy, and every other right and remedy shall be cumulative and in addition to every other right and remedy given hereunder or now or hereafter existing at law or in equity or otherwise. The assertion or employment of any right or remedy hereunder, or otherwise, by Indemnitee shall not prevent the concurrent assertion or employment of any other right or remedy by Indemnitee.

12. Term. This Agreement shall continue until and terminate upon the later of: (a) five (5) years after the date that Indemnitee shall have ceased to serve as a director or and/or officer, employee or agent of the Company; or (b) one (1) year after the final termination of any proceeding, including any appeal then pending, in respect to which Indemnitee was granted rights of indemnification or advancement of expenses hereunder.

No legal action shall be brought and no cause of action shall be asserted by or in the right of the Company against an Indemnitee or an Indemnitee's estate, spouse, heirs, executors or personal or legal representatives after the expiration of five (5) years from the date of accrual of such cause of action, and any claim or cause of action of the Company shall be extinguished and deemed released unless asserted by the timely filing of a legal action within such five-year period; provided, however, that if any shorter period of limitations is otherwise applicable to such cause of action, such shorter period shall govern.

13. Subrogation. Except as provided in Section 11(b) above, in the event of any payment under this Agreement, the Company shall be subrogated to the extent of such payment to all of the rights of recovery of Indemnitee (other than against the Fund Indemnitor), who, at the request and expense of the Company, shall execute all papers required and shall do everything that may be reasonably necessary to secure such rights, including the execution of such documents necessary to enable the Company effectively to bring suit to enforce such rights.

14. Interpretation of Agreement. It is understood that the parties hereto intend this Agreement to be interpreted and enforced so as to provide indemnification to Indemnitee to the fullest extent now or hereafter permitted by law.

15. Severability. If any provision of this Agreement shall be held to be invalid, illegal or unenforceable for any reason whatsoever, (a) the validity, legality and enforceability of the remaining provisions of the Agreement (including without limitation, all portions of any paragraphs of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that are not themselves invalid, illegal or unenforceable) shall not in any way be affected or impaired thereby; and (b) to the fullest extent possible, the provisions of this Agreement (including, without limitation, all portions of any paragraph of this Agreement containing any such provision held to be invalid, illegal or unenforceable, that are not themselves invalid, illegal or unenforceable) shall be construed so as to give effect to the intent manifested by the provision held invalid, illegal or unenforceable and to give effect to Section 14 hereof.

16. Amendment and Waiver. No supplement, modification, amendment, or cancellation of this Agreement shall be binding unless executed in writing by the parties hereto. No waiver of any of the provisions of this Agreement shall be deemed or shall constitute a waiver of any other provision hereof (whether or not similar) nor shall such waiver constitute a continuing waiver.

17. Notice. Except as otherwise provided herein, any notice or demand which, by the provisions hereof, is required or which may be given to or served upon the parties hereto shall be in writing and, if by telegram, telecopy or telex, shall be deemed to have been validly served, given or delivered when sent, if by overnight delivery, courier or personal delivery, shall be deemed to have been validly served, given or delivered upon actual delivery and, if mailed, shall be deemed to have been validly served, given or delivered three (3) business days after deposit in the United States mail, as registered or certified mail, with proper postage prepaid and addressed to the party or parties to be notified at the addresses set forth on the signature page of this Agreement (or such other address(es) as a party may designate for itself by like notice). If to the Company, notices and demands shall be delivered to the attention of the Secretary of the Company.

18. Governing Law. This Agreement shall be governed exclusively by and construed according to the laws of the Commonwealth of Massachusetts, as applied to contracts between Massachusetts residents entered into and to be performed entirely within Massachusetts.

19. Counterparts. This Agreement may be executed in one or more counterparts, each of which shall for all purposes be deemed to be an original but all of which together shall constitute but one and the same Agreement. Only one such counterpart need be produced to evidence the existence of this Agreement.

20. Headings. The headings of the sections of this Agreement are inserted for convenience only and shall not be deemed to constitute part of this Agreement or to affect the construction hereof.

21. Entire Agreement. This Agreement constitutes the entire agreement between the parties with respect to the subject matter hereof and supersedes all prior agreements, understandings and negotiations, written and oral, between the parties with respect to the subject matter of this Agreement; provided, however, that this Agreement is a supplement to and in furtherance of the Company's Certificate of Incorporation, Bylaws, the Code and any other applicable law, and shall not be deemed a substitute therefor, and does not diminish or abrogate any rights of Indemnitee thereunder.

22. Amendment and Restatement of Prior Agreement. Upon the effectiveness of this Agreement, the Prior Agreement shall be amended and restated in its entirety and be of no further force and effect, and shall be superseded and replaced in its entirety by this Agreement.

IN WITNESS WHEREOF, the parties hereto have entered into this Agreement effective as of the date first above written.

COMPANY

REGENXBIO INC.

By: _____
Name: _____
Title: _____

INDEMNITEE

_____
[Name]

Address:

REGENXBIO Inc.

2014 STOCK PLAN

ADOPTED ON SEPTEMBER 16, 2014
AMENDED ON JANUARY 8, 2015
AMENDED ON MAY 14, 2015

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REGENXBIO INC. 2014 STOCK PLAN

SECTION 1. ESTABLISHMENT AND PURPOSE.

The purpose of this Plan is to offer persons selected by the Company an opportunity to acquire a proprietary interest in the success of the Company, or to increase such interest, by acquiring Shares of the Company's Stock. The Plan provides both for the direct award or sale of Shares and for the grant of Options to purchase Shares. Options granted under the Plan may be ISOs intended to qualify under Code Section 422 or Nonstatutory Options which are not intended to so qualify.

Capitalized terms are defined in Section 12.

SECTION 2. ADMINISTRATION.

(a) Committees of the Board of Directors. The Plan may be administered by one or more Committees. Each Committee shall consist, as required by applicable law, of one or more members of the Board of Directors who have been appointed by the Board of Directors. Each Committee shall have such authority and be responsible for such functions as the Board of Directors has assigned to it. If no Committee has been appointed, the entire Board of Directors shall administer the Plan. Any reference to the Board of Directors in the Plan shall be construed as a reference to the Committee (if any) to whom the Board of Directors has assigned a particular function.

(b) Authority of the Board of Directors. Subject to the provisions of the Plan, the Board of Directors shall have full authority and discretion to take any actions it deems necessary or advisable for the administration of the Plan. Notwithstanding anything to the contrary in the Plan, with respect to the terms and conditions of awards granted to Participants outside the United States, the Board of Directors may vary from the provisions of the Plan to the extent it determines it necessary and appropriate to do so; provided that it may not vary from those Plan terms requiring stockholder approval pursuant to Section 11(d) below. All decisions, interpretations and other actions of the Board of Directors shall be final and binding on all Purchasers, all Optionees and all persons deriving their rights from a Purchaser or Optionee.

SECTION 3. ELIGIBILITY.

(a) General Rule. Only Employees, Outside Directors and Consultants shall be eligible for the grant of Nonstatutory Options or the direct award or sale of Shares. Only Employees shall be eligible for the grant of ISOs.

(b) Ten-Percent Stockholders. A person who owns more than 10% of the total combined voting power of all classes of outstanding stock of the Company, its Parent or any of its Subsidiaries shall not be eligible for the grant of an ISO unless (i) the Exercise Price is at least 110% of the Fair Market Value of a Share on the Date of Grant and (ii) such ISO by its terms is not exercisable after the expiration of five years from the Date of Grant. For purposes of this Subsection (b), in determining stock ownership, the attribution rules of Code Section 424(d) shall be applied.

SECTION 4. STOCK SUBJECT TO PLAN.

(a) Basic Limitation. Not more than 2,500,000 Shares may be issued under the Plan, subject to Subsection (b) below and Section 8(a).[1] All of these Shares may be issued upon the exercise of ISOs. The number of Shares that are subject to Options or other rights outstanding at any time under the Plan may not exceed the number of Shares that then remain available for issuance under the Plan. The Company, during the term of the Plan, shall at all times reserve and keep available sufficient Shares to satisfy the requirements of the Plan. Shares offered under the Plan may be authorized but unissued Shares or treasury Shares.

(b) Additional Shares. In the event that Shares previously issued under the Plan are reacquired by the Company, such Shares shall be added to the number of Shares then available for issuance under the Plan. In the event that Shares that otherwise would have been issuable under the Plan are withheld by the Company in payment of the Purchase Price, Exercise Price or withholding taxes, such Shares shall remain available for issuance under the Plan. In the event that an outstanding Option or other right for any reason expires or is canceled, the Shares allocable to the unexercised portion of such Option or other right shall be added to the number of Shares then available for issuance under the Plan.

SECTION 5. TERMS AND CONDITIONS OF AWARDS OR SALES.

(a) Stock Grant or Purchase Agreement. Each award of Shares under the Plan shall be evidenced by a Stock Grant Agreement between the Grantee and the Company. Each sale of Shares under the Plan (other than upon exercise of an Option) shall be evidenced by a Stock Purchase Agreement between the Purchaser and the Company. Such award or sale shall be subject to all applicable terms and conditions of the Plan and may be subject to any other terms and conditions which are not inconsistent with the Plan and which the Board of Directors deems appropriate for inclusion in a Stock Grant Agreement or Stock Purchase Agreement. The provisions of the various Stock Grant Agreements and Stock Purchase Agreements entered into under the Plan need not be identical.

(b) Duration of Offers and Nontransferability of Rights. Any right to purchase Shares under the Plan (other than an Option) shall automatically expire if not exercised by the Purchaser within 30 days (or such other period as may be specified in the Award Agreement) after the grant of such right was communicated to the Purchaser by the Company. Such right is not transferable and may be exercised only by the Purchaser to whom such right was granted.

(c) Purchase Price. The Board of Directors shall determine the Purchase Price of Shares to be offered under the Plan at its sole discretion. The Purchase Price shall be payable in a form described in Section 7.

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1 Please refer to Exhibit A for a schedule of the initial share reserve and any subsequent increases in the reserve.

SECTION 6. TERMS AND CONDITIONS OF OPTIONS.

(a) Stock Option Agreement. Each grant of an Option under the Plan shall be evidenced by a Stock Option Agreement between the Optionee and the Company. The Option shall be subject to all applicable terms and conditions of the Plan and may be subject to any other terms and conditions that are not inconsistent with the Plan and that the Board of Directors deems appropriate for inclusion in a Stock Option Agreement. The provisions of the various Stock Option Agreements entered into under the Plan need not be identical.

(b) Number of Shares. Each Stock Option Agreement shall specify the number of Shares that are subject to the Option and shall provide for the adjustment of such number in accordance with Section 8. The Stock Option Agreement shall also specify whether the Option is an ISO or a Nonstatutory Option.

(c) Exercise Price. Each Stock Option Agreement shall specify the Exercise Price. The Exercise Price of an Option shall not be less than 100% of the Fair Market Value of a Share on the Date of Grant, and in the case of an ISO a higher percentage may be required by Section 3(b). Subject to the preceding sentence, the Exercise Price shall be determined by the Board of Directors at its sole discretion. The Exercise Price shall be payable in a form described in Section 7. This Subsection (c) shall not apply to an Option granted pursuant to an assumption of, or substitution for, another option in a manner that complies with Code Section 424(a) (whether or not the Option is an ISO).

(d) Exercisability. Each Stock Option Agreement shall specify the date when all or any installment of the Option is to become exercisable. No Option shall be exercisable unless the Optionee (i) has delivered an executed copy of the Stock Option Agreement to the Company or (ii) otherwise agrees to be bound by the terms of the Stock Option Agreement. The Board of Directors shall determine the exercisability provisions of the Stock Option Agreement at its sole discretion.

(e) Basic Term. The Stock Option Agreement shall specify the term of the Option. The term shall not exceed 10 years from the Date of Grant, and in the case of an ISO, a shorter term may be required by Section 3(b). Subject to the preceding sentence, the Board of Directors at its sole discretion shall determine when an Option is to expire.

(f) Termination of Service (Except by Death). If an Optionee's Service terminates for any reason other than the Optionee's death, then the Optionee's Options shall expire on the earliest of the following dates:

(i) The expiration date determined pursuant to Subsection (e) above;

(ii) The date three months after the termination of the Optionee's Service for any reason other than Disability, or such earlier or later date as the Board of Directors may determine (but in no event earlier than 30 days after the termination of the Optionee's Service); or

(iii) The date six months after the termination of the Optionee's Service by reason of Disability, or such later date as the Board of Directors may determine.

The Optionee may exercise all or part of the Optionee's Options at any time before the expiration of such Options under the preceding sentence, but only to the extent that such Options had become exercisable before the Optionee's Service terminated (or became exercisable as a result of the termination) and the underlying Shares had vested before the Optionee's Service terminated (or vested as a result of the termination). The balance of such Options shall lapse when the Optionee's Service terminates. In the event that the Optionee dies after the termination of the Optionee's Service but before the expiration of the Optionee's Options, all or part of such Options may be exercised (prior to expiration) by the executors or administrators of the Optionee's estate or by any person who has acquired such Options directly from the Optionee by beneficiary designation, bequest or inheritance, but only to the extent that such Options had become exercisable before the Optionee's Service terminated (or became exercisable as a result of the termination) and the underlying Shares had vested before the Optionee's Service terminated (or vested as a result of the termination).

(g) Leaves of Absence. For purposes of Subsection (f) above, Service shall be deemed to continue while the Optionee is on a bona fide leave of absence, if such leave was approved by the Company in writing and if continued crediting of Service for this purpose is expressly required by the terms of such leave or by applicable law (as determined by the Company).

(h) Death of Optionee. If an Optionee dies while the Optionee is in Service, then the Optionee's Options shall expire on the earlier of the following dates:

(i) The expiration date determined pursuant to Subsection (e) above; or

(ii) The date 12 months after the Optionee's death, or such earlier or later date as the Board of Directors may determine (but in no event earlier than six months after the Optionee's death).

All or part of the Optionee's Options may be exercised at any time before the expiration of such Options under the preceding sentence by the executors or administrators of the Optionee's estate or by any person who has acquired such Options directly from the Optionee by beneficiary designation, bequest or inheritance, but only to the extent that such Options had become exercisable before the Optionee's death (or became exercisable as a result of the death) and the underlying Shares had vested before the Optionee's death (or vested as a result of the Optionee's death). The balance of such Options shall lapse when the Optionee dies.

(i) Pre-Exercise Restrictions on Transfer of Options or Shares. An Option shall be transferable by the Optionee only by (i) a beneficiary designation, (ii) a will or (iii) the laws of descent and distribution, except as provided in the next sentence. If the applicable Stock Option Agreement so provides, a Nonstatutory Option shall also be transferable by gift or domestic relations order to a Family Member of the Optionee. An ISO may be

exercised during the lifetime of the Optionee only by the Optionee or by the Optionee's guardian or legal representative. In addition, an Option shall comply with all conditions of Rule 12h-1(f)(1) under the Exchange Act until the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act. Such conditions include, without limitation, the transferability restrictions set forth in Rule 12h-1(f)(1)(iv) and (v) under the Exchange Act, which shall apply to an Option and, prior to exercise, to the Shares to be issued upon exercise of such Option during the period commencing on the Date of Grant and ending on the earlier of (i) the date when the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act or (ii) the date when the Company makes a determination that it will cease to rely on the exemption afforded by Rule 12h-1(f)(1) under the Exchange Act. During such period, an Option and, prior to exercise, the Shares to be issued upon exercise of such Option shall be restricted as to any pledge, hypothecation or other transfer by the Optionee, including any short position, any "put equivalent position" (as defined in Rule 16a-1(h) under the Exchange Act) or any "call equivalent position" (as defined in Rule 16a-1(b) under the Exchange Act).

(j) No Rights as a Stockholder. An Optionee, or a transferee of an Optionee, shall have no rights as a stockholder with respect to any Shares covered by the Optionee's Option until such person becomes entitled to receive such Shares by filing a notice of exercise and paying the Exercise Price pursuant to the terms of such Option.

(k) Modification, Extension and Assumption of Options. Within the limitations of the Plan, the Board of Directors may modify, extend or assume outstanding Options or may accept the cancellation of outstanding Options (whether granted by the Company or another issuer) in return for the grant of new Options or a different type of award for the same or a different number of Shares and at the same or a different Exercise Price (if applicable). The foregoing notwithstanding, no modification of an Option shall, without the consent of the Optionee, impair the Optionee's rights or increase the Optionee's obligations under such Option.

(l) Company's Right to Cancel Certain Options. Any other provision of the Plan or a Stock Option Agreement notwithstanding, the Company shall have the right at any time to cancel an Option that was not granted in compliance with Rule 701 under the Securities Act. Prior to canceling such Option, the Company shall give the Optionee not less than 30 days' notice in writing. If the Company elects to cancel such Option, it shall deliver to the Optionee consideration with an aggregate Fair Market Value equal to the excess of (i) the Fair Market Value of the Shares subject to such Option as of the time of the cancellation over (ii) the Exercise Price of such Option. The consideration may be delivered in the form of cash or cash equivalents, in the form of Shares, or a combination of both. If the consideration would be a negative amount, such Option may be cancelled without the delivery of any consideration.

SECTION 7. PAYMENT FOR SHARES.

(a) General Rule. The entire Purchase Price or Exercise Price of Shares issued under the Plan shall be payable in cash or cash equivalents at the time when such Shares are purchased, except as otherwise provided in this Section 7. In addition, the Board of Directors in its sole discretion may also permit payment through any of the methods described in (b) through (g) below:

(b) Services Rendered. Shares may be awarded under the Plan in consideration of services rendered to the Company, a Parent or a Subsidiary prior to the award.

(c) Promissory Note. All or a portion of the Purchase Price or Exercise Price (as the case may be) of Shares issued under the Plan may be paid with a full-recourse promissory note. The Shares shall be pledged as security for payment of the principal amount of the promissory note and interest thereon. The interest rate payable under the terms of the promissory note shall not be less than the minimum rate (if any) required to avoid the imputation of additional interest under the Code. Subject to the foregoing, the Board of Directors (at its sole discretion) shall specify the term, interest rate, amortization requirements (if any) and other provisions of such note.

(d) Surrender of Stock. All or any part of the Exercise Price may be paid by surrendering, or attesting to the ownership of, Shares that are already owned by the Optionee. Such Shares shall be surrendered to the Company in good form for transfer and shall be valued at their Fair Market Value as of the date when the Option is exercised.

(e) Exercise/Sale. If the Stock is publicly traded, all or part of the Exercise Price and any withholding taxes may be paid by the delivery (on a form prescribed by the Company) of an irrevocable direction to a securities broker approved by the Company to sell Shares and to deliver all or part of the sales proceeds to the Company.

(f) Net Exercise. An Option may permit exercise through a "net exercise" arrangement pursuant to which the Company will reduce the number of Shares issued upon exercise by the largest whole number of Shares having an aggregate Fair Market Value (determined by the Board of Directors as of the exercise date) that does not exceed the aggregate Exercise Price or the sum of the aggregate Exercise Price plus all or a portion of the minimum amount required to be withheld under applicable tax law (with the Company accepting from the Optionee payment of cash or cash equivalents to satisfy any remaining balance of the aggregate Exercise Price and, if applicable, any additional withholding obligation not satisfied through such reduction in Shares); *provided* that to the extent Shares subject to an Option are withheld in this manner, the number of Shares subject to the Option following the net exercise will be reduced by the sum of the number of Shares withheld and the number of Shares delivered to the Optionee as a result of the exercise.

(g) Other Forms of Payment. To the extent that an Award Agreement so provides, the Purchase Price or Exercise Price of Shares issued under the Plan may be paid in any other form permitted by the Delaware General Corporation Law, as amended.

SECTION 8. ADJUSTMENT OF SHARES.

(a) General. In the event of a subdivision of the outstanding Stock, a declaration of a dividend payable in Shares, a combination or consolidation of the outstanding Stock into a lesser number of Shares, a reclassification, or any other increase or decrease in the number of issued shares of Stock effected without receipt of consideration by the Company, proportionate adjustments shall automatically be made in each of (i) the number and kind of Shares available for future grants under Section 4, (ii) the number and kind of Shares covered by

each outstanding Option and any outstanding and unexercised right to purchase Shares that has not yet expired pursuant to Section 5(b), (iii) the Exercise Price under each outstanding Option and the Purchase Price applicable to any unexercised stock purchase right described in clause (ii) above, and (iv) any repurchase price that applies to Shares granted under the Plan pursuant to the terms of a Company repurchase right under the applicable Award Agreement. In the event of a declaration of an extraordinary dividend payable in a form other than Shares in an amount that has a material effect on the Fair Market Value of the Stock, a recapitalization, a spin-off, or a similar occurrence, the Board of Directors at its sole discretion may make appropriate adjustments in one or more of the items listed in clauses (i) through (iv) above; provided, however, that the Board of Directors shall in any event make such adjustments as may be required by Section 25102(o) of the California Corporations Code. No fractional Shares shall be issued under the Plan as a result of an adjustment under this Section 8(a), although the Board of Directors in its sole discretion may make a cash payment in lieu of fractional Shares.

(b) Corporate Transactions. In the event that the Company is a party to a merger or consolidation, or in the event of a sale of all or substantially all of the Company's stock or assets, all Shares acquired under the Plan and all Options and other Plan awards outstanding on the effective date of the transaction shall be treated in the manner described in the definitive transaction agreement (or, in the event the transaction does not entail a definitive agreement to which the Company is party, in the manner determined by the Board of Directors in its capacity as administrator of the Plan, with such determination having final and binding effect on all parties), which agreement or determination need not treat all Options and awards (or all portions of an Option or an award) in an identical manner. The treatment specified in the transaction agreement may include (without limitation) one or more of the following with respect to each outstanding Option or award:

(i) Continuation of the Option or award by the Company (if the Company is the surviving corporation).

(ii) Assumption of the Option by the surviving corporation or its parent in a manner that complies with Code Section 424(a) (whether or not the Option is an ISO).

(iii) Substitution by the surviving corporation or its parent of a new option for the Option in a manner that complies with Code Section 424(a) (whether or not the Option is an ISO).

(iv) Cancellation of the Option and a payment to the Optionee with respect to each Share subject to the portion of the Option that is vested as of the transaction date equal to the excess of (A) the value, as determined by the Board of Directors in its absolute discretion, of the property (including cash) received by the holder of a share of Stock as a result of the transaction, over (B) the per-Share Exercise Price of the Option (such excess, the "Spread"). Such payment shall be made in the form of cash, cash equivalents, or securities of the surviving corporation or its parent having a value equal to the Spread. In addition, any escrow, holdback, earn-out or similar provisions in the transaction agreement may apply to such payment to the same extent and in the same manner as such provisions apply to the holders of Stock. If the Spread applicable to an Option is zero or a negative number, then the Option may be cancelled without making a payment to the Optionee.

(v) Cancellation of the Option without the payment of any consideration; provided that the Optionee shall be notified of such treatment and given an opportunity to exercise the Option (to the extent the Option is vested or becomes vested as of the effective date of the transaction) during a period of not less than five (5) business days preceding the effective date of the transaction, unless (A) a shorter period is required to permit a timely closing of the transaction and (B) such shorter period still offers the Optionee a reasonable opportunity to exercise the Option. Any exercise of the Option during such period may be contingent upon the closing of the transaction.

(vi) Suspension of the Optionee's right to exercise the Option during a limited period of time preceding the closing of the transaction if such suspension is administratively necessary to permit the closing of the transaction.

(vii) Termination of any right the Optionee has to exercise the Option prior to vesting in the Shares subject to the Option (i.e., "early exercise"), such that following the closing of the transaction the Option may only be exercised to the extent it is vested.

For the avoidance of doubt, the Board of Directors has discretion to accelerate, in whole or part, the vesting and exercisability of an Option or other Plan award in connection with a corporate transaction covered by this Section 8(b).

(c) Reservation of Rights. Except as provided in this Section 8, a Participant shall have no rights by reason of (i) any subdivision or consolidation of shares of stock of any class, (ii) the payment of any dividend or (iii) any other increase or decrease in the number of shares of stock of any class. Any issuance by the Company of shares of stock of any class, or securities convertible into shares of stock of any class, shall not affect, and no adjustment by reason thereof shall be made with respect to, the number or Exercise Price of Shares subject to an Option. The grant of an Option pursuant to the Plan shall not affect in any way the right or power of the Company to make adjustments, reclassifications, reorganizations or changes of its capital or business structure, to merge or consolidate or to dissolve, liquidate, sell or transfer all or any part of its business or assets.

SECTION 9. PRE-EXERCISE INFORMATION REQUIREMENT.

(a) Application of Requirement. This Section 9 shall apply only during a period that (i) commences when the Company begins to rely on the exemption described in Rule 12h-1(f)(1) under the Exchange Act, as determined by the Company in its sole discretion, and (ii) ends on the earlier of (A) the date when the Company ceases to rely on such exemption, as determined by the Company in its sole discretion, or (B) the date when the Company becomes subject to the reporting requirements of Section 13 or 15(d) of the Exchange Act. In addition, this Section 9 shall in no event apply to an Optionee after he or she has fully exercised all of his or her Options.

(b) Scope of Requirement. The Company shall provide to each Optionee the information described in Rule 701(e)(3), (4) and (5) under the Securities Act. Such information shall be provided at six-month intervals, and the financial statements included in such information shall not be more than 180 days old. The foregoing notwithstanding, the Company shall not be required to provide such information unless the Optionee has agreed in writing, on a form prescribed by the Company, to keep such information confidential.

SECTION 10. MISCELLANEOUS PROVISIONS.

(a) Securities Law Requirements. Shares shall not be issued under the Plan unless, in the opinion of counsel acceptable to the Board of Directors, the issuance and delivery of such Shares comply with (or are exempt from) all applicable requirements of law, including (without limitation) the Securities Act, the rules and regulations promulgated thereunder, state securities laws and regulations, and the regulations of any stock exchange or other securities market on which the Company's securities may then be traded. The Company shall not be liable for a failure to issue Shares as a result of such requirements.

(b) No Retention Rights. Nothing in the Plan or in any right or Option granted under the Plan shall confer upon the Participant any right to continue in Service for any period of specific duration or interfere with or otherwise restrict in any way the rights of the Company (or any Parent or Subsidiary employing or retaining the Participant) or of the Participant, which rights are hereby expressly reserved by each, to terminate his or her Service at any time and for any reason, with or without cause.

(c) Treatment as Compensation. Any compensation that an individual earns or is deemed to earn under this Plan shall not be considered a part of his or her compensation for purposes of calculating contributions, accruals or benefits under any other plan or program that is maintained or funded by the Company, a Parent or a Subsidiary.

(d) Governing Law. The Plan and all awards, sales and grants under the Plan shall be governed by, and construed in accordance with, the laws of the State of Delaware, as such laws are applied to contracts entered into and performed in such State.

(e) Conditions and Restrictions on Shares. Shares issued under the Plan shall be subject to such forfeiture conditions, rights of repurchase, rights of first refusal, other transfer restrictions and such other terms and conditions as the Board of Directors may determine. Such conditions and restrictions shall be set forth in the applicable Award Agreement and shall apply in addition to any restrictions that may apply to holders of Shares generally. In addition, Shares issued under the Plan shall be subject to conditions and restrictions imposed either by applicable law or by Company policy, as adopted from time to time, designed to ensure compliance with applicable law or laws with which the Company determines in its sole discretion to comply including in order to maintain any statutory, regulatory or tax advantage.

(f) Tax Matters.

(i) As a condition to the award, grant, issuance, vesting, purchase, exercise or transfer of any award, or Shares issued pursuant to any award, granted under this Plan, the Participant shall make such arrangements as the Board of Directors may require or permit for the satisfaction of any federal, state, local or foreign withholding tax obligations that may arise in connection with such event.

(ii) Unless otherwise expressly set forth in an Award Agreement, it is intended that awards granted under the Plan shall be exempt from Code Section 409A, and any ambiguity in the terms of an Award Agreement and the Plan shall be interpreted consistently with this intent. To the extent an award is not exempt from Code Section 409A (any such award, a "409A Award"), any ambiguity in the terms of such award and the Plan shall be interpreted in a manner that to the maximum extent permissible supports the award's compliance with the requirements of that statute. Notwithstanding anything to the contrary permitted under the Plan, in no event shall a modification of an Award not already subject to Code Section 409A be given effect if such modification would cause the Award to become subject to Code Section 409A unless the parties explicitly acknowledge and consent to the modification as one having that effect. A 409A Award shall be subject to such additional rules and requirements as specified by the Board of Directors from time to time in order for it to comply with the requirements of Code Section 409A. In this regard, if any amount under a 409A Award is payable upon a "separation from service" to an individual who is considered a "specified employee" (as each term is defined under Code Section 409A), then no such payment shall be made prior to the date that is the earlier of (i) six months and one day after the Participant's separation from service or (ii) the Participant's death, but only to the extent such delay is necessary to prevent such payment from being subject to Section 409A(a)(1). In addition, if a transaction subject to Section 8(b) constitutes a payment event with respect to any 409A Award, then the transaction with respect to such award must also constitute a "change in control event" as defined in Treasury Regulation Section 1.409A-3(i)(5) to the extent required by Code Section 409A.

(iii) Neither the Company nor any member of the Board of Directors shall have any liability to a Participant in the event an award held by the Participant fails to achieve its intended characterization under applicable tax law.

SECTION 11. DURATION AND AMENDMENTS; STOCKHOLDER APPROVAL.

(a) Term of the Plan. The Plan, as set forth herein, shall become effective on the date of its adoption by the Board of Directors, subject to approval of the Company's stockholders under Subsection (d) below. The Plan shall terminate automatically 10 years after the later of (i) the date when the Board of Directors adopted the Plan or (ii) the date when the Board of Directors approved the most recent increase in the number of Shares reserved under Section 4 that was also approved by the Company's stockholders. The Plan may be terminated on any earlier date pursuant to Subsection (b) below.

(b) Right to Amend or Terminate the Plan. Subject to Subsection (d) below, the Board of Directors may amend, suspend or terminate the Plan at any time and for any reason.

(c) Effect of Amendment or Termination. No Shares shall be issued or sold and no Option granted under the Plan after the termination thereof, except upon exercise of an Option (or any other right to purchase Shares) granted under the Plan prior to such termination. The termination of the Plan, or any amendment thereof, shall not affect any Share previously issued or any Option previously granted under the Plan.

(d) Stockholder Approval. To the extent required by applicable law, the Plan will be subject to approval of the Company's stockholders within 12 months of its adoption date. To the extent required by applicable law, any amendment of the Plan will be subject to the approval of the Company's stockholders within 12 months of the amendment date if it (i) increases the number of Shares available for issuance under the Plan (except as provided in Section 8), or (ii) materially changes the class of persons who are eligible for the grant of ISOs. In addition, an amendment effecting any other material change to the Plan terms will be subject to approval of the Company's stockholder only if required by applicable law. Stockholder approval shall not be required for any other amendment of the Plan.

SECTION 12. DEFINITIONS.

(a) **"Award Agreement**" means a Stock Grant Agreement, Stock Option Agreement or Stock Purchase Agreement.

(b) **"Board of Directors**" means the Board of Directors of the Company, as constituted from time to time.

(c) **"Code**" means the Internal Revenue Code of 1986, as amended.

(d) **"Committee**" means a committee of the Board of Directors, as described in Section 2(a).

(e) **"Company**" means REGENXBIO Inc., a Delaware corporation.

(f) **"Consultant**" means a person, excluding Employees and Outside Directors, who performs bona fide services for the Company, a Parent or a Subsidiary as a consultant or advisor and who qualifies as a consultant or advisor under Rule 701(c)(1) of the Securities Act or under Instruction A.1.(a)(1) of Form S-8 under the Securities Act.

(g) **"Date of Grant**" means the date of grant specified in the applicable Stock Option Agreement, which date shall be the later of (i) the date on which the Board of Directors resolved to grant the Option or (ii) the first day of the Optionee's Service.

(h) "**Disability**" means that the Optionee is unable to engage in any substantial gainful activity by reason of any medically determinable physical or mental impairment.

(i) "**Employee**" means any individual who is a common-law employee of the Company, a Parent or a Subsidiary.

(j) "**Exchange Act**" means the Securities Exchange Act of 1934, as amended.

(k) "**Exercise Price**" means the amount for which one Share may be purchased upon exercise of an Option, as specified by the Board of Directors in the applicable Stock Option Agreement.

(l) "**Fair Market Value**" means the fair market value of a Share, as determined by the Board of Directors in good faith. Such determination shall be conclusive and binding on all persons.

(m) "**Family Member**" means (i) any child, stepchild, grandchild, parent, stepparent, grandparent, spouse, former spouse, sibling, niece, nephew, mother-in-law, father-in-law, son-in-law, daughter-in-law, brother-in-law or sister-in-law, including adoptive relationships, (ii) any person sharing the Optionee's household (other than a tenant or employee), (iii) a trust in which persons described in Clause (i) or (ii) have more than 50% of the beneficial interest, (iv) a foundation in which persons described in Clause (i) or (ii) or the Optionee control the management of assets and (v) any other entity in which persons described in Clause (i) or (ii) or the Optionee own more than 50% of the voting interests.

(n) "**Grantee**" means a person to whom the Board of Directors has awarded Shares under the Plan.

(o) "**ISO**" means an Option that qualifies as an incentive stock option as described in Code Section 422(b). Notwithstanding its designation as an ISO, an Option that does not qualify as an ISO under applicable law shall be treated for all purposes as a Nonstatutory Option.

(p) "**Nonstatutory Option**" means an Option that does not qualify as an incentive stock option as described in Code Section 422(b) or 423(b).

(q) "**Option**" means an ISO or Nonstatutory Option granted under the Plan and entitling the holder to purchase Shares.

(r) "**Optionee**" means a person who holds an Option.

(s) "**Outside Director**" means a member of the Board of Directors who is not an Employee.

(t) "**Parent**" means any corporation (other than the Company) in an unbroken chain of corporations ending with the Company, if each of the corporations other than the Company owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain. A corporation that attains the status of a Parent on a date after the adoption of the Plan shall be considered a Parent commencing as of such date.

(u) "**Participant**" means a Grantee, Optionee or Purchaser.

(v) "**Plan**" means this REGENXBIO Inc. 2014 Stock Plan.

(w) "**Purchase Price**" means the consideration for which one Share may be acquired under the Plan (other than upon exercise of an Option), as specified by the Board of Directors.

(x) "**Purchaser**" means a person to whom the Board of Directors has offered the right to purchase Shares under the Plan (other than upon exercise of an Option).

(y) "**Securities Act**" means the Securities Act of 1933, as amended.

(z) "**Service**" means service as an Employee, Outside Director or Consultant.

(aa) "**Share**" means one share of Stock, as adjusted in accordance with Section 8 (if applicable).

(bb) "**Stock**" means the Common Stock of the Company.

(cc) "**Stock Grant Agreement**" means the agreement between the Company and a Grantee who is awarded Shares under the Plan that contains the terms, conditions and restrictions pertaining to the award of such Shares.

(dd) "**Stock Option Agreement**" means the agreement between the Company and an Optionee that contains the terms, conditions and restrictions pertaining to the Optionee's Option.

(ee) "**Stock Purchase Agreement**" means the agreement between the Company and a Purchaser who purchases Shares under the Plan that contains the terms, conditions and restrictions pertaining to the purchase of such Shares.

(ff) "**Subsidiary**" means any corporation (other than the Company) in an unbroken chain of corporations beginning with the Company, if each of the corporations other than the last corporation in the unbroken chain owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain. A corporation that attains the status of a Subsidiary on a date after the adoption of the Plan shall be considered a Subsidiary commencing as of such date.

EXHIBIT A

SCHEDULE OF SHARES RESERVED FOR ISSUANCE UNDER THE PLAN

Date of Board Approval	Date of Stockholder Approval	Number of Shares Added	Cumulative Number of Shares
September 16, 2014	September 16, 2014	Not Applicable	2,500,000
January 8, 2015	January 8, 2015	300,000	2,800,000
May 14, 2015	May 14, 2015	1,300,000	4,100,000

REGENXBIO INC.

2015 EQUITY INCENTIVE PLAN

(AS ADOPTED ON JUNE 17, 2015)

REGENXBIO INC.
2015 EQUITY INCENTIVE PLAN

ARTICLE 1. INTRODUCTION.

The Board adopted the Plan to become effective immediately, although no Awards may be granted prior to the Registration Date. The purpose of the Plan is to promote the long-term success of the Company and the creation of stockholder value by (a) encouraging Service Providers to focus on critical long-range corporate objectives, (b) encouraging the attraction and retention of Service Providers with exceptional qualifications and (c) linking Service Providers directly to stockholder interests through increased stock ownership. The Plan seeks to achieve this purpose by providing for Awards in the form of Options (which may constitute ISOs or NSOs), SARs, Restricted Shares, Stock Units and Performance Cash Awards.

ARTICLE 2. ADMINISTRATION.

2.1 General. The Plan may be administered by the Board or one or more Committees. Each Committee shall have the authority and be responsible for such functions as have been assigned to it.

2.2 Section 162(m). To the extent an Award is intended to qualify as "performance-based compensation" within the meaning of Code Section 162(m), the Plan will be administered by a Committee of two or more "outside directors" within the meaning of Code Section 162(m).

2.3 Section 16. To the extent desirable to qualify transactions hereunder as exempt under Exchange Act Rule 16b-3, the transactions contemplated hereunder will be approved by the entire Board or a Committee of two or more "non-employee directors" within the meaning of Exchange Act Rule 16b-3.

2.4 Powers of Administrator. Subject to the terms of the Plan, and in the case of a Committee, subject to the specific duties delegated to the Committee, the Administrator shall have the authority to (a) select the Service Providers who are to receive Awards under the Plan, (b) determine the type, number, vesting requirements and other features and conditions of such Awards, (c) determine whether and to what extent any Performance Goals have been attained, (d) interpret the Plan and Awards granted under the Plan, (e) make, amend and rescind rules relating to the Plan and Awards granted under the Plan, including rules relating to sub-plans established for the purposes of satisfying applicable foreign laws or for qualifying for favorable tax treatment under applicable foreign laws, (f) impose such restrictions, conditions or limitations as it determines appropriate as to the timing and manner of any resales by a Participant of any Common Shares issued pursuant to an Award, including restrictions under an insider trading policy and restrictions as to the use of a specified brokerage firm for such resales, and (g) make all other decisions relating to the operation of the Plan and Awards granted under the Plan.

2.5 Effect of Administrator's Decisions. The Administrator's decisions, determinations and interpretations shall be final and binding on all Participants and any other holders of Awards.

2.6 Governing Law. The Plan shall be governed by, and construed in accordance with, the laws of the State of Delaware (except its choice-of-law provisions).

ARTICLE 3. SHARES AVAILABLE FOR GRANTS.

3.1 Basic Limitation. Common Shares issued pursuant to the Plan may be authorized but unissued shares or treasury shares. The aggregate number of Common Shares issued under the Plan shall not exceed the sum of (a) shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date), (b) the number of Common Shares reserved under the Predecessor Plan that are not issued or subject to outstanding awards under the Predecessor Plan on the Registration Date, (c) any Common Shares subject to outstanding options under the Predecessor Plan on the Registration Date that subsequently expire or lapse unexercised and Common Shares issued pursuant to awards granted under the Predecessor Plans that are outstanding on the Registration Date and that are subsequently forfeited to or repurchased by the Company and (d) the additional Common Shares described in Articles 3.2 and 3.3; provided, however, that no more than Common Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date), in the aggregate, shall be added to the Plan pursuant to clauses (b) and (c). The number of Common Shares that are subject to Stock Awards outstanding at any time under the Plan may not exceed the number of Common Shares that then remain available for issuance under the Plan. The numerical limitations in this Article 3.1 shall be subject to adjustment pursuant to Article 9.

3.2 Annual Increase in Shares. As of the first business day of each fiscal year of the Company during the term of the Plan, commencing on January 1, 2016, the aggregate number of Common Shares that may be issued under the Plan shall automatically increase by a number equal to the least of (a) 4% of the total number of Common Shares outstanding on December 31 of the prior year, or (b) a number of Common Shares determined by the Board.

3.3 Shares Returned to Reserve. To the extent that Options, SARs or Stock Units granted under this Plan are forfeited or expire for any other reason before being exercised or settled in full, the Common Shares subject to such Options, SARs or Stock Units shall again become available for issuance under the Plan. If SARs are exercised, then only the number of Common Shares (if any) actually issued to the Participant in settlement of such SARs shall reduce the number available under Article 3.1 and the balance shall again become available for issuance under the Plan. If Stock Units are settled, then only the number of Common Shares (if any) actually issued to the Participant in settlement of such Stock Units shall reduce the number available under Article 3.1 and the balance shall again become available for issuance under the Plan. If Restricted Shares or Common Shares issued upon the exercise of Options or otherwise under the Plan are reacquired by the Company pursuant to a forfeiture provision, repurchase right or for any other reason prior to the shares having become vested, then such Common Shares shall again become available for issuance under the Plan. Common Shares applied to pay

the Exercise Price of Options or to satisfy tax withholding obligations related to any Award shall again become available for issuance under the Plan. To the extent that an Award is settled in cash rather than Common Shares, the cash settlement shall not reduce the number of Shares available for issuance under the Plan.

3.4 Awards Not Reducing Share Reserve in Article 3.1. Any dividend equivalents paid or credited under the Plan with respect to Stock Units shall not be applied against the number of Common Shares that may be issued under the Plan, whether or not such dividend equivalents are converted into Stock Units. In addition, Common Shares subject to Substitute Awards granted by the Company shall not reduce the number of Common Shares that may be issued under Article 3.1, nor shall shares subject to Substitute Awards again be available for Awards under the Plan in the event of any forfeiture, expiration or cash settlement of such Substitute Awards.

3.5 Share Limits. Subject to adjustment in accordance with Article 9:

(a) The aggregate number of Common Shares subject to Options and SARs that may be granted under this Plan during any calendar year to any one Participant shall not exceed Common Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date), except that the Company may grant to a new Employee in the calendar year in which his or her Service as an Employee first commences Options and/or SARs that cover (in the aggregate) up to an additional Common Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date);

(b) The aggregate number of Common Shares subject to Restricted Share awards and Stock Units that may be granted under this Plan during any calendar year to any one Participant shall not exceed Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date), except that the Company may grant to a new Employee in the calendar year in which his or her Service as an Employee first commences Restricted Share awards and Stock Units that cover (in the aggregate) up to an additional Common Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date);

(c) No Participant shall be paid more than $1 million in cash in any calendar year pursuant to Performance Cash Awards granted under the Plan;

(d) No more than Common Shares (subject to adjustment pursuant to a stock split to be effected prior to the IPO Date) plus the additional Common Shares described in Article 3.2 may be issued under the Plan upon the exercise of ISOs; and

(e) The maximum aggregate grant date fair value (as determined in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718, or any successor thereto) of Options and SARs that may be granted under this Plan to an Outside Director as compensation for services as an Outside Director during a calendar year shall not exceed $500,000.

(f) The maximum aggregate grant date fair value (as determined in accordance with Financial Accounting Standards Board Accounting Standards Codification Topic 718, or any successor thereto) of Common Shares subject to Restricted Share and Stock Units that may be granted under this Plan to an Outside Director as compensation for services as an Outside Director during a calendar year shall not exceed $500,000.

ARTICLE 4. ELIGIBILITY.

4.1 Incentive Stock Options. Only Employees who are common-law employees of the Company, a Parent or a Subsidiary shall be eligible for the grant of ISOs. In addition, an Employee who owns more than 10% of the total combined voting power of all classes of outstanding stock of the Company or any of its Parents or Subsidiaries shall not be eligible for the grant of an ISO unless the additional requirements set forth in Code Section 422(c)(5) are satisfied.

4.2 Other Awards. Awards other than ISOs may only be granted to Service Providers.

ARTICLE 5. OPTIONS.

5.1 Stock Option Agreement. Each grant of an Option under the Plan shall be evidenced by a Stock Option Agreement between the Optionee and the Company. Such Option shall be subject to all applicable terms of the Plan and may be subject to any other terms that are not inconsistent with the Plan. The Stock Option Agreement shall specify whether the Option is intended to be an ISO or an NSO. The provisions of the various Stock Option Agreements entered into under the Plan need not be identical.

5.2 Number of Shares. Each Stock Option Agreement shall specify the number of Common Shares subject to the Option, which number shall adjust in accordance with Article 9.

5.3 Exercise Price. Each Stock Option Agreement shall specify the Exercise Price, which shall not be less than 100% of the Fair Market Value of a Common Share on the date of grant. The preceding sentence shall not apply to an Option that is a Substitute Award granted in a manner that would satisfy the requirements of Code Section 409A and, if applicable, Code Section 424(a).

5.4 Exercisability and Term. Each Stock Option Agreement shall specify the date or event when all or any installment of the Option is to become vested and/or exercisable. The Stock Option Agreement shall also specify the term of the Option; provided that, except to the extent necessary to comply with applicable foreign law, the term of an Option shall in no event exceed 10 years from the date of grant. A Stock Option Agreement may provide for accelerated vesting and/or exercisability upon certain specified events and may provide for expiration prior to the end of its term in the event of the termination of the Optionee's Service.

5.5 Death of Optionee. After an Optionee's death, any vested and exercisable Options held by such Optionee may be exercised by his or her beneficiary or beneficiaries. Each Optionee may designate one or more beneficiaries for this purpose by filing the prescribed form with the Company. A beneficiary designation may be changed by filing the prescribed form with the Company at any time before the Optionee's death. If no beneficiary was designated or if no designated beneficiary survives the Optionee, then any vested and exercisable Options held by the Optionee may be exercised by his or her estate.

5.6 Modification or Assumption of Options. Within the limitations of the Plan, the Administrator may modify, reprice, extend or assume outstanding options or may accept the cancellation of outstanding options (whether granted by the Company or by another issuer) in return for the grant of new Options for the same or a different number of shares and at the same or a different exercise price or in return for the grant of a different type of Award. The foregoing notwithstanding, no modification of an Option shall, without the consent of the Optionee, impair his or her rights or obligations under such Option.

5.7 Buyout Provisions. The Administrator may at any time (a) offer to buy out for a payment in cash or cash equivalents an Option previously granted or (b) authorize an Optionee to elect to cash out an Option previously granted, in either case at such time and based upon such terms and conditions as the Administrator shall establish.

5.8 Payment for Option Shares. The entire Exercise Price of Common Shares issued upon exercise of Options shall be payable in cash or cash equivalents at the time when such Common Shares are purchased. In addition, the Administrator may, in its sole discretion and to the extent permitted by applicable law, accept payment of all or a portion of the Exercise Price through any one or a combination of the following forms or methods:

(a) Subject to any conditions or limitations established by the Administrator, by surrendering Common Shares that are already owned by the Optionee with a Fair Market Value on the date of surrender equal to the aggregate exercise price of the Common Shares as to which such Option will be exercised;

(b) By delivering (on a form prescribed by the Company) an irrevocable direction to a securities broker approved by the Company to sell all or part of the Common Shares being purchased under the Plan and to deliver all or part of the sales proceeds to the Company;

(c) Subject to such conditions and requirements as the Administrator may impose from time to time, through a net exercise procedure; or

(d) Through any other form or method consistent with applicable laws, regulations and rules.

ARTICLE 6. STOCK APPRECIATION RIGHTS.

6.1 SAR Agreement. Each grant of a SAR under the Plan shall be evidenced by a SAR Agreement between the Optionee and the Company. Such SAR shall be subject to all applicable terms of the Plan and may be subject to any other terms that are not inconsistent with the Plan. The provisions of the various SAR Agreements entered into under the Plan need not be identical.

6.2 Number of Shares. Each SAR Agreement shall specify the number of Common Shares to which the SAR pertains, which number shall adjust in accordance with Article 9.

6.3 Exercise Price. Each SAR Agreement shall specify the Exercise Price, which shall in no event be less than 100% of the Fair Market Value of a Common Share on the date of grant. The preceding sentence shall not apply to a SAR that is a Substitute Award granted in a manner that would satisfy the requirements of Code Section 409A.

6.4 Exercisability and Term. Each SAR Agreement shall specify the date when all or any installment of the SAR is to become vested and exercisable. The SAR Agreement shall also specify the term of the SAR; provided that except to the extent necessary to comply with applicable foreign law, the term of a SAR shall not exceed 10 years from the date of grant. A SAR Agreement may provide for accelerated vesting and exercisability upon certain specified events and may provide for expiration prior to the end of its term in the event of the termination of the Optionee's Service.

6.5 Exercise of SARs. Upon exercise of a SAR, the Optionee (or any person having the right to exercise the SAR after his or her death) shall receive from the Company (a) Common Shares, (b) cash or (c) a combination of Common Shares and cash, as the Administrator shall determine. The amount of cash and/or the Fair Market Value of Common Shares received upon exercise of SARs shall, in the aggregate, not exceed the amount by which the Fair Market Value (on the date of surrender) of the Common Shares subject to the SARs exceeds the Exercise Price. If, on the date when a SAR expires, the Exercise Price is less than the Fair Market Value on such date but any portion of such SAR has not been exercised or surrendered, then such SAR shall automatically be deemed to be exercised as of such date with respect to such portion. A SAR Agreement may also provide for an automatic exercise of the SAR on an earlier date.

6.6 Death of Optionee. After an Optionee's death, any vested and exercisable SARs held by such Optionee may be exercised by his or her beneficiary or beneficiaries. Each Optionee may designate one or more beneficiaries for this purpose by filing the prescribed form with the Company. A beneficiary designation may be changed by filing the prescribed form with the Company at any time before the Optionee's death. If no beneficiary was designated or if no designated beneficiary survives the Optionee, then any vested and exercisable SARs held by the Optionee at the time of his or her death may be exercised by his or her estate.

6.7 Modification or Assumption of SARs. Within the limitations of the Plan, the Administrator may modify, reprice, extend or assume outstanding SARs or may accept the cancellation of outstanding SARs (whether granted by the Company or by another issuer) in return for the grant of new SARs for the same or a different number of shares and at the same or a different exercise price or in return for the grant of a different type of Award. The foregoing notwithstanding, no modification of a SAR shall, without the consent of the Optionee, impair his or her rights or obligations under such SAR.

ARTICLE 7. RESTRICTED SHARES.

7.1 Restricted Stock Agreement. Each grant of Restricted Shares under the Plan shall be evidenced by a Restricted Stock Agreement between the recipient and the Company.

Such Restricted Shares shall be subject to all applicable terms of the Plan and may be subject to any other terms that are not inconsistent with the Plan. The provisions of the various Restricted Stock Agreements entered into under the Plan need not be identical.

7.2 Payment for Awards. Restricted Shares may be sold or awarded under the Plan for such consideration as the Administrator may determine, including (without limitation) cash, cash equivalents, property, cancellation of other equity awards, full-recourse promissory notes, past services and future services, and such other methods of payment as are permitted by applicable law.

7.3 Vesting Conditions. Each Award of Restricted Shares may or may not be subject to vesting and/or other conditions as the Administrator may determine. Vesting shall occur, in full or in installments, upon satisfaction of the conditions specified in the Restricted Stock Agreement. Such conditions, at the Administrator's discretion, may include one or more Performance Goals. A Restricted Stock Agreement may provide for accelerated vesting upon certain specified events.

7.4 Voting and Dividend Rights. The holders of Restricted Shares awarded under the Plan shall have the same voting, dividend and other rights as the Company's other stockholders, unless the Administrator otherwise provides. A Restricted Stock Agreement, however, may require that any cash dividends paid on Restricted Shares (a) be accumulated and paid when such Restricted Shares vest, or (b) be invested in additional Restricted Shares. Such additional Restricted Shares shall be subject to the same conditions and restrictions as the shares subject to the Stock Award with respect to which the dividends were paid. In addition, unless the Administrator provides otherwise, if any dividends or other distributions are paid in Common Shares, such Common Shares shall be subject to the same restrictions on transferability and forfeitability as the Restricted Shares with respect to which they were paid.

ARTICLE 8. STOCK UNITS.

8.1 Stock Unit Agreement. Each grant of Stock Units under the Plan shall be evidenced by a Stock Unit Agreement between the recipient and the Company. Such Stock Units shall be subject to all applicable terms of the Plan and may be subject to any other terms that are not inconsistent with the Plan. The provisions of the various Stock Unit Agreements entered into under the Plan need not be identical.

8.2 Payment for Awards. To the extent that an Award is granted in the form of Stock Units, no cash consideration shall be required of the Award recipients.

8.3 Vesting Conditions. Each Award of Stock Units may or may not be subject to vesting, as determined by the Administrator. Vesting shall occur, in full or in installments, upon satisfaction of the conditions specified in the Stock Unit Agreement. Such conditions, at the Administrator's discretion, may include one or more Performance Goals. A Stock Unit Agreement may provide for accelerated vesting upon certain specified events.

8.4 Voting and Dividend Rights. The holders of Stock Units shall have no voting rights. Prior to settlement or forfeiture, Stock Units awarded under the Plan may, at the Administrator's discretion, provide for a right to dividend equivalents. Such right entitles the

holder to be credited with an amount equal to all cash dividends paid on one Common Share while the Stock Unit is outstanding. Dividend equivalents may be converted into additional Stock Units. Settlement of dividend equivalents may be made in the form of cash, in the form of Common Shares, or in a combination of both. Prior to distribution, any dividend equivalents shall be subject to the same conditions and restrictions as the Stock Units to which they attach.

8.5 Form and Time of Settlement of Stock Units. Settlement of vested Stock Units may be made in the form of (a) cash, (b) Common Shares or (c) any combination of both, as determined by the Administrator. The actual number of Stock Units eligible for settlement may be larger or smaller than the number included in the original Award, based on predetermined performance factors, including Performance Goals. Methods of converting Stock Units into cash may include (without limitation) a method based on the average Fair Market Value of Common Shares over a series of trading days. Vested Stock Units shall be settled in such manner and at such time(s) as specified in the Stock Unit Agreement. Until an Award of Stock Units is settled, the number of such Stock Units shall be subject to adjustment pursuant to Article 9.

8.6 Death of Recipient. Any Stock Units that become payable after the recipient's death shall be distributed to the recipient's beneficiary or beneficiaries. Each recipient of Stock Units under the Plan may designate one or more beneficiaries for this purpose by filing the prescribed form with the Company. A beneficiary designation may be changed by filing the prescribed form with the Company at any time before the Award recipient's death. If no beneficiary was designated or if no designated beneficiary survives the Award recipient, then any Stock Units that become payable after the recipient's death shall be distributed to the recipient's estate.

8.7 Modification or Assumption of Stock Units. Within the limitations of the Plan, the Administrator may modify or assume outstanding stock units or may accept the cancellation of outstanding stock units (whether granted by the Company or by another issuer) in return for the grant of new Stock Units for the same or a different number of shares or in return for the grant of a different type of Award. The foregoing notwithstanding, no modification of a Stock Unit shall, without the consent of the Participant, impair his or her rights or obligations under such Stock Unit.

8.8 Creditors' Rights. A holder of Stock Units shall have no rights other than those of a general creditor of the Company. Stock Units represent an unfunded and unsecured obligation of the Company, subject to the terms and conditions of the applicable Stock Unit Agreement.

ARTICLE 9. ADJUSTMENTS; DISSOLUTIONS AND LIQUIDATIONS; CORPORATE TRANSACTIONS.

9.1 Adjustments. In the event of a subdivision of the outstanding Common Shares, a declaration of a dividend payable in Common Shares or a combination or consolidation of the outstanding Common Shares (by reclassification or otherwise) into a lesser number of Common Shares, corresponding proportionate adjustments shall automatically be made in each of the following:

(a) The number and kind of shares available for issuance under Article 3, including the numerical share limits in Articles 3.1, 3.2 and 3.5;

(b) The number and kind of shares covered by each outstanding Option, SAR and Stock Unit; and

(c) The Exercise Price applicable to each outstanding Option and SAR, and the repurchase price, if any, applicable to Restricted Shares.

In the event of a declaration of an extraordinary dividend payable in a form other than Common Shares in an amount that has a material effect on the price of Common Shares, a recapitalization, a spin-off or a similar occurrence, the Administrator shall make such adjustments as it, in its sole discretion, deems appropriate in one or more of the foregoing. Any adjustment in the number of and kind of shares subject to an Award under this Article 9.1 shall be rounded down to the nearest whole share, although the Administrator in its sole discretion may make a cash payment in lieu of a fractional share. Except as provided in this Article 9, a Participant shall have no rights by reason of any issuance by the Company of stock of any class or securities convertible into stock of any class, any subdivision or consolidation of shares of stock of any class, the payment of any stock dividend or any other increase or decrease in the number of shares of stock of any class.

9.2 Dissolution or Liquidation. To the extent not previously exercised or settled, Options, SARs and Stock Units shall terminate immediately prior to the dissolution or liquidation of the Company.

9.3 Corporate Transactions. In the event that the Company is a party to a merger, consolidation, or a Change in Control (other than one described in Article 14.6(d)), all Common Shares acquired under the Plan and all Awards outstanding on the effective date of the transaction shall be treated in the manner described in the definitive transaction agreement (or, in the event the transaction does not entail a definitive agreement to which the Company is party, in the manner determined by the Administrator, with such determination having final and binding effect on all parties), which agreement or determination need not treat all Awards (or portions thereof) in an identical manner. Unless an Award Agreement provides otherwise, the treatment specified in the transaction agreement or by the Administrator shall include (without limitation) one or more of the following with respect to each outstanding Award:

(a) The continuation of such outstanding Awards by the Company (if the Company is the surviving entity);

(b) The assumption of such outstanding Awards by the surviving entity or its parent, provided that the assumption of an Option or a SAR shall comply with applicable tax requirements;

(c) The substitution by the surviving entity or its parent of an equivalent award for outstanding Awards (including, but not limited to, an award to acquire the same consideration paid to the holders of Common Shares in the transaction), provided that the substitution of an Option or a SAR shall comply with applicable tax requirements;

(d) The cancellation of outstanding Options and SARs without payment of any consideration. The Optionees shall be able to exercise such Options and SARs (to the extent the Options and SARs are vested or become vested as of the effective date of the transaction) during a period of not less than five full business days preceding the closing date of the transaction, unless (i) a shorter period is required to permit a timely closing of the transaction and (ii) such shorter period still offers the Optionees a reasonable opportunity to exercise such Options and SARs. Any exercise of such Options and SARs during such period may be contingent on the closing of the transaction;

(e) Full exercisability of outstanding Options and SARs and full vesting of the Common Shares subject to Options and SARs, followed by cancellation of such Options and SARs. The full exercisability of such Options and SARs and full vesting of such Common Shares may be contingent on the closing of the transaction. The Optionees shall be able to exercise such Options and SARs during a period of not less than five full business days preceding the closing date of such merger or consolidation, unless (i) a shorter period is required to permit a timely closing of such merger or consolidation and (ii) such shorter period still offers the Optionees a reasonable opportunity to exercise such Options and SARs. Any exercise of such Options and SARs during such period may be contingent on the closing of such merger or consolidation;

(f) The cancellation of the Options and SARs and a payment to the Optionee with respect to each Share subject to the portion of the Award that is vested as of the transaction date equal to the excess of (A) the value, as determined by the Administrator in its absolute discretion, of the property (including cash) received by the holder of a Common Share as a result of the transaction, over (B) the per-share Exercise Price of the Option or SAR (such excess, the "**Spread**"). Such payment shall be made in the form of cash, cash equivalents, or securities of the surviving entity or its parent having a value equal to the Spread. In addition, any escrow, holdback, earn-out or similar provisions in the transaction agreement may apply to such payment to the same extent and in the same manner as such provisions apply to the holders of Common Shares, but only to the extent the application of such provisions does not adversely affect the status of the Option or SAR as exempt from Code Section 409A. If the Spread applicable to an Option or SAR is zero or a negative number, then the Option or SAR may be cancelled without making a payment to the Optionee;

(g) The cancellation of outstanding Stock Units and a payment to the holder thereof with respect to each Common Share subject to the Stock Unit (whether or not such Stock Unit is then vested) equal to the value, as determined by the Administrator in its absolute discretion, of the property (including cash) received by the holder of a Common Share as a result of the transaction (the "**Transaction Value**"). Such payment shall be made in the form of cash, cash equivalents, or securities of the surviving entity or its parent having a value equal to the Transaction Value. In addition, such payment may be subject to vesting based on the Participant's continuing Service, provided that the vesting schedule shall not be less favorable to the Participant than the schedule under which such Stock Units would have vested, and if required under applicable tax rules, such payment may be deferred until the settlement date specified in the Stock Unit Agreement. In addition, any escrow, holdback, earn-out or similar provisions in the transaction agreement may apply to such payment to the

same extent and in the same manner as such provisions apply to the holders of Common Shares. In the event that a Stock Unit is subject to Code Section 409A, the payment described in this clause (g) shall be made on the settlement date specified in the applicable Stock Unit Agreement, provided that settlement may be accelerated in accordance with Treasury Regulation Section 1.409A-3(j)(4); or

(h) The assignment of any reacquisition or repurchase rights held by the Company in respect of an Award of Restricted Shares to the surviving entity or its parent, with corresponding proportionate adjustments made to the price per share to be paid upon exercise of any such reacquisition or repurchase rights.

For avoidance of doubt, the Administrator shall have the discretion, exercisable either at the time an Award is granted or at any time while the Award remains outstanding, to provide for the acceleration of vesting upon the occurrence of a Change in Control, whether or not the Award is to be assumed or replaced in the transaction, or in connection with a termination of the Participant's Service following a transaction.

Any action taken under this Article 9.3 shall either preserve an Award's status as exempt from Code Section 409A or comply with Code Section 409A.

ARTICLE 10. OTHER AWARDS.

10.1 Performance Cash Awards. A Performance Cash Award is a cash award that may be granted subject to the attainment of specified Performance Goals during a Performance Period. A Performance Cash Award may also require the completion of a specified period of continuous Service. The length of the Performance Period, the Performance Goals to be attained during the Performance Period, and the degree to which the Performance Goals have been attained shall be determined conclusively by the Administrator. Each Performance Cash Award shall be set forth in a written agreement or in a resolution duly adopted by the Administrator which shall contain provisions determined by the Administrator and not inconsistent with the Plan. The terms of various Performance Cash Awards need not be identical.

10.2 Awards Under Other Plans. The Company may grant awards under other plans or programs. Such awards may be settled in the form of Common Shares issued under this Plan. Such Common Shares shall be treated for all purposes under the Plan like Common Shares issued in settlement of Stock Units and shall, when issued, reduce the number of Common Shares available under Article 3.

ARTICLE 11. LIMITATION ON RIGHTS.

11.1 Retention Rights. Neither the Plan nor any Award granted under the Plan shall be deemed to give any individual a right to remain a Service Provider. The Company and its Parents, Subsidiaries and Affiliates reserve the right to terminate the Service of any Service Provider at any time, with or without cause, subject to applicable laws, the Company's certificate of incorporation and by-laws and a written employment agreement (if any).

11.2 Stockholders' Rights. Except as set forth in Article 7.4 or 8.4 above, a Participant shall have no dividend rights, voting rights or other rights as a stockholder with

respect to any Common Shares covered by his or her Award prior to the time when a stock certificate for such Common Shares is issued or, if applicable, the time when he or she becomes entitled to receive such Common Shares by filing any required notice of exercise and paying any required Exercise Price. No adjustment shall be made for cash dividends or other rights for which the record date is prior to such time, except as expressly provided in the Plan.

11.3 Regulatory Requirements. Any other provision of the Plan notwithstanding, the obligation of the Company to issue Common Shares under the Plan shall be subject to all applicable laws, rules and regulations and such approval by any regulatory body as may be required. The Company reserves the right to restrict, in whole or in part, the delivery of Common Shares pursuant to any Award prior to the satisfaction of all legal requirements relating to the issuance of such Common Shares, to their registration, qualification or listing or to an exemption from registration, qualification or listing. The inability of the Company to obtain authority from any regulatory body having jurisdiction, which authority is deemed necessary by the Company's counsel to be necessary to the lawful issuance and sale of any Common Shares hereunder, will relieve the Company of any liability in respect of the failure to issue or sell such Common Shares as to which such requisite authority will not have been obtained.

11.4 Transferability of Awards. The Administrator may, in its sole discretion, permit transfer of an Award in a manner consistent with applicable law. Unless otherwise determined by the Administrator, Awards shall be transferable by a Participant only by (a) beneficiary designation, (b) a will or (c) the laws of descent and distribution. An ISO may only be transferred by will or by the laws of descent and distribution and may be exercised during the lifetime of the Optionee only by the Optionee or by the Optionee's guardian or legal representative.

11.5 Other Conditions and Restrictions on Common Shares. Any Common Shares issued under the Plan shall be subject to such forfeiture conditions, rights of repurchase, rights of first refusal, other transfer restrictions and such other terms and conditions as the Administrator may determine. Such conditions and restrictions shall be set forth in the applicable Award Agreement and shall apply in addition to any restrictions that may apply to holders of Common Shares generally. In addition, Common Shares issued under the Plan shall be subject to such conditions and restrictions imposed either by applicable law or by Company policy, as adopted from time to time, designed to ensure compliance with applicable law or laws with which the Company determines in its sole discretion to comply including in order to maintain any statutory, regulatory or tax advantage.

ARTICLE 12. TAXES.

12.1 General. As a condition to an Award under the Plan, a Participant or his or her successor shall make arrangements satisfactory to the Company for the satisfaction of any federal, state, local or foreign withholding tax obligations that arise in connection with any Award granted under the Plan. The Company shall not be required to issue any Common Shares or make any cash payment under the Plan until such obligations are satisfied.

12.2 Share Withholding. To the extent that applicable law subjects a Participant to tax withholding obligations, the Administrator may permit such Participant to satisfy all or part

of such obligations by having the Company withhold all or a portion of any Common Shares that otherwise would be issued to him or her or by surrendering all or a portion of any Common Shares that he or she previously acquired. Such Common Shares shall be valued at their Fair Market Value on the date when they are withheld or surrendered. Any payment of taxes by assigning Common Shares to the Company may be subject to restrictions including any restrictions required by SEC, accounting or other rules.

12.3 Section 162(m) Matters. The Administrator, in its sole discretion, may determine whether an Award is intended to qualify as "performance-based compensation" within the meaning of Code Section 162(m). The Administrator may grant Awards that are based on Performance Goals but that are not intended to qualify as performance-based compensation. With respect to any Award that is intended to qualify as performance-based compensation, the Administrator shall designate the Performance Goal(s) applicable to, and the formula for calculating the amount payable under, an Award within 90 days following commencement of the applicable Performance Period (or such earlier time as may be required under Code Section 162(m)), and in any event at a time when achievement of the applicable Performance Goal(s) remains substantially uncertain. Prior to the payment of any Award that is intended to constitute performance-based compensation, the Administrator shall certify in writing whether and the extent to which the Performance Goal(s) were achieved for such Performance Period. The Administrator shall have the right to reduce or eliminate (but not to increase) the amount payable under an Award that is intended to constitute performance-based compensation.

12.4 Section 409A Matters. Except as otherwise expressly set forth in an Award Agreement, it is intended that Awards granted under the Plan either be exempt from, or comply with, the requirements of Code Section 409A. To the extent an Award is subject to Code Section 409A (a "**409A Award**"), the terms of the Plan, the Award and any written agreement governing the Award shall be interpreted to comply with the requirements of Code Section 409A so that the Award is not subject to additional tax or interest under Code Section 409A, unless the Administrator expressly provides otherwise. A 409A Award shall be subject to such additional rules and requirements as specified by the Administrator from time to time in order for it to comply with the requirements of Code Section 409A. In this regard, if any amount under a 409A Award is payable upon a "separation from service" to an individual who is considered a "specified employee" (as each term is defined under Code Section 409A), then no such payment shall be made prior to the date that is the earlier of (i) six months and one day after the Participant's separation from service or (ii) the Participant's death, but only to the extent such delay is necessary to prevent such payment from being subject to Code Section 409A(a)(1).

12.5 Limitation on Liability. Neither the Company nor any person serving as Administrator shall have any liability to a Participant in the event an Award held by the Participant fails to achieve its intended characterization under applicable tax law.

ARTICLE 13. FUTURE OF THE PLAN.

13.1 Term of the Plan. The Plan, as set forth herein, shall become effective on the Registration Date. The Plan shall remain in effect until the earlier of (a) the date when the Plan is terminated under Article 13.2 or (b) the 10th anniversary of the date when the Board adopted the Plan.

13.2 Amendment or Termination. The Board may, at any time and for any reason, amend or terminate the Plan. No Awards shall be granted under the Plan after the termination thereof. The termination of the Plan, or any amendment thereof, shall not affect any Award previously granted under the Plan.

13.3 Stockholder Approval. An amendment of the Plan shall be subject to the approval of the Company's stockholders only to the extent required by applicable laws, regulations or rules.

ARTICLE 14. DEFINITIONS.

14.1 **"Administrator"** means the Board or any Committee administering the Plan in accordance with Article 2.

14.2 **"Affiliate"** means any entity other than a Subsidiary, if the Company and/or one or more Subsidiaries own not less than 50% of such entity.

14.3 **"Award"** means any award granted under the Plan, including as an Option, a SAR, a Restricted Share, a Stock Unit or a Performance Cash Award.

14.4 **"Award Agreement"** means a Stock Option Agreement, an SAR Agreement, a Restricted Stock Agreement, a Stock Unit Agreement or such other agreement evidencing an Award granted under the Plan.

14.5 **"Board"** means the Company's Board of Directors, as constituted from time to time.

14.6 **"Change in Control"** means:

(a) Any "person" (as such term is used in Sections 13(d) and 14(d) of the Exchange Act) becomes the "beneficial owner" (as defined in Rule 13d-3 of the Exchange Act), directly or indirectly, of securities of the Company representing more than fifty percent (50%) of the total voting power represented by the Company's then-outstanding voting securities;

(b) The consummation of the sale or disposition by the Company of all or substantially all of the Company's assets;

(c) The consummation of a merger or consolidation of the Company with or into any other entity, other than a merger or consolidation which would result in the voting securities of the Company outstanding immediately prior thereto continuing to represent (either by remaining outstanding or by being converted into voting securities of the surviving entity or its parent) more than fifty percent (50%) of the total voting power represented by the voting securities of the Company or such surviving entity or its parent outstanding immediately after such merger or consolidation; or

(d) Individuals who are members of the Board (the "**Incumbent Board**") cease for any reason to constitute at least a majority of the members of the Board over a period of 12 months; provided, however, that if the appointment or election (or nomination for election) of any new Board member was approved or recommended by a majority vote of the members of the Incumbent Board then still in office, such new member shall, for purposes of this Plan, be considered as a member of the Incumbent Board.

A transaction shall not constitute a Change in Control if its sole purpose is to change the state of the Company's incorporation or to create a holding company that will be owned in substantially the same proportions by the persons who held the Company's securities immediately before such transaction. In addition, if a Change in Control constitutes a payment event with respect to any Award which provides for a deferral of compensation and is subject to Code Section 409A, then notwithstanding anything to the contrary in the Plan or applicable Award Agreement the transaction with respect to such Award must also constitute a "change in control event" as defined in Treasury Regulation Section 1.409A-3(i)(5) to the extent required by Code Section 409A.

14.7 **"Code"** means the Internal Revenue Code of 1986, as amended.

14.8 **"Committee"** means a committee of one or more members of the Board, or of other individuals satisfying applicable laws, appointed by the Board to administer the Plan.

14.9 **"Common Share"** means one share of the common stock of the Company.

14.10 **"Company"** means Histogenics Corporation, a Delaware corporation.

14.11 **"Consultant"** means a consultant or adviser who provides *bona fide* services to the Company, a Parent, a Subsidiary or an Affiliate as an independent contractor and who qualifies as a consultant or advisor under Instruction A.1.(a)(1) of Form S-8 under the Securities Act of 1933, as amended.

14.12 **"Employee"** means a common-law employee of the Company, a Parent, a Subsidiary or an Affiliate.

14.13 **"Exchange Act"** means the Securities Exchange Act of 1934, as amended.

14.14 **"Exercise Price,"** in the case of an Option, means the amount for which one Common Share may be purchased upon exercise of such Option, as specified in the applicable Stock Option Agreement. "Exercise Price," in the case of a SAR, means an amount, as specified in the applicable SAR Agreement, which is subtracted from the Fair Market Value of one Common Share in determining the amount payable upon exercise of such SAR.

14.15 **"Fair Market Value"** means the closing price of a Common Share on any established stock exchange or a national market system on the applicable date or, if the applicable date is not a trading day, on the last trading day prior to the applicable date, as reported in a source that the Administrator deems reliable. If Common Shares are no longer traded on an established stock exchange or a national market system, the Fair Market Value shall be determined by the Administrator in good faith on such basis as it deems appropriate. The Administrator's determination shall be conclusive and binding on all persons.

14.16 **"ISO"** means an incentive stock option described in Code Section 422(b).

14.17 **"NSO"** means a stock option not described in Code Sections 422 or 423.

14.18 **"Option"** means an ISO or NSO granted under the Plan and entitling the holder to purchase Common Shares.

14.19 **"Optionee"** means an individual or estate holding an Option or SAR.

14.20 **"Outside Director"** means a member of the Board who is not an Employee.

14.21 **"Parent"** means any corporation (other than the Company) in an unbroken chain of corporations ending with the Company, if each of the corporations other than the Company owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain. A corporation that attains the status of a Parent on a date after the adoption of the Plan shall be considered a Parent commencing as of such date.

14.22 **"Participant"** means an individual or estate holding an Award.

14.23 **"Performance Cash Award"** means an award of cash granted under Article 10.1 of the Plan.

14.24 **"Performance Goal"** means a goal established by the Administrator for the applicable Performance Period based on one or more of the performance criteria set forth in **Appendix A**. Depending on the performance criteria used, a Performance Goal may be expressed in terms of overall Company performance or the performance of a business unit, division, Subsidiary, Affiliate or an individual. A Performance Goal may be measured either in absolute terms or relative to the performance of one or more comparable companies or one or more relevant indices. The Administrator may adjust the results under any performance criterion to exclude any of the following events that occurs during a Performance Period: (a) asset write-downs, (b) litigation, claims, judgments or settlements, (c) the effect of changes in tax laws, accounting principles or other laws or provisions affecting reported results, (d) accruals for reorganization and restructuring programs, (e) extraordinary, unusual or non-recurring items, (f) exchange rate effects for non-U.S. dollar denominated net sales and operating earnings, or (g) statutory adjustments to corporate tax rates; provided, however, that if an Award is intended to qualify as "performance-based compensation" within the meaning of Code Section 162(m), such adjustment(s) shall only be made to the extent consistent with Code Section 162(m).

14.25 **"Performance Period"** means a period of time selected by the Administrator over which the attainment of one or more Performance Goals will be measured for the purpose of determining a Participant's right to a Performance Cash Award or an Award of Restricted Shares or Stock Units that vests based on the achievement of Performance Goals. Performance Periods may be of varying and overlapping duration, at the discretion of the Administrator.

14.26 **"Plan"** means this REGENXBIO Inc. 2015 Equity Incentive Plan, as amended from time to time.

14.27 **"Predecessor Plan"** means the Company's 2014 Stock Plan, as amended.

14.28 **"Registration Date"** means the effective date of the registration statement filed by the Company with the Securities and Exchange Commission pursuant to Form S-1.

14.29 **"Restricted Share"** means a Common Share awarded under the Plan.

14.30 **"Restricted Stock Agreement"** means the agreement between the Company and the recipient of a Restricted Share that contains the terms, conditions and restrictions pertaining to such Restricted Share.

14.31 **"SAR"** means a stock appreciation right granted under the Plan.

14.32 **"SAR Agreement"** means the agreement between the Company and an Optionee that contains the terms, conditions and restrictions pertaining to his or her SAR.

14.33 **"Service"** means service as an Employee, Outside Director or Consultant.

14.34 **"Service Provider"** means any individual who is an Employee, Outside Director or Consultant.

14.35 **"Stock Award"** means any award of an Option, a SAR, a Restricted Share or a Stock Unit under the Plan.

14.36 **"Stock Option Agreement"** means the agreement between the Company and an Optionee that contains the terms, conditions and restrictions pertaining to his or her Option.

14.37 **"Stock Unit"** means a bookkeeping entry representing the equivalent of one Common Share, as awarded under the Plan.

14.38 **"Stock Unit Agreement"** means the agreement between the Company and the recipient of a Stock Unit that contains the terms, conditions and restrictions pertaining to such Stock Unit.

14.39 **"Subsidiary"** means any corporation (other than the Company) in an unbroken chain of corporations beginning with the Company, if each of the corporations other than the last corporation in the unbroken chain owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain. A corporation that attains the status of a Subsidiary on a date after the adoption of the Plan shall be considered a Subsidiary commencing as of such date

14.40 **“Substitute Awards”** means Awards or Common Shares issued by the Company in assumption of, or substitution or exchange for, Awards previously granted, or the right or obligation to make future awards, in each case by a corporation acquired by the Company or any Affiliate or with which the Company or any Affiliate combines to the extent permitted by NASDAQ Marketplace Rule 5635 or any successor thereto.

APPENDIX A

PERFORMANCE CRITERIA

The Administrator may establish Performance Goals derived from one or more of the following criteria when it makes Awards of Restricted Shares or Stock Units that vest entirely or in part on the basis of performance or when it makes Performance Cash Awards:

- Earnings (before or after taxes)
- Earnings per share
- Earnings before interest, taxes and depreciation
- Earnings before interest, taxes, depreciation and amortization
- Total stockholder return
- Return on equity or average stockholders' equity
- Return on assets, investment or capital employed
- Operating income
- Gross margin
- Operating margin
- Net operating income
- Net operating income after tax
- Return on operating revenue
- Objective corporate or individual strategic goals
- Sales or revenue (using a measure thereof that complies with Section 162(m))
- Expense or cost reduction
- Working capital
- Economic value added (or an equivalent metric)
- Market share
- Cash measures including cash flow and cash balance
- Operating cash flow
- Cash flow per share
- Share price
- Debt reduction
- Customer satisfaction
- Stockholders' equity
- Contract awards or backlog
- Objective individual performance goals
- To the extent that an Award is not intended to comply with Code Section 162(m), other measures of performance selected by the Administrator

REGENXBIO INC.

2015 EMPLOYEE STOCK PURCHASE PLAN

(AS ADOPTED ON JUNE 17, 2015)

REGENXBIO INC.

2015 EMPLOYEE STOCK PURCHASE PLAN

SECTION 1. PURPOSE OF THE PLAN.

The Board adopted the Plan effective as of the IPO Date. The purpose of the Plan is to provide Eligible Employees with an opportunity to increase their proprietary interest in the success of the Company by purchasing Stock from the Company on favorable terms and to pay for such purchases through payroll deductions or other approved contributions.

SECTION 2. ADMINISTRATION OF THE PLAN.

(a) **Committee Composition.** The Committee shall administer the Plan. The Committee shall consist exclusively of one or more members of the Board, who shall be appointed by the Board.

(b) **Committee Responsibilities.** The Committee shall interpret the Plan and make all other policy decisions relating to the operation of the Plan. The Committee may adopt such rules, guidelines and forms as it deems appropriate to implement the Plan. The Committee's determinations under the Plan shall be final and binding on all persons.

SECTION 3. STOCK OFFERED UNDER THE PLAN.

(a) **Authorized Shares.** The number of shares of Stock available for purchase under the Plan shall be of the Company's Stock (subject to adjustment pursuant to Subsection (c) below), plus the additional shares described in Subsection (b) below. Shares of Stock issued pursuant to the Plan may be authorized but unissued shares or treasury shares.

(b) **Annual Increase in Shares.** As of the first business day of each fiscal year of the Company during the term of the Plan, commencing on January 1, 2016, the aggregate number of shares of Stock that may be issued under the Plan shall automatically increase by a number equal to the least of (i) 1% of the total number of shares of Stock actually issued and outstanding on the last business day of the prior fiscal year (excluding any rights to purchase shares of common shares that may be outstanding, such as options or warrants), (ii) shares of Stock (subject to adjustment pursuant to Subsection (c) below), or (iii) a number of shares of Stock determined by the Board.

(c) **Anti-Dilution Adjustments.** In the event that any dividend or other distribution (whether in the form of cash, stock or other securities or other property), recapitalization, stock split, reverse stock split, reorganization, merger, consolidation, split-up, spin-off, combination, repurchase, or exchange of Stock or other securities of the Company, or other similar change in the corporate structure of the Company affecting the Stock and effected without receipt or payment of consideration by the Company occurs, then in order to prevent dilution or enlargement of the benefits or potential benefits intended to be made available under

the Plan, there will be a proportionate adjustment of the number and class of Stock that may be delivered under the Plan, the Purchase Price per share and the number of shares of Stock covered by each option under the Plan which has not yet been exercised, and the numerical limits of Sections 3(a), 3(b)(ii) and 9(c).

(d) **Reorganizations.** Any other provision of the Plan notwithstanding, in the event of a Corporate Reorganization, the Plan may be continued or assumed by the surviving corporation or its parent corporation. If such acquirer refuses to continue or assume the Plan, then, immediately prior to the effective time of the Corporate Reorganization, any Offering Period then in progress shall terminate, and, a new Purchase Date for each such Offering Period will be set, immediately prior to the effective time of the Corporate Reorganization. In the event a new Purchase Date is set under this Section 3(d), Participants will be given notice of the new Purchase Date. The Plan shall in no event be construed to restrict in any way the Company's right to undertake a dissolution, liquidation, merger, consolidation or other reorganization.

SECTION 4. ENROLLMENT AND PARTICIPATION.

(a) **Offering Periods and Purchase Periods.**

(i) **Base Offering Periods**. The Committee may establish Offering Periods of such frequency and duration as it may from time to time determine as appropriate (the "***Base Offering Periods***"); provided that a Base Offering Period shall in no event be longer than 27 months (or such other period as may be imposed under applicable tax law). The Base Offering Periods are intended to qualify under Code Section 423. Unless changed by the Committee, the Plan shall operate such that two Base Offering Periods, each of six months' duration and each including a single six-month Purchase Period, will commence on July 1 and January 1 of each year, except that the first Base Offering Period will commence on the IPO Date and shall end on or about December 31, 2015. The Committee may determine that the first Base Offering Period applicable to the Eligible Employees of a new Participating Company shall commence on any later date specified by the Committee.

(ii) **Additional Offering Periods.** At the discretion of the Committee, additional Offering Periods (the "Additional Offering Periods") may be conducted under the Plan or, if necessary or advisable, in the sole discretion of the Committee, under a separate sub-plan or sub-plans permitting grants to Eligible Employees of certain Participating Companies (each, a "Sub-Plan"). Such Additional Offering Periods may, but need not, qualify under Code Section 423, and may be designed to achieve desired tax or other objectives in particular locations outside the United States of America or to comply with local laws applicable to offerings in such foreign jurisdictions. The Committee shall determine the commencement and duration of each Additional Offering Period, and Additional Offering Periods may be consecutive or overlapping. The other terms and conditions of each Additional Offering Period shall be those set forth in this Plan document or in the applicable Sub-Plan, with such changes or additional features as the Committee determines necessary to comply with local law. Each Sub-Plan shall be considered a separate plan from the Plan (the "Statutory Plan"). The total number of Shares authorized to be issued under the Plan as provided in Section 3 above applies in the aggregate to both the Statutory Plan and any Sub-Plan. Unless otherwise superseded by the terms of such Sub-Plan, the provisions of this Plan document shall govern the operation of such Sub-Plan.

(iii) **Separate Offerings.** Each Base Offering Period and Additional Offering Period conducted under the Plan or any Sub-Plan is intended to constitute a separate "offering" for purposes of Code Section 423.

(iv) **Equal Rights and Privileges.** To the extent an Offering Period is intended to qualify under Code Section 423, all participants in such Offering Period shall have the same rights and privileges with respect to their participation in such Offering Period in accordance with Code Section 423 and the regulations thereunder except for differences that may be mandated by local law and are consistent with the requirements of Code Section 423(b)(5).

(b) **Enrollment At IPO.** Each individual who, on the IPO Date, qualifies as an Eligible Employee shall automatically become a Participant on such day, and shall be considered to have been granted an option to participate in the first Offering Period under the Plan at the maximum applicable participation rate. Each Participant who was automatically enrolled on the IPO Date shall file the prescribed enrollment form with the Company. The enrollment form shall be filed at the prescribed location by a date specified by the Company, but in no event later than 30 days after the IPO Date. If a Participant who was automatically enrolled on the IPO Date fails to file such form in a timely manner, then such Participant shall be deemed to have withdrawn from the Plan under Section 6(a). A former Participant who is deemed to have withdrawn from the Plan shall not be a Participant until he or she re-enrolls in the Plan under Subsection (c) below. Re-enrollment may be effective only at the commencement of an Offering Period.

(c) **Enrollment After IPO.** Any individual who qualifies as an Eligible Employee on the first day of any Offering Period other than the first Offering Period may elect to become a Participant on such day by filing the prescribed enrollment form with the Company. The enrollment form shall be filed at the prescribed location at least 10 business days (or such other period as the Committee or its designee may designate) prior to such day.

(d) **Duration of Participation.** Once enrolled in the Plan, a Participant shall continue to participate in the Plan until he or she:

(i) Reaches the end of the Offering Period or Purchase Period, as applicable, in which his or her employee contributions were discontinued under Section 5(c) or 9(b);

(ii) Is deemed to withdraw from the Plan under Subsection (b) above;

(iii) Withdraws from the Plan under Section 6(a); or

(iv) Ceases to be an Eligible Employee.

A Participant whose employee contributions were discontinued automatically under Section 9(b) shall automatically resume participation at the beginning of the earliest Offering Period ending in a later calendar year, if he or she then is an Eligible Employee. In all other cases, a former Participant may again become a Participant, if he or she then is an Eligible Employee, by following the procedure described in Subsection (b) above.

SECTION 5. EMPLOYEE CONTRIBUTIONS.

(a) **Commencement of Payroll Deductions.** A Participant may purchase shares of Stock under the Plan by means of payroll deductions or other approved contributions in form and substance satisfactory to the Committee. Payroll deductions or other approved contributions shall commence as soon as reasonably practicable after the Company has received the prescribed enrollment form. In jurisdictions where payroll deductions are not permitted under local law, Participants may purchase shares of Stock by making contributions in the form that is acceptable and approved by the Committee.

(b) **Amount of Payroll Deductions.** An Eligible Employee shall designate on the prescribed enrollment form the portion of his or her Compensation that he or she elects to have withheld for the purchase of Stock. Such portion shall be a whole percentage of the Eligible Employee's Compensation, but not less than 1% nor more than 15%.

(c) **Reducing Withholding Rate or Discontinuing Payroll Deductions.** If a Participant wishes to reduce his or her rate of payroll withholding, such Participant may do so by filing a new enrollment form with the Company at the prescribed location at any time. The new withholding rate shall be effective as soon as reasonably practicable after the Company has received such form. The new withholding rate may be 0% or any whole percentage of the Participant's Compensation, but not more than his or her old withholding rate. No Participant shall make more than two elections under this Subsection (c) during any Purchase Period. (In addition, employee contributions may be discontinued automatically pursuant to Section 9(b).)

(d) **Increasing Withholding Rate.** If a Participant wishes to increase his or her rate of payroll withholding, such Participant may do so by filing a new enrollment form with the Company at the prescribed location at any time. The new withholding rate may be effective on the first day of the next-upcoming Offering Period in which the Participant participates, provided that the Participant has filed the enrollment form with the Company at the prescribed location at least 10 business days (or such other period as the Committee or its designee may designate) prior to such day. The new withholding rate may be any whole percentage of the Participant's Compensation, but not less than 1% nor more than 15%. An increase in a Participant's rate of payroll withholding may not take effect during an Offering Period.

SECTION 6. WITHDRAWAL FROM THE PLAN.

(a) **Withdrawal.** A Participant may elect to withdraw from the Plan (or, if applicable, from an Offering Period) by filing the prescribed form with the Company at the prescribed location at any time before a Purchase Date. As soon as reasonably practicable

thereafter, payroll deductions or other approved contributions shall cease and the entire amount credited to the Participant's Plan Account with respect to such Offering Period shall be refunded to him or her in cash, without interest (except as otherwise required by the laws of the local jurisdiction). No partial withdrawals from an Offering Period shall be permitted.

(b) **Re-Enrollment After Withdrawal.** A former Participant who has withdrawn from the Plan shall not be a Participant until he or she re-enrolls in the Plan under Section 4(b). Re-enrollment may be effective only at the commencement of an Offering Period.

SECTION 7. CHANGE IN EMPLOYMENT STATUS.

(a) **Termination of Employment.** Termination of employment as an Eligible Employee for any reason, including death, shall be treated as an automatic withdrawal from the Plan under Section 6(a). (A transfer from one Participating Company to another shall not be treated as a termination of employment provided that each Participating Company is then participating in the same Offering Period.)

(b) **Leave of Absence.** For purposes of the Plan, employment shall not be deemed to terminate when the Participant goes on a military leave, a sick leave or another bona fide leave of absence, if the leave was approved by the Company in writing. Employment, however, shall be deemed to terminate on the first day following three months after the Participant goes on a leave, unless a contract or statute guarantees his or her right to return to work. Employment shall be deemed to terminate in any event when the approved leave ends, unless the Participant immediately returns to work.

(c) **Death.** In the event of the Participant's death, the amount credited to his or her Plan Account shall be paid to a beneficiary designated by him or her for this purpose on the prescribed form or, if none, to the Participant's estate. Such form shall be valid only if it was filed with the Company at the prescribed location before the Participant's death.

SECTION 8. PLAN ACCOUNTS AND PURCHASE OF SHARES.

(a) **Plan Accounts.** The Company shall maintain a Plan Account on its books in the name of each Participant. Whenever an amount is deducted from the Participant's Compensation under the Plan, such amount shall be credited to the Participant's Plan Account. Amounts credited to Plan Accounts shall not be trust funds and may be commingled with the Company's general assets and applied to general corporate purposes. Unless otherwise required by the laws of the local jurisdiction, no interest shall be credited to Plan Accounts.

(b) **Purchase Price.** The Purchase Price for each share of Stock purchased on a Purchase Date shall be the lower of:

(i) 85% of the Fair Market Value of such share on the first day of such Offering Period or, in the case of the first Offering Period under the Plan, 85% of the price at which one share of Stock is offered to the public in the IPO; or

(ii) 85% of the Fair Market Value of such share on the Purchase Date.

(c) **Number of Shares Purchased.** On each Purchase Date, each Participant shall be deemed to have elected to purchase the number of shares of Stock calculated in accordance with this Subsection (c), unless the Participant has previously elected to withdraw from the Offering Period in accordance with Section 6(a). The amount then in the Participant's Plan Account shall be divided by the Purchase Price, and the number of shares that results shall be purchased from the Company with the funds in the Participant's Plan Account. The foregoing number of shares of Stock purchasable by a Participant are subject to the limitations set forth in Section 9. The Committee may determine with respect to all Participants that any fractional share, as calculated under this Subsection (c), shall be (i) rounded down to the next lower whole share or (ii) credited as a fractional share.

(d) **Available Shares Insufficient.** In the event that the aggregate number of shares that all Participants elect to purchase with respect to a particular Purchase Period exceeds (i) the number of shares of Stock that were available under Section 3 above for sale under the Plan on the first day of the applicable Offering Period, or (ii) the number of shares that were available under Section 3 above for sale under the Plan on the applicable Purchase Date, then the number of shares to which each Participant is entitled shall be determined by multiplying the number of shares available for issuance by a fraction. The numerator of such fraction is the number of shares that such Participant has elected to purchase, and the denominator of such fraction is the number of shares that all Participants have elected to purchase. The Company may make a pro rata allocation of the shares available on the first day of an applicable Offering Period pursuant to the preceding sentence, notwithstanding any authorization of additional shares for issuance under the Plan by the Company's stockholders subsequent to such date. In the event of a pro-rata allocation under this Section (d), the Committee may determine in its discretion to continue all Offering Periods then in effect or terminate all Offering Periods then in effect pursuant to Section 14.

(e) **Issuance of Stock.** The shares of Stock purchased by a Participant under the Plan may be registered in the name of such Participant, or jointly in the name of such Participant and his or her spouse as joint tenants with the right of survivorship or as community property (with or without the right of survivorship). The Company may permit or require that shares be deposited directly with a broker designated by the Company or to a designated agent of the Company, and the Company may utilize electronic or automated methods of share transfer. The Company may require that shares be retained with such broker or agent for a designated period of time and/or may establish other procedures to permit tracking of disqualifying dispositions of such shares. (The two preceding sentences shall apply whether or not the Participant is required to pay income tax in the United States.)

(f) **Tax Withholding.** To the extent required by applicable federal, state, local or foreign law, a Participant shall make arrangements satisfactory to the Company for the satisfaction of any withholding tax obligations that arise in connection with the Plan. The Company shall not be required to issue any shares of Stock under the Plan until such obligations, if any, are satisfied.

(g) **Unused Cash Balances.** Subject to the final sentence of Section 8(c), an amount remaining in the Participant's Plan Account that represents the Purchase Price for any fractional share shall be carried over in the Participant's Plan Account to the next Purchase

Period. Any amount remaining in the Participant's Plan Account that represents the Purchase Price for whole shares that could not be purchased by reason of Subsections (c) or (d) above or Section 9(b) shall be refunded to the Participant in cash, without interest (except as otherwise required by the laws of the local jurisdiction).

(h) **Stockholder Approval.** Any other provision of the Plan notwithstanding, no shares of Stock shall be purchased under the Plan unless and until the Company's stockholders have approved the adoption of the Plan.

SECTION 9. PLAN LIMITATIONS.

(a) **Five Percent Limit.** Any other provision of the Plan notwithstanding, no Participant shall be granted a right to purchase Stock under the Plan if such Participant, immediately after his or her election to purchase such Stock, would own stock possessing more than 5% of the total combined voting power or value of all classes of stock of the Company or any parent or Subsidiary of the Company, determined in accordance with applicable tax law.

(b) **Dollar Limit.** Any other provision of the Plan notwithstanding, no Participant shall purchase Stock with a Fair Market Value in excess of the following limit:

(i) In the case of Stock purchased during an Offering Period that commenced in the current calendar year, the limit shall be equal to (A) $25,000 minus (B) the Fair Market Value of the Stock that the Participant previously purchased under the Plan in the current calendar year.

(ii) In the case of Stock purchased during an Offering Period that commenced in the immediately preceding calendar year, the limit shall be equal to (A) $50,000 minus (B) the Fair Market Value of the Stock that the Participant previously purchased under the Plan in the current calendar year and in the immediately preceding calendar year.

(iii) In the case of Stock purchased during an Offering Period that commenced in the second calendar year before the current calendar year, the limit shall be equal to (A) $75,000 minus (B) the Fair Market Value of the Stock that the Participant previously purchased under the Plan in the current calendar year and in the immediately preceding two calendar years.

For all purposes under this Subsection (b), (A) the Fair Market Value of Stock shall be determined as of the beginning of the Offering Period in which such Stock is purchased; and (B) this Plan shall be aggregated with any other employee stock purchase plans of the Company (or any parent or Subsidiary of the Company) described in Code Section 423. If a Participant is precluded by this Subsection (b) from purchasing additional Stock under the Plan, then his or her employee contributions shall automatically be discontinued and shall automatically resume at the beginning of the next Offering Period with a scheduled Purchase Date in the next calendar year, provided that he or she is an Eligible Employee at the beginning of such Offering Period.

(c) **Purchase Period Share Purchase Limit.** Any other provision of the Plan notwithstanding, no Participant shall purchase more than shares of Stock with respect to any Purchase Period; provided that the Committee may, for future Offering Periods, increase or decrease in its absolute discretion, the maximum number of shares of Stock that a Participant may purchase during each Purchase Period.

SECTION 10. RIGHTS NOT TRANSFERABLE.

The rights of any Participant under the Plan, or any Participant's interest in any Stock or moneys to which he or she may be entitled under the Plan, shall not be transferable by voluntary or involuntary assignment or by operation of law, or in any other manner other than by beneficiary designation or the laws of descent and distribution. If a Participant in any manner attempts to transfer, assign or otherwise encumber his or her rights or interest under the Plan, other than by beneficiary designation or the laws of descent and distribution, then such act shall be treated as an election by the Participant to withdraw from the Plan under Section 6(a).

SECTION 11. NO RIGHTS AS AN EMPLOYEE.

Nothing in the Plan or in any right granted under the Plan shall confer upon the Participant any right to continue in the employ of a Participating Company for any period of specific duration or interfere with or otherwise restrict in any way the rights of the Participating Companies or of the Participant, which rights are hereby expressly reserved by each, to terminate his or her employment at any time and for any reason, with or without cause.

SECTION 12. NO RIGHTS AS A STOCKHOLDER.

A Participant shall have no rights as a stockholder with respect to any shares of Stock that he or she may have a right to purchase under the Plan until such shares have been purchased on the applicable Purchase Date.

SECTION 13. Securities Law Requirements.

Shares of Stock shall not be issued, and the Company shall have no liability for failure to issue shares of Stock, under the Plan unless the issuance and delivery of such shares comply with (or are exempt from) all applicable requirements of law, including (without limitation) the Securities Act of 1933, as amended, the rules and regulations promulgated thereunder, state securities laws and regulations, and the regulations of any stock exchange or other securities market on which the Company's securities may then be traded.

SECTION 14. AMENDMENT OR DISCONTINUANCE.

(a) **General Rule.** The Committee, in its sole discretion, may amend, suspend, or terminate the Plan, or any part thereof, at any time and for any reason. If the Plan is terminated, the Committee, in its discretion, may elect to terminate all outstanding Offering Periods either immediately or upon completion of the purchase of shares of Stock on the next Purchase Date, or may elect to permit Offering Periods to expire in accordance with their terms

(and subject to any adjustment pursuant to Section 3(c) or (d)). If the Offering Periods are terminated prior to expiration, all amounts then credited to Participants' accounts which have not been used to purchase shares of Stock will be returned to the Participants (without interest thereon, except as otherwise required by the laws of the local jurisdiction) as soon as administratively practicable.

(b) **Committee's Discretion.** Without stockholder consent and without limiting Section 14(a), the Committee will be entitled to change the Offering Periods, limit the frequency and/or number of changes in the amount withheld during an Offering Period, establish the exchange ratio applicable to amounts withheld in a currency other than U.S. dollars, permit payroll withholding in excess of the amount designated by a Participant in order to adjust for delays or mistakes in the Company's processing of properly completed withholding elections, establish reasonable waiting and adjustment periods and/or accounting and crediting procedures to ensure that amounts applied toward the purchase of Stock for each Participant properly correspond with amounts withheld from the Participant's Compensation, and establish such other limitations or procedures as it determines in its sole discretion advisable which are consistent with the Plan.

(c) **Accounting Consideration.** In the event the Committee determines that the ongoing operation of the Plan may result in unfavorable financial accounting consequences, the Committee may, in its discretion and, to the extent necessary or desirable, modify, amend or terminate the Plan to reduce or eliminate such accounting consequence including, but not limited to:

(i) Amending the Plan to conform with the safe harbor definition under Financial Accounting Standards Board Accounting Standards Codification Topic 718, including with respect to an Offering Period underway at the time;

(ii) Altering the Purchase Price for any Offering Period including an Offering Period underway at the time of the change in Purchase Price;

(iii) Shortening any Offering Period by setting a new Purchase Date, including an Offering Period underway at the time of the Committee's action;

(iv) Reducing the maximum percentage of Compensation a Participant may elect to set aside as payroll deductions; and

(v) Reducing the maximum number of shares of Stock a Participant may purchase during any Purchase Period.

Such modifications or amendments will not require stockholder approval or the consent of any Plan Participants.

(d) **Stockholder Approval.** Except as provided in Section 3, any increase in the aggregate number of shares of Stock that may be issued under the Plan shall be subject to the approval of the Company's stockholders. In addition, any other amendment of the Plan shall be subject to the approval of the Company's stockholders to the extent required under Section 14(e) or by any applicable law or regulation.

(e) **Plan Termination.** The Plan shall terminate automatically 20 years after its adoption by the Board, unless (i) the Plan is extended by the Board and (ii) the extension is approved within 12 months by a vote of the stockholders of the Company.

SECTION 15. DEFINITIONS.

(a) **"Board"** means the Board of Directors of the Company, as constituted from time to time.

(b) **"Code"** means the Internal Revenue Code of 1986, as amended.

(c) **"Committee"** means a committee of the Board, as described in Section 2.

(d) **"Company"** means REGENXBIO Inc., a Delaware corporation.

(e) **"Compensation"** means (i) the total compensation paid in cash to a Participant by a Participating Company, including salaries, wages, bonuses, incentive compensation, commissions, overtime pay and shift premiums, plus (ii) any pre-tax contributions made by the Participant under Code Sections 401(k) or 125. "Compensation" shall exclude all non-cash items, moving or relocation allowances, cost-of-living equalization payments, car allowances, tuition reimbursements, imputed income attributable to cars or life insurance, severance pay, fringe benefits, contributions or benefits received under employee benefit plans, income attributable to equity compensation awards of the Company, and similar items. The Committee shall determine whether a particular item is included in Compensation.

(f) **"Corporate Reorganization"** means:

(i) The consummation of a merger or consolidation of the Company with or into another entity or any other corporate reorganization; or

(ii) The sale, transfer or other disposition of all or substantially all of the Company's assets or the complete liquidation or dissolution of the Company.

(g) **"Eligible Employee"** means a common law employee of a Participating Company who is customarily employed for more than five months per calendar year and at least 20 hours per week. The foregoing notwithstanding, an individual shall not be considered an Eligible Employee if his or her participation in the Plan is prohibited by the law of any country that has jurisdiction over him or her. In addition, the Committee may determine prior to the commencement of an Offering Period not to exclude part-time employees or exclude employees whose customary employment is for fewer hours per week or fewer months in a calendar year; provided that such terms are applied in an identical manner to all employees of every Participating Company in such Offering Period.

(h) **"Exchange Act"** means the Securities Exchange Act of 1934, as amended.

(i) **"Fair Market Value"** means the price at which Stock was last sold in the principal U.S. market for the Stock on the applicable date or, if the applicable date was not a trading day, on the last trading day prior to the applicable date. If Stock is no longer traded on a

public U.S. securities market, the Fair Market Value shall be determined by the Committee in good faith on such basis as it deems appropriate. The Committee's determination shall be conclusive and binding on all persons.

(j) **"IPO"** means the Company's initial offering of Stock to the public.

(k) **"IPO Date"** means the effective date of the registration statement filed by the Company with the Securities and Exchange Commission for its initial offering of Stock to the public.

(l) **"Offering Period"** means any period, including as the context requires Base Offering Periods and Additional Offering Periods, with respect to which the right to purchase Stock may be granted under the Plan, as determined pursuant to Section 4(a).

(m) **"Participant"** means an Eligible Employee who participates in the Plan or any Sub-Plan, as provided in Section 4.

(n) **"Participating Company"** means (i) the Company and (ii) each present or future Subsidiary designated by the Committee as a Participating Company.

(o) **"Plan"** means this REGENXBIO Inc. 2015 Employee Stock Purchase Plan, as it may be amended from time to time.

(p) **"Plan Account"** means the account established for each Participant pursuant to Section 8(a).

(q) **"Purchase Date"** means the last trading day of a Purchase Period.

(r) **"Purchase Period"** means a period within an Offering Period (which for an Offering Period with only a single Purchase Period would be coterminous with the Offering Period) during which contributions may be made toward the purchase of Stock under the Plan, as determined pursuant to Section 4(a).

(s) **"Purchase Price"** means the price at which Participants may purchase Stock under the Plan, as determined pursuant to Section 8(b).

(t) **"Stock"** means the Common Stock of the Company.

(u) **"Subsidiary"** means any corporation (other than the Company) in an unbroken chain of corporations beginning with the Company, if each of the corporations other than the last corporation in the unbroken chain owns stock possessing 50% or more of the total combined voting power of all classes of stock in one of the other corporations in such chain.

This Employment Agreement (this "Agreement") is entered into as of June 30, 2015, by and between Kenneth T. Mills (the "Employee") and REGENXBIO Inc., a Delaware corporation (the "Company").

1. **Position.**

(a) During your employment with the Company pursuant to this Agreement, you will hold the title of President and Chief Executive Officer. As the President and Chief Executive Officer, you will report directly to the Company's Board of Directors (the "Board"). By signing this Agreement, you agree to perform the duties and fulfill the responsibilities normally inherent in the position of President and Chief Executive Officer and such other duties and responsibilities as may from time to time reasonably be assigned to you. In addition, you shall serve on the Board. In the event that you cease to be employed by the Company, you shall promptly resign from the Board if requested to do so by a majority of the Board.

(b) You agree that, to the best of your ability and experience, you will at all times loyally and conscientiously perform all of the duties and obligations required of and from you pursuant to the express and implicit terms hereof, and to the reasonable satisfaction of the Company. During the term of your employment with the Company, you further agree that (i) you will devote substantially all of your business time and attention to the business of the Company, (ii) the Company will be entitled to all of the benefits and profits arising from or incident to all such business services, (iii) you will not render commercial or professional services of any nature to any person or organization outside of the Company without the prior written approval of the Board, and (iv) you will not directly or indirectly engage or participate in any business that is competitive in any manner with the business of the Company. Notwithstanding the above, you may continue, on your own time, at your own expense and so as to not interfere with your duties and responsibilities at the Company to (i) serve as a member of an advisory board or board of directors of other companies that are not competitive in any manner with the Company, (ii) accept speaking or presentation engagements in exchange for honoraria, and (iii) participate in civic, educational, charitable or fraternal organizations. This Agreement does not prevent you from owning no more than one percent (1%) of the outstanding equity securities of a corporation whose stock is listed on a national stock exchange and is a competitor or potential competitor of the Company.

2. **Offer Letter.** Subject to the consummation of the initial public offering of the Company's common stock (the "IPO"), this Agreement shall supersede and replace the offer letter you entered into with the Company, dated February 1, 2015, other than the Proprietary Information and Inventions Agreement, which shall continue in full force and effect. If the IPO is not consummated, then this Agreement shall be null and void.

3. **Compensation**.

(a) ***Base Salary.*** You will be paid a monthly salary at a rate of $41,666.67, which is equivalent to $500,000 on an annualized basis, which will be paid semi-monthly in accordance with the Company's standard payroll procedures.

(b) ***Incentive Bonus.*** You shall be eligible for an annual incentive bonus with a target amount equal to 50% of your Base Salary (the "Annual Target Bonus"). Any bonus for the fiscal year in which the IPO is consummated shall be prorated between your pre-IPO and post- IPO target bonus percentages. Such bonus (if any) shall be awarded based on objective and/or subjective criteria established in advance by the Board or the Compensation Committee of the Board (the "Compensation Committee"). The Company shall determine when to pay to you any earned incentive bonus, but shall in no event pay such bonus more than 2 ½ months following the close of the fiscal year for which it is earned. Except as provided in Section 9, any earned incentive bonus shall be paid to you only if you are employed by the Company at the time of payment. The determinations of the Board or the Compensation Committee with respect to such bonus shall be final and binding.

(c) ***Annual Review.*** Your compensation will be reviewed by the Board or Compensation Committee annually.

For purposes of this Agreement, "Cause" shall mean (i) the conviction of, or the entering a plea of guilty or no contest (or pleading or accepting deferred adjudication or receiving unadjudicated probation) to or for, any felony or any crime involving moral turpitude, (ii) the commission of a material breach of any of the covenants, terms and provisions of this Agreement or the Proprietary Information and Inventions Agreement you will enter into as a condition of your employment, (iii) the commission of an act of fraud, embezzlement, misappropriation, willful misconduct or breach of fiduciary duty against the Company or other similar conduct materially harmful or potentially materially harmful to the Company's best interest, as determined by the Board, in its reasonable sole discretion, (iv) the failure to perform assigned duties or responsibilities as the President and Chief Executive Officer (other than a failure resulting from

Disability (as defined below)); provided, however, that you shall be given written notice of, and shall have a ten (10) day period following such notice to cure a failure or refusal under this subclause (iv)), or (v) the violation of any federal or state law or regulation applicable to the Company's business.

For purposes of this Agreement, "Good Reason" shall mean the occurrence of any of the following, without your written consent: (i) a significant reduction in your duties or responsibilities or your removal from the position contemplated by this Agreement, unless you are assigned comparable duties or responsibilities or employed in a different position, respectively; (ii) a significant reduction in the number of employees who report directly to you; (ii) a significant reduction (thirty percent (30%) or more) in your base salary as in effect immediately prior to such reduction; (iii) a significant reduction in the type or level of employee benefits to which you are entitled that results in a significant reduction to your overall benefits package (other than a reduction of such employee benefits applicable to all Company employees), as determined by the Board in its sole discretion; or (iv) relocation of your principal workplace by more than 35 miles. Good reason will not be deemed to occur unless you give the Company written notice of the condition within 90 days after the condition comes into existence and the Company fails to remedy the condition with 30 days after receiving said notice.

4. **Benefits.** As an employee of the Company, you will also be eligible to receive certain employee benefits including paid time off and medical, dental, life, and long term disability insurance. You will also be eligible to participate in our 401(k) savings plan.

5. **At-Will Employment; Proprietary Information and Inventions Agreement.** Employment with the Company is for no specific period of time. Your employment with the Company is "at will," meaning that either you or the Company may terminate your employment at any time and for any reason, with or without cause. In addition, you should note that the Company may modify your job title, salary or benefits at its discretion. You agree and affirm that your continued employment with the Company and this Agreement are contingent upon your agreement to comply with the Proprietary Information and Inventions Agreement, previously executed, a copy of which is attached hereto as Exhibit A.

6. **Indemnification.** The Company shall indemnify you to the fullest extent allowed by law, in accordance with the terms of the Company's Certificate of Incorporation and Bylaws. You shall become a party to the Company's standard Indemnification Agreement.

7. **Evidence of Employment Eligibility.** For purposes of federal immigration law, you will be required to provide to the Company documentary evidence of your identity and eligibility for employment in the United States. Such documentation must be provided to us within three (3) business days of your Start Date, or our employment relationship with you may be terminated.

8. Company Handbook. As a Company employee, you will be expected to abide by the Company's rules of operation and standards of conduct. Specifically, you will be required to sign an acknowledgment that you have read and that you understand such rules and standards, which are set forth in the Company Handbook.

9. **Termination of Employment and Severance Benefits.**

(a) ***Preconditions*.** Any other provision of this Agreement notwithstanding, the remaining Subsections of this Section 9 shall not apply unless each of the following requirements is satisfied:

(i) You have executed a general release of all known and unknown claims that you may then have against the Company or persons affiliated with the Company in a form prescribed by the Company, without alterations. You shall execute and return the release on or before the date specified by the Company in the prescribed form (the "Release Deadline"). The Release Deadline shall in no event be later than sixty (60) days after your termination of employment. If the 60 day period described in the prior sentence spans two calendar years, then the payments will begin on the first payroll period, following expiration of the revocation period, in the second calendar year. If you fail to return the release on or before the Release Deadline, or if you revoke the release, then you shall not be entitled to the benefits described in this Section 9; and

(ii) You have returned all property of the Company in your possession.

(b) ***Termination of Employment*.** Except for the severance benefits provided below, the Company's obligations under this Agreement may be terminated upon the occurrence of any of the following events:

(i) The Company's determination in good faith that it is terminating you for Cause ("Termination for Cause");

(ii) The Company's determination that it is terminating you without Cause, which determination may be made by the Company at any time at the Company's sole discretion, for any or no reason ("Termination Without Cause");

(iii) Thirty (30) days following delivery by you of a written notice to the Company stating that you are electing to terminate your employment with the Company ("Voluntary Termination");

(iv) Following your death or Disability (as defined below); or

(v) Your determination in good faith that you are electing to terminate your employment with the Company for Good Reason.

(c) ***Severance Benefits.*** You shall be entitled to receive severance benefits upon termination of employment only as set forth in this Section 9(c):

(i) ***Voluntary Termination.*** In the event of a Voluntary Termination you shall not be entitled to receive payment of any severance benefits. You will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Voluntary Termination and your benefits will be continued under the Company's then existing benefit plans and policies to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of your Voluntary Termination and in accordance with applicable law.

(ii) ***Involuntary Termination/No Change in Control.*** If your employment is terminated under Section 9(b)(ii) or (v) above (such termination, an "Involuntary Termination"), you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Severance Benefits"). The Severance Benefits shall consist of salary continuation for twelve (12) months of monthly Base Salary amounts; provided that if you become employed during this period, then the Company's obligation to pay Severance Benefits shall cease upon commencement of your new employment. If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is twelve (12) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to

avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings.

(iii) ***Involuntary Termination/ Change in Control.*** If your employment is terminated in an Involuntary Termination immediately prior to or in the eighteen (18) months following a Change in Control, you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Change in Control Severance Benefits"). The Change in Control Severance Benefits shall consist of (A) salary continuation for eighteen (18) months' of monthly Base Salary and (B) a monthly amount equal to your Annual Target Bonus multiplied by 1.5 then divided by eighteen (18). If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is eighteen (18) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings. If immediately prior to or following a Change in Control (as defined in the Company's 2015 Equity Incentive Plan), your employment with the Company (or the Company's successor) is terminated in an Involuntary Termination during the remaining vesting period of the options then outstanding as of the date of closing of the Change in Control (the "Options"), then one hundred percent (100%) of the unvested shares subject to the Options shall automatically vest.

(iv) ***Termination for Cause*.** In the event of your Termination for Cause, you will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Termination for Cause.

(v) ***Termination by Reason of Death or Disability*.** In the event that your employment with the Company terminates as a result of your death or Disability (as defined below), you or your estate or representative will receive all salary and unpaid vacation accrued as of the date of your death or Disability, all severance benefits payable under Section 9(b)(ii) above and any other benefits payable under the Company's then existing benefit plans and policies, to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of death or Disability and in accordance with applicable law. For purposes of this Agreement, "Disability" shall mean that you have been unable to perform your duties hereunder as the result of physical or mental incapacity lasting at least forty-five (45) consecutive calendar days or ninety (90) calendar days during any consecutive twelve-month period, after which time such incapacity is determined to be permanent by a physician chosen by the Company and its insurers and acceptable to you or to your legal representative (with such agreement on acceptability not to be unreasonably withheld).

10. **Tax Matters.**

(a) ***Withholding.*** All forms of compensation referred to in this Agreement are subject to reduction to reflect applicable withholding and payroll taxes and other deductions required by law.

(b) ***Tax Advice.*** You are encouraged to obtain your own tax advice regarding your compensation from the Company. You agree that the Company does not have a duty to design its compensation policies in a manner that minimizes your tax liabilities, and you will not make any claim against the Company or the Board related to tax liabilities arising from your compensation.

(c) ***280G.*** Notwithstanding anything contained in this Agreement to the contrary, if any of the payments or benefits received or to be received by you pursuant to this Agreement when taken together with payments and benefits provided to you under any other plans, contracts, or arrangements with the Company (all such payments and benefits, the "Total Payments"), would be subject to any excise tax imposed under Code Section 4999 (together with any interest or penalties, the "Excise Tax"), then such Total Payments will be reduced to the extent necessary so that no portion thereof will be subject to the Excise Tax;

provided, however, that if you would receive in the aggregate greater value (as determined under Code Section 280G and the regulations thereunder) on an after tax basis if the Total Payments were not subject to such reduction, then no such reduction will be made. To effect the reduction described herein, if applicable, the Company will first reduce or eliminate the payments and benefits provided under this Agreement. All calculations required to be made under this Section will be made by the Company's independent public accountants, subject to the right of your representative to review the same.

11. **Miscellaneous Provisions.**

(a) ***Choice of Law*.** The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the State of Maryland, without giving effect to the principles of conflicts of law.

(b) ***Counterparts*.** This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together will constitute one and the same instrument.

(c) ***Severability*.** In the event that any provision hereof becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable, or void, this Agreement shall continue in full force and effect without such provision.

(d) ***Acknowledgment*.** You acknowledge that you have had the opportunity to discuss this matter with and obtain advice from your private attorney, have had sufficient time to read, and have carefully read and fully understand, all the provisions of this Agreement, and are knowingly and voluntarily entering into this Agreement.

(e) **Arbitration.** Any controversy or claim arising out of this Agreement and any and all claims relating to the Employee's Employment with the Company shall be settled by final and binding arbitration. The arbitration shall take place in Washington, D.C., or, at the Employee's option, the County in which the Employee primarily worked when the arbitrable dispute or claim first arose. The arbitration shall be administered by the American Arbitration Association under its National Rules for the Resolution of Employment Disputes. Any award or finding shall be confidential. The Employee and the Company agree to provide one another with reasonable access to documents and witnesses in connection with the resolution of the dispute. The Employee and the Company shall share the costs of arbitration equally. Each party shall be responsible for its own attorneys' fees, and the arbitrator may not award attorneys' fees unless

a statute or contract at issue specifically authorizes such an award. This Section 11(e) shall not apply to claims for workers' compensation benefits or unemployment insurance benefits. This Section 11(e) also shall not apply to claims concerning the ownership, validity, infringement, misappropriation, disclosure, misuse or enforceability of any confidential information, patent right, copyright, mask work, trademark or any other trade secret or intellectual property held or sought by either the Employee or the Company (whether or not arising under the Proprietary Information and Inventions Agreement between the Employee and the Company).

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IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by its duly authorized officer, as of the day and year first above written.

REGENXBIO INC.

By: /s/ Vittal Vasista

Name: Vittal Vasista

Title: CFO

EMPLOYEE

By: /s/ Kenneth T. Mills

Date: June 30, 2015

This Employment Agreement (this "Agreement") is entered into as of June 30, 2015, by and between Stephen Yoo (the "Employee") and REGENXBIO Inc., a Delaware corporation (the "Company").

1. **Position.**

   (a) During your employment with the Company pursuant to this Agreement, you will hold the title of Chief Medical Officer. As the Chief Medical Officer you shall report directly to the Company's President and Chief Executive Officer. By signing this Agreement, you agree to perform the duties and fulfill the responsibilities normally inherent in the position of Chief Medical Officer and such other duties and responsibilities as may from time to time reasonably be assigned to you.

   (b) You agree that, to the best of your ability and experience, you will at all times loyally and conscientiously perform all of the duties and obligations required of and from you pursuant to the express and implicit terms hereof, and to the reasonable satisfaction of the Company. During the term of your employment with the Company, you further agree that (i) you will devote substantially all of your business time and attention to the business of the Company, (ii) the Company will be entitled to all of the benefits and profits arising from or incident to all such business services, (iii) you will not render commercial or professional services of any nature to any person or organization outside of the Company without the prior written approval of the Board, and (iv) you will not directly or indirectly engage or participate in any business that is competitive in any manner with the business of the Company. Notwithstanding the above, you may continue, on your own time, at your own expense and so as to not interfere with your duties and responsibilities at the Company to (i) serve as a member of an advisory board or board of directors of other companies that are not competitive in any manner with the Company, (ii) accept speaking or presentation engagements in exchange for honoraria, and (iii) participate in civic, educational, charitable or fraternal organizations. This Agreement does not prevent you from owning no more than one percent (1%) of the outstanding equity securities of a corporation whose stock is listed on a national stock exchange and is a competitor or potential competitor of the Company.

2. **Offer Letter.** Subject to the consummation of the initial public offering of the Company's common stock (the "IPO"), this Agreement shall supersede and replace the offer letter you entered into with the Company, dated August 15, 2014, other than the Proprietary Information and Inventions Agreement, which shall continue in full force and effect. If the IPO is not consummated, then this Agreement shall be null and void.

3. **Compensation**.

(a) ***Base Salary.*** You will be paid a monthly salary at a rate of $28,333.33, which is equivalent to $340,000 on an annualized basis, which will be paid semi-monthly in accordance with the Company's standard payroll procedures.

(b) ***Incentive Bonus.*** You shall be eligible for an annual incentive bonus with a target amount equal to 35% of your Base Salary (the "Annual Target Bonus"). Such bonus (if any) shall be awarded based on objective and/or subjective criteria established in advance by the Board or the Compensation Committee of the Board (the "Compensation Committee"). Any bonus for the fiscal year in which the IPO is consummated shall be prorated between your pre-IPO and post- IPO target bonus percentages. Any incentive bonus earned by you for any fiscal year shall only be paid to you only if you remain employed by the Company through the end of the fiscal year for which the bonus is earned. The Company shall determine when to pay to you any earned incentive bonus, but shall in no event pay such bonus more than 2 ½ months following the close of the fiscal year for which it is earned. Except as provided in Section 9, any earned incentive bonus shall be paid to you only if you are employed by the Company at the time of payment. The determinations of the Board or the Compensation Committee with respect to such bonus shall be final and binding.

(c) ***Annual Review.*** Your compensation will be reviewed by the Board or Compensation Committee annually.

For purposes of this Agreement, "Cause" shall mean (i) the conviction of, or the entering a plea of guilty or no contest (or pleading or accepting deferred adjudication or receiving unadjudicated probation) to or for, any felony or any crime involving moral turpitude, (ii) the commission of a material breach of any of the covenants, terms and provisions of this Agreement or the Proprietary Information and Inventions Agreement you will enter into as a condition of your employment, (iii) the commission of an act of fraud, embezzlement, misappropriation, willful misconduct or breach of fiduciary duty against the Company or other similar conduct materially harmful or potentially materially harmful to the Company's best interest, as determined by the Board, in its reasonable sole discretion, (iv) the failure to perform assigned duties or responsibilities as the Chief Medical Officer (other than a failure resulting from Disability (as defined below)); provided, however, that you shall be given written

notice of, and shall have a ten (10) day period following such notice to cure a failure or refusal under this subclause (iv)), or (v) the violation of any federal or state law or regulation applicable to the Company's business.

For purposes of this Agreement, "Good Reason" shall mean the occurrence of any of the following, without your written consent: (i) a significant reduction in your duties or responsibilities or your removal from the position contemplated by this Agreement, unless you are assigned comparable duties or responsibilities or employed in a different position, respectively; (ii) a significant reduction in the number of employees who report directly to you; (ii) a significant reduction (thirty percent (30%) or more) in your base salary as in effect immediately prior to such reduction; or (iii) a significant reduction in the type or level of employee benefits to which you are entitled that results in a significant reduction to your overall benefits package (other than a reduction of such employee benefits applicable to all Company employees), as determined by the Company's Board of Directors in its sole discretion; or (iv) relocation of your principal workplace by more than 35 miles. Good reason will not be deemed to occur unless you give the Company written notice of the condition within 90 days after the condition comes into existence and the Company fails to remedy the condition with 30 days after receiving said notice.

4. **Benefits.** As an employee of the Company, you will also be eligible to receive certain employee benefits including paid time off and medical, dental, life, and long term disability insurance. You will also be eligible to participate in our 401(k) savings plan.

5. **At-Will Employment; Proprietary Information and Inventions Agreement.** Employment with the Company is for no specific period of time. Your employment with the Company is "at will," meaning that either you or the Company may terminate your employment at any time and for any reason, with or without cause. In addition, you should note that the Company may modify your job title, salary or benefits at its discretion. You agree and affirm that your continued employment with the Company is contingent upon your agreement to comply with the Proprietary Information and Inventions Agreement, previously executed, a copy of which is attached hereto as Exhibit A.

6. **Indemnification.** The Company shall indemnify you to the fullest extent allowed by law, in accordance with the terms of the Company's Certificate of Incorporation and Bylaws. You shall become a party to the Company's standard Indemnification Agreement.

7. **Evidence of Employment Eligibility.** For purposes of federal immigration law, you will be required to provide to the Company documentary evidence of your identity and eligibility for employment in the United States. Such documentation must be provided to us within three (3) business days of your Start Date, or our employment relationship with you may be terminated.

8. Company Handbook. As a Company employee, you will be expected to abide by the Company's rules of operation and standards of conduct. Specifically, you will be required to sign an acknowledgment that you have read and that you understand such rules and standards, which are set forth in the Company Handbook.

9. **Termination of Employment and Severance Benefits.**

(a) ***Preconditions*.** Any other provision of this Agreement notwithstanding, the remaining Subsections of this Section 9 shall not apply unless each of the following requirements is satisfied:

(i) You have executed a general release of all known and unknown claims that you may then have against the Company or persons affiliated with the Company in a form prescribed by the Company, without alterations. You shall execute and return the release on or before the date specified by the Company in the prescribed form (the "Release Deadline"). The Release Deadline shall in no event be later than sixty (60) days after your termination of employment. If the 60 day period described in the prior sentence spans two calendar years, then the payments will begin on the first payroll period, following expiration of the revocation period, in the second calendar year. If you fail to return the release on or before the Release Deadline, or if you revoke the release, then you shall not be entitled to the benefits described in this Section 9; and

(ii) You have returned all property of the Company in your possession.

(b) ***Termination of Employment*.** Except for the severance benefits provided below, the Company's obligations under this Agreement may be terminated upon the occurrence of any of the following events:

(i) The Company's determination in good faith that it is terminating you for Cause ("Termination for Cause");

(ii) The Company's determination that it is terminating you without Cause, which determination may be made by the Company at any time at the Company's sole discretion, for any or no reason ("Termination Without Cause");

(iii) Thirty (30) days following delivery by you of a written notice to the Company stating that you are electing to terminate your employment with the Company ("Voluntary Termination");

(iv) Following your death or Disability (as defined below); or

(v) Your determination in good faith that you are electing to terminate your employment with the Company for Good Reason.

(c) ***Severance Benefits.*** You shall be entitled to receive severance benefits upon termination of employment only as set forth in this Section 9(c):

(i) ***Voluntary Termination.*** In the event of a Voluntary Termination you shall not be entitled to receive payment of any severance benefits. You will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Voluntary Termination and your benefits will be continued under the Company's then existing benefit plans and policies to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of your Voluntary Termination and in accordance with applicable law.

(ii) ***Involuntary Termination/No Change in Control.*** If your employment is terminated under Section 9(b)(ii) or (v) above (such termination, an "Involuntary Termination"), you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Severance Benefits"). The Severance Benefits shall consist of salary continuation for nine (9) months of monthly Base Salary amounts; provided that if you become employed during this period, then the Company's obligation to pay Severance Benefits shall cease upon commencement of your new employment. If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is nine (9) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the

contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings.

(iii) ***Involuntary Termination/ Change in Control.*** If your employment is terminated in an Involuntary Termination immediately prior to or in the eighteen months following a Change in Control, you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Change in Control Severance Benefits"). The Change in Control Severance Benefits shall consist of salary continuation for twelve (12) months' of monthly Base Salary plus a monthly amount equal to your Annual Target Bonus divided by twelve (12). If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is twelve (12) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings. If immediately prior to or following a Change in Control (as defined in the Company's 2015 Equity Incentive Plan), your employment with the Company (or the Company's successor) is terminated in an Involuntary Termination during the remaining vesting period of the options then outstanding as of the date of closing of the Change in Control (the "Options"), then one hundred percent (100%) of the unvested shares subject to the Options shall automatically vest.

(iv) ***Termination for Cause*.** In the event of your Termination for Cause, you will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Termination for Cause.

(v) ***Termination by Reason of Death or Disability*.** In the event that your employment with the Company terminates as a result of your death or Disability (as defined below), you or your estate or representative will receive all salary and unpaid vacation accrued as of the date of your death or Disability, all severance benefits payable under Section 9(b)(ii) above and any other benefits payable under the Company's then existing benefit plans and policies, to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of death or Disability and in accordance with applicable law. For purposes of this Agreement, "Disability" shall mean that you have been unable to perform your duties hereunder as the result of physical or mental incapacity lasting at least forty-five (45) consecutive calendar days or ninety (90) calendar days during any consecutive twelve-month period, after which time such incapacity is determined to be permanent by a physician chosen by the Company and its insurers and acceptable to you or to your legal representative (with such agreement on acceptability not to be unreasonably withheld).

10. **Tax Matters.**

(a) ***Withholding.*** All forms of compensation referred to in this Agreement are subject to reduction to reflect applicable withholding and payroll taxes and other deductions required by law.

(b) ***Tax Advice.*** You are encouraged to obtain your own tax advice regarding your compensation from the Company. You agree that the Company does not have a duty to design its compensation policies in a manner that minimizes your tax liabilities, and you will not make any claim against the Company or the Board related to tax liabilities arising from your compensation.

(c) ***280G.*** Notwithstanding anything contained in this Agreement to the contrary, if any of the payments or benefits received or to be received by you pursuant to this Agreement when taken together with payments and benefits provided to you under any other plans, contracts, or arrangements with the Company (all such payments and benefits, the "Total Payments"), would be subject to any excise tax imposed under Code Section 4999 (together with any interest or penalties, the "Excise Tax"), then such Total Payments will be reduced to the extent necessary so that no portion thereof will be subject to the Excise Tax;

provided, however, that if you would receive in the aggregate greater value (as determined under Code Section 280G and the regulations thereunder) on an after tax basis if the Total Payments were not subject to such reduction, then no such reduction will be made. To effect the reduction described herein, if applicable, the Company will first reduce or eliminate the payments and benefits provided under this Agreement. All calculations required to be made under this Section will be made by the Company's independent public accountants, subject to the right of your representative to review the same.

11. **Miscellaneous Provisions.**

(a) ***Choice of Law*.** The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the State of Maryland, without giving effect to the principles of conflicts of law.

(b) ***Counterparts*.** This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together will constitute one and the same instrument.

(c) ***Severability*.** In the event that any provision hereof becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable, or void, this Agreement shall continue in full force and effect without such provision.

(d) ***Acknowledgment*.** You acknowledge that you have had the opportunity to discuss this matter with and obtain advice from your private attorney, have had sufficient time to read, and have carefully read and fully understand, all the provisions of this Agreement, and are knowingly and voluntarily entering into this Agreement.

(e) **Arbitration.** Any controversy or claim arising out of this Agreement and any and all claims relating to the Employee's Employment with the Company shall be settled by final and binding arbitration. The arbitration shall take place in Washington, D.C., or, at the Employee's option, the County in which the Employee primarily worked when the arbitrable dispute or claim first arose. The arbitration shall be administered by the American Arbitration Association under its National Rules for the Resolution of Employment Disputes. Any award or finding shall be confidential. The Employee and the Company agree to provide one another with reasonable access to documents and witnesses in connection with the resolution of the dispute. The Employee and the Company shall share the costs of arbitration equally. Each party shall be responsible for its own attorneys' fees, and the arbitrator may not award attorneys' fees unless

a statute or contract at issue specifically authorizes such an award. This Section 11(e) shall not apply to claims for workers' compensation benefits or unemployment insurance benefits. This Section 11(e) also shall not apply to claims concerning the ownership, validity, infringement, misappropriation, disclosure, misuse or enforceability of any confidential information, patent right, copyright, mask work, trademark or any other trade secret or intellectual property held or sought by either the Employee or the Company (whether or not arising under the Proprietary Information and Inventions Agreement between the Employee and the Company).

[*The remainder of this page intentionally left blank*]

IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by its duly authorized officer, as of the day and year first above written.

REGENXBIO INC.

By: /s/ Kenneth T. Mills

Name: Kenneth T. Mills

Title: President and CEO

EMPLOYEE

By: /s/ Stephen Yoo

Date: June 30, 2015

This Employment Agreement (this "Agreement") is entered into as of June 30, 2015, by and between Vittal K. Vasista (the "Employee") and REGENXBIO Inc., a Delaware corporation (the "Company").

1. **Position.**

   (a) During your employment with the Company pursuant to this Agreement, you will hold the title of Chief Financial Officer. As the Chief Financial Officer you shall report directly to the Company's President and Chief Executive Officer. By signing this Agreement, you agree to perform the duties and fulfill the responsibilities normally inherent in the position of Chief Financial Officer and such other duties and responsibilities as may from time to time reasonably be assigned to you.

   (b) You agree that, to the best of your ability and experience, you will at all times loyally and conscientiously perform all of the duties and obligations required of and from you pursuant to the express and implicit terms hereof, and to the reasonable satisfaction of the Company. During the term of your employment with the Company, you further agree that (i) you will devote substantially all of your business time and attention to the business of the Company, (ii) the Company will be entitled to all of the benefits and profits arising from or incident to all such business services, (iii) you will not render commercial or professional services of any nature to any person or organization outside of the Company without the prior written approval of the Board, and (iv) you will not directly or indirectly engage or participate in any business that is competitive in any manner with the business of the Company. Notwithstanding the above, you may continue, on your own time, at your own expense and so as to not interfere with your duties and responsibilities at the Company to (i) serve as a member of an advisory board or board of directors of other companies that are not competitive in any manner with the Company, (ii) accept speaking or presentation engagements in exchange for honoraria, and (iii) participate in civic, educational, charitable or fraternal organizations. This Agreement does not prevent you from owning no more than one percent (1%) of the outstanding equity securities of a corporation whose stock is listed on a national stock exchange and is a competitor or potential competitor of the Company.

2. **Offer Letter.** Subject to the consummation of the initial public offering of the Company's common stock (the "IPO"), this Agreement shall supersede and replace the offer letter you entered into with the Company, dated February 1, 2015, other than the Proprietary Information and Inventions Agreement, which shall continue in full force and effect. If the IPO is not consummated, then this Agreement shall be null and void.

3. **Compensation**.

(a) ***Base Salary.*** You will be paid a monthly salary at a rate of $26,250, which is equivalent to $315,000 on an annualized basis, which will be paid semi-monthly in accordance with the Company's standard payroll procedures.

(b) ***Incentive Bonus.*** You shall be eligible for an annual incentive bonus with a target amount equal to 35% of your Base Salary (the "Annual Target Bonus"). Such bonus (if any) shall be awarded based on objective and/or subjective criteria established in advance by the Board or the Compensation Committee of the Board (the "Compensation Committee"). Any bonus for the fiscal year in which the IPO is consummated shall be prorated between your pre-IPO and post- IPO target bonus percentages. Any incentive bonus earned by you for any fiscal year shall only be paid to you only if you remain employed by the Company through the end of the fiscal year for which the bonus is earned. The Company shall determine when to pay to you any earned incentive bonus, but shall in no event pay such bonus more than 2 ½ months following the close of the fiscal year for which it is earned. Except as provided in Section 9, any earned incentive bonus shall be paid to you only if you are employed by the Company at the time of payment. The determinations of the Board or the Compensation Committee with respect to such bonus shall be final and binding.

(c) ***Annual Review.*** Your compensation will be reviewed by the Board or Compensation Committee annually.

For purposes of this Agreement, "Cause" shall mean (i) the conviction of, or the entering a plea of guilty or no contest (or pleading or accepting deferred adjudication or receiving unadjudicated probation) to or for, any felony or any crime involving moral turpitude, (ii) the commission of a material breach of any of the covenants, terms and provisions of this Agreement or the Proprietary Information and Inventions Agreement you will enter into as a condition of your employment, (iii) the commission of an act of fraud, embezzlement, misappropriation, willful misconduct or breach of fiduciary duty against the Company or other similar conduct materially harmful or potentially materially harmful to the Company's best interest, as determined by the Board, in its reasonable sole discretion, (iv) the failure to perform assigned duties or responsibilities as the Chief Financial Officer (other than a failure resulting from Disability (as defined below)); provided, however, that you shall be given written

notice of, and shall have a ten (10) day period following such notice to cure a failure or refusal under this subclause (iv)), or (v) the violation of any federal or state law or regulation applicable to the Company's business.

For purposes of this Agreement, "Good Reason" shall mean the occurrence of any of the following, without your written consent: (i) a significant reduction in your duties or responsibilities or your removal from the position contemplated by this Agreement, unless you are assigned comparable duties or responsibilities or employed in a different position, respectively; (ii) a significant reduction in the number of employees who report directly to you; (ii) a significant reduction (thirty percent (30%) or more) in your base salary as in effect immediately prior to such reduction; (iii) a significant reduction in the type or level of employee benefits to which you are entitled that results in a significant reduction to your overall benefits package (other than a reduction of such employee benefits applicable to all Company employees), as determined by the Company's Board of Directors in its sole discretion; or (iv) relocation of your principal workplace by more than 35 miles. Good reason will not be deemed to occur unless you give the Company written notice of the condition within 90 days after the condition comes into existence and the Company fails to remedy the condition with 30 days after receiving said notice.

4. **Benefits.** As an employee of the Company, you will also be eligible to receive certain employee benefits including paid time off and medical, dental, life, and long term disability insurance. You will also be eligible to participate in our 401(k) savings plan.

5. **At-Will Employment; Proprietary Information and Inventions Agreement.** Employment with the Company is for no specific period of time. Your employment with the Company is "at will," meaning that either you or the Company may terminate your employment at any time and for any reason, with or without cause. In addition, you should note that the Company may modify your job title, salary or benefits at its discretion. You agree and affirm that your continued employment with the Company and this Agreement are contingent upon your agreement to comply with the Proprietary Information and Inventions Agreement, previously executed, a copy of which is attached hereto as Exhibit A.

6. **Indemnification.** The Company shall indemnify you to the fullest extent allowed by law, in accordance with the terms of the Company's Certificate of Incorporation and Bylaws. You shall become a party to the Company's standard Indemnification Agreement.

7. **Evidence of Employment Eligibility.** For purposes of federal immigration law, you will be required to provide to the Company documentary evidence of your identity and eligibility for employment in the United States. Such documentation must be provided to us within three (3) business days of your Start Date, or our employment relationship with you may be terminated.

8. Company Handbook. As a Company employee, you will be expected to abide by the Company's rules of operation and standards of conduct. Specifically, you will be required to sign an acknowledgment that you have read and that you understand such rules and standards, which are set forth in the Company Handbook.

9. **Termination of Employment and Severance Benefits.**

(a) ***Preconditions*.** Any other provision of this Agreement notwithstanding, the remaining Subsections of this Section 9 shall not apply unless each of the following requirements is satisfied:

(i) You have executed a general release of all known and unknown claims that you may then have against the Company or persons affiliated with the Company in a form prescribed by the Company, without alterations. You shall execute and return the release on or before the date specified by the Company in the prescribed form (the "Release Deadline"). The Release Deadline shall in no event be later than sixty (60) days after your termination of employment. If the 60 day period described in the prior sentence spans two calendar years, then the payments will begin on the first payroll period, following expiration of the revocation period, in the second calendar year. If you fail to return the release on or before the Release Deadline, or if you revoke the release, then you shall not be entitled to the benefits described in this Section 9; and

(ii) You have returned all property of the Company in your possession.

(b) ***Termination of Employment*.** Except for the severance benefits provided below, the Company's obligations under this Agreement may be terminated upon the occurrence of any of the following events:

(i) The Company's determination in good faith that it is terminating you for Cause ("Termination for Cause");

(ii) The Company's determination that it is terminating you without Cause, which determination may be made by the Company at any time at the Company's sole discretion, for any or no reason ("Termination Without Cause");

(iii) Thirty (30) days following delivery by you of a written notice to the Company stating that you are electing to terminate your employment with the Company ("Voluntary Termination");

(iv) Following your death or Disability (as defined below); or

(v) Your determination in good faith that you are electing to terminate your employment with the Company for Good Reason.

(c) ***Severance Benefits.*** You shall be entitled to receive severance benefits upon termination of employment only as set forth in this Section 9(c):

(i) ***Voluntary Termination.*** In the event of a Voluntary Termination you shall not be entitled to receive payment of any severance benefits. You will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Voluntary Termination and your benefits will be continued under the Company's then existing benefit plans and policies to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of your Voluntary Termination and in accordance with applicable law.

(ii) ***Involuntary Termination/No Change in Control.*** If your employment is terminated under Section 9(b)(ii) or (v) above (such termination, an "Involuntary Termination"), you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Severance Benefits"). The Severance Benefits shall consist of salary continuation for nine (9) months of monthly Base Salary amounts; provided that if you become employed during this period, then the Company's obligation to pay Severance Benefits shall cease upon commencement of your new employment. If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is nine (9) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the

Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings.

(iii) ***Involuntary Termination/ Change in Control*.** If your employment is terminated in an Involuntary Termination immediately prior to or in the eighteen (18) months following a Change in Control, you, or your estate or representative, if applicable, will be entitled to receive payment of severance benefits on the date of your Involuntary Termination (the "Change in Control Severance Benefits"). The Change in Control Severance Benefits shall consist of salary continuation for twelve (12) months' of monthly Base Salary plus a monthly amount equal to your Annual Target Bonus divided by twelve (12). If you elect to continue your health insurance coverage under the Consolidated Omnibus Budget Reconciliation Act ("COBRA") following the Separation, then the Company shall pay your monthly premium under COBRA until the earliest of (A) the date that is twelve (12) months following your Involuntary Termination (the "Continuation Period"), (B) the expiration of your continuation coverage under COBRA and (C) the date when you are offered substantially equivalent health insurance coverage in connection with new employment or self-employment. Notwithstanding anything to the contrary above, if deemed necessary or advisable by the Company in its sole discretion to avoid adverse tax consequences to the Company or any employee thereof, such COBRA premium payments will be treated as taxable compensation income to you, subject to all applicable withholdings. If the Company decides to treat the COBRA premium payments as taxable income compensation to you, the Company will gross-up the amount of the payments to cover all applicable withholdings. If immediately prior to or following a Change in Control (as defined in the Company's 2015 Equity Incentive Plan), your employment with the Company (or the Company's successor) is terminated in an Involuntary Termination during the remaining vesting period of the options then outstanding as of the date of closing of the Change in Control (the "Options"), then one hundred percent (100%) of the unvested shares subject to the Options shall automatically vest.

(iv) ***Termination for Cause*.** In the event of your Termination for Cause, you will receive payment(s) for all salary and unpaid vacation accrued as of the date of your Termination for Cause.

(v) ***Termination by Reason of Death or Disability.*** In the event that your employment with the Company terminates as a result of your death or Disability (as defined below), you or your estate or representative will receive all salary and unpaid vacation accrued as of the date of your death or Disability, all severance benefits payable under Section 9(b)(ii) above and any other benefits payable under the Company's then existing benefit plans and policies, to the extent permitted under such plans and policies and in accordance with such plans and policies in effect on the date of death or Disability and in accordance with applicable law. For purposes of this Agreement, "Disability" shall mean that you have been unable to perform your duties hereunder as the result of physical or mental incapacity lasting at least forty-five (45) consecutive calendar days or ninety (90) calendar days during any consecutive twelve-month period, after which time such incapacity is determined to be permanent by a physician chosen by the Company and its insurers and acceptable to you or to your legal representative (with such agreement on acceptability not to be unreasonably withheld).

10. **Tax Matters.**

(a) ***Withholding.*** All forms of compensation referred to in this Agreement are subject to reduction to reflect applicable withholding and payroll taxes and other deductions required by law.

(b) ***Tax Advice.*** You are encouraged to obtain your own tax advice regarding your compensation from the Company. You agree that the Company does not have a duty to design its compensation policies in a manner that minimizes your tax liabilities, and you will not make any claim against the Company or the Board related to tax liabilities arising from your compensation.

(c) ***280G.*** Notwithstanding anything contained in this Agreement to the contrary, if any of the payments or benefits received or to be received by you pursuant to this Agreement when taken together with payments and benefits provided to you under any other plans, contracts, or arrangements with the Company (all such payments and benefits, the "Total Payments"), would be subject to any excise tax imposed under Code Section 4999 (together with any interest or penalties, the "Excise Tax"), then such Total Payments will be reduced to the extent necessary so that no portion thereof will be subject to the Excise Tax; provided, however, that if you would receive in the aggregate greater value (as determined under Code Section 280G and the regulations thereunder) on an after tax basis if the Total Payments were not subject to such reduction, then no such reduction will be made. To effect the

reduction described herein, if applicable, the Company will first reduce or eliminate the payments and benefits provided under this Agreement. All calculations required to be made under this Section will be made by the Company's independent public accountants, subject to the right of your representative to review the same.

11. **Miscellaneous Provisions.**

(a) ***Choice of Law*.** The validity, interpretation, construction and performance of this Agreement shall be governed by the laws of the State of Maryland, without giving effect to the principles of conflicts of law.

(b) ***Counterparts*.** This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together will constitute one and the same instrument.

(c) ***Severability*.** In the event that any provision hereof becomes or is declared by a court of competent jurisdiction to be illegal, unenforceable, or void, this Agreement shall continue in full force and effect without such provision.

(d) ***Acknowledgment*.** You acknowledge that you have had the opportunity to discuss this matter with and obtain advice from your private attorney, have had sufficient time to read, and have carefully read and fully understand, all the provisions of this Agreement, and are knowingly and voluntarily entering into this Agreement.

(e) **Arbitration.** Any controversy or claim arising out of this Agreement and any and all claims relating to the Employee's Employment with the Company shall be settled by final and binding arbitration. The arbitration shall take place in Washington, D.C., or, at the Employee's option, the County in which the Employee primarily worked when the arbitrable dispute or claim first arose. The arbitration shall be administered by the American Arbitration Association under its National Rules for the Resolution of Employment Disputes. Any award or finding shall be confidential. The Employee and the Company agree to provide one another with reasonable access to documents and witnesses in connection with the resolution of the dispute. The Employee and the Company shall share the costs of arbitration equally. Each party shall be responsible for its own attorneys' fees, and the arbitrator may not award attorneys' fees unless a statute or contract at issue specifically authorizes such an award. This Section 11(e) shall not apply to claims for workers' compensation benefits or unemployment insurance benefits. This Section 11(e) also shall not apply to claims concerning the ownership, validity,

infringement, misappropriation, disclosure, misuse or enforceability of any confidential information, patent right, copyright, mask work, trademark or any other trade secret or intellectual property held or sought by either the Employee or the Company (whether or not arising under the Proprietary Information and Inventions Agreement between the Employee and the Company).

[*The remainder of this page intentionally left blank*]

IN WITNESS WHEREOF, each of the parties has executed this Agreement, in the case of the Company by its duly authorized officer, as of the day and year first above written.

REGENXBIO INC.

By: /s/ Kenneth T. Mills

Name: Kenneth T. Mills

Title: President and CEO

EMPLOYEE

By: /s/ Vittal Vasista

Date: June 30, 2015

REGENXBIO INC.
COMPENSATION PROGRAM FOR NON-EMPLOYEE DIRECTORS

ADOPTED , 2015

A. Cash Compensation

1. Board retainer: $35,000 per year, paid in quarterly installments in arrears.
2. Chairman of the Board retainer: $30,000 per year, paid in quarterly installments.
3. Committee chair retainer: $15,000 per year for the Audit Committee chair, $10,000 per year for the Compensation Committee chair, and $8,000 per year for the Corporate Governance and Nominating Committee chair, paid in quarterly installments in arrears.
4. Committee member retainer: $7,500 per year for members of the Audit Committee, $5,000 per year for the members of the Compensation Committee, and $4,000 per year for the Governance and Nominating Committee, paid in quarterly installments in arrears.

B. Equity Compensation

1. Initial Stock Option grant: stock option to purchase 25,000 shares of the Company's common stock. The option shall vest in equal monthly installments over the 36 months following the grant date, with immediate full vesting in the event of a change in control. The options will be granted by the Compensation Committee under the 2015 Equity Incentive Plan (the "EIP") in conjunction with the director's initial appointment or election to the Board. Upon consummation of the initial public offering of the Company's common stock (the "IPO"), Messrs. Engleman and Fox and Ms. Samuels will be granted their respective Initial Stock Option.
2. Annual Stock Option grant: stock option to purchase 12,500 shares of the Company's common stock. The option shall vest in equal monthly installments over the 12 months following the grant date, with immediate full vesting in the event of a change in control. The stock option will be granted by the Compensation Committee under the EIP in conjunction with the Annual Meeting of the Company's stockholders. Upon consummation of the IPO, Messrs. Beshar, Hayden and Karabelas will be granted their respective Annual Stock Option in a pro-rated amount to reflect their service from the month of the IPO through the date of the Company's next Annual Meeting of the Company's stockholders.

CONFIDENTIAL TREATMENT REQUESTED

UNIVERSITY *of* PENNSYLVANIA

License Agreement

This License Agreement (this "*Agreement*") is between The Trustees of the University of Pennsylvania, a Pennsylvania nonprofit corporation ("*Penn*"), and ReGenX, LLC, a Delaware limited liability company ("*Company*"). This Agreement is being signed on February 20, 2009 (the "*Execution Date*"). This Agreement will be effective on February 24, 2009 (the "*Effective Date*").

BACKGROUND

Penn owns certain intellectual property developed by Dr. James M. Wilson, M.D., Ph.D. of Penn's School of Medicine ("*Dr. Wilson*") relating to a gene therapy technology platform based on certain novel adeno associated viruses discovered by Dr. Wilson at Penn. Penn also owns certain letters patent and/or applications, including provisional patent applications, for letters patent relating to the intellectual property. The Company desires to obtain an exclusive license under the patent rights and related know how to exploit the intellectual property relating to the gene therapy technology platform. Company also desires to fund further research by Dr. Wilson under a separate sponsored research agreement and to obtain an exclusive option under such sponsored research agreement to negotiate for an exclusive license in any intellectual property created, conceived or reduced to practice pursuant to such SRA. Penn has determined that the exploitation of the intellectual property by Company is in the best interest of Penn and is consistent with its educational and research missions and goals.

In consideration of the mutual obligations contained in this Agreement, and intending to be legally bound, the parties agree as follows:

1. LICENSE

1.1 License Grants.

(a) License to Patent Rights. Subject to the limitations set forth in Section 1.1(b) Penn hereby grants to Company an exclusive, worldwide license under the Patent Rights to make, have made, use, import, offer for sale and sell Licensed Products in the Field of Use during the Term (as such terms may be defined in Section 1.2)(the "*Patent License*"). The Patent License includes the right to sublicense as permitted by this Agreement.

(b) Limitations With Respect to Certain Patent Rights.

(i) The parties acknowledge that with respect to the patents and patent applications listed on Exhibit B-1 only (the "**** *Licensed Patents*"), Penn has already granted a nonexclusive license to **** pursuant to the agreement identified in Exhibit B-1 (the "**** *License*"). Accordingly, the rights granted to Penn herein with respect to the **** Licensed Patents only shall be nonexclusive for so long as such preexisting license grant remains in effect. Penn shall have no right to grant or authorize any third party to grant any further rights or licenses in the Field of Use with respect to the **** Licensed Patents during the Term and the rights granted to Company with respect to the **** Licensed Patents shall

automatically become exclusive upon the expiration or termination of the existing license under the **** License without further action by either party. Penn will promptly notify Company of any change with respect to the rights licensed pursuant to the **** License.

(ii) The parties acknowledge that with respect to the patents and patent applications listed on Exhibit B-2 only (the "*GSK Licensed Patents*"), Penn has already granted a license to SmithKline Beecham Corporation dba GlaxoSmithKline ("*GSK*") pursuant to the agreement identified in Exhibit B-2 (the "*GSK License*"). The parties agree that for long as GSK maintains a license to the GSK Licensed Patents in the Field of Use, the rights granted herein with respect to the GSK Licensed Patents only will be subject to the rights granted to GSK prior to the Effective Date. The parties acknowledge that Company is seeking a sublicense under the GSK Licensed Patents directly from GSK and understand that Penn may receive royalties ("*Company Sublicense Royalty Revenues*") or other payments ("*Company Sublicense Non-Royalty Revenues*") under the GSK License as a result of sublicenses granted to the Company. The parties agree that any Company Sublicense Royalty Revenues shall be offset against any amounts due to Penn hereunder. ****. Penn shall have no right to grant or authorize any third party to grant any further rights or licenses in the Field of Use with respect to the GSK Licensed Patents during the Term and to the extent any rights with respect to the GSK Licensed Patents in the Field of Use revert to Penn whether through expiration or termination of the GSK License, by contract or otherwise, such rights shall be automatically included within the scope of the license granted pursuant to Section 1.1(a) without further action by either party. Penn will promptly notify Company of any change with respect to the rights licensed pursuant to the GSK License.

(c) License to Background Know-How. Penn hereby grants to Company a non-exclusive, worldwide, license (the "*Background Know-How License*") under the Background Know-How to make, have made, use, import, offer for sale and sell and otherwise exploit Licensed Products in the Field of Use and to practice the Licensed Processes in connection with the exercise of the foregoing rights in 1.1(a) and 1.1(b) (as such terms may be defined in Section 1.2). The Background Know-How License includes the right to sublicense as permitted by this Agreement. ****.

(d) No Other Licenses. Except as expressly provided in this Section 1.1, no other rights or licenses are granted by Penn. Any intellectual property created or conceived during the performance of the Sponsored Research Agreement between Penn and Company being entered into simultaneously with this Agreement (the "*SRA*") will be governed by the terms of the SRA.

1.2 Related Definitions.

"*Affiliate*" means a legal entity that is controlling, controlled by or under common control with Company and that has executed either this Agreement or a written Joinder Agreement agreeing to be bound by all of the terms and conditions of this Agreement. For purposes of this

Section 1.2, the word "*control*" means (x) the direct or indirect ownership of more than fifty percent (50%) of the outstanding voting securities of a legal entity, (y) the right to receive fifty percent (50%) or more of the profits or earnings of a legal entity, or (z) the right to determine the policy decisions of a legal entity.

"*Background Know-How*" means all Know-How that (a) was developed by Dr. Wilson , or other Penn researchers working under his direct supervision, at Penn, and (b) is related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn prior to the date hereof, and (c) is owned by Penn, (d) is necessary or useful for the practice of the Patent Rights in connection with the manufacture, use, sale, importation and/or other exploitation of the Licensed Products or the practice of the Licensed Processes in the Territory in the Field of Use, including, without, limitation, any Know-How necessary for the Company to the manufacture or have manufactured the materials produced by the Penn Vector Core or Dr. Wilson's lab at Penn.

"*Field of Use*" means any and all fields of use.

"*Know-How*" means any and all information, discoveries, software, methods, works of authorship, techniques, formulae, data, biological materials, processes, unpatentable inventions and other know-how, not including the Patent Rights, developed prior to the Effective Date.

"*Licensed IP*" means the Patent Rights and Background Know-How.

"*Licensed Process*" means any process or machine covered by the Licensed IP or any claim thereof, whether or not the claim is issued or pending.

"*Licensed Products*" means any products that are made, made for, used, imported, offered for sale or sold by or for Company or its Affiliates or sublicensees and that either (i) in the absence of this Agreement, would infringe or misappropriate the Licensed IP, or any claim thereof whether or not the claim is issued or pending, or (ii) use, or are manufactured using, a Licensed Process. Licensed Products include Licensed Pharmaceutical Products and Licensed Reagents.

"*Licenses*" means the Patent License and the Background Know-How License.

"*Patent Rights*" means (i) all of Penn's patent rights represented by or issuing from the United States patents and patent applications (including provisional patent applications) listed in Exhibit A, as well as any continuations, continuations-in-part (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A), divisionals, reexaminations, renewals, re-issues, substitutions, extensions and foreign counterparts of any of the foregoing, and all other patents and patent applications that claim priority from or have common priority with any of the foregoing patents and patent applications, (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A) and including any patents issuing from any of the foregoing; and (ii) all patentable inventions (to the extent they are or become available for license) that (a) were discovered by Dr. Wilson, or other Penn researchers working under his direct supervision, at Penn prior to the Effective Date hereof, and (b) are related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn prior to the date hereof, and (c) are owned by Penn.

"*SRA*" means the Sponsored Research Agreement between the Company and Penn entered into simultaneously herewith, or thereafter.

"*Territory*" means worldwide.

1.3 Reservation of Rights by Penn. Penn reserves the fully-paid, royalty free right to use, and to permit other non-commercial entities to use, the Patent Rights, but not to authorize any commercial third party to use, the Patent Rights solely for educational and research purposes.

1.4 U.S. Government Rights. The parties acknowledge that the United States government retains rights in intellectual property funded under any grant or similar contract with a Federal agency. The License is expressly subject to all applicable United States government rights, including, but not limited to, any applicable requirement that products, which result from such intellectual property and are sold in the United States, must be substantially manufactured in the United States.

1.5 Sublicense Conditions. The Company's right to sublicense granted by Penn under the License is subject to each of the following conditions:

(a) Within **** after Company enters into a sublicense agreement, Company will deliver to Penn a complete and accurate copy of the entire executed sublicense agreement written in the English language.

(b) In each sublicense agreement, Company will require the sublicensee, and any further sublicensees, to comply with the terms and conditions of this Agreement.

(c) Company's execution of a sublicense agreement will not relieve Company of any of its obligations under this Agreement. ****.

2. DILIGENCE

2.1 Development Plan. Company will deliver to Penn, on or before the first anniversary of the Effective Date, a copy of the Company's development plan for the Patent Rights (the "*Development Plan*"). The purpose of the Development Plan is (a) to present the Company's strategy to bring the Patent Rights to commercialization, (b) to project the timeline for completing the necessary tasks to accomplish the goals of the strategy. Company will provide Penn with a written update to the Development Plan at least once every two years after the Effective Date.

2.2 Company's Efforts. Company will use commercially reasonable efforts to develop, commercialize, market and sell a Licensed Product in any part of the Territory. Commercially reasonable efforts shall mean efforts consistent with those utilized by ****

****. The Company will achieve each of the diligence events by the applicable completion date listed in the table below for the first Licensed Product:

DILIGENCE EVENT	COMPLETION DATE
****	****
****	****
****	****

****.

2.3 Satisfaction of Diligence. Upon the earlier of the satisfaction of each of the Diligence events specified or upon first Sale of a US government drug regulatory agency (or foreign equivalent) approved Licensed Product in the Territory, Company shall be deemed to have fully satisfied all of its obligations under this Section 2.

3. FEES AND ROYALTIES

3.1 Equity Issuance. In partial consideration for the Licenses, Company will issue to Penn on the Effective Date such number of shares of Common Stock of the Company as will cause Penn to own at least **** of the capital stock of Company (or ownership units of an LLC, as appropriate) on a fully diluted basis on the Effective Date, assuming the exercise, conversion and exchange of all outstanding securities of Company for or into shares of Common Stock (or ownership units, as appropriate). The issuance of equity or ownership units to Penn will be pursuant to a Stock Purchase Agreement and a Stockholders Agreement, or their LLC equivalents, between Company and Penn), the forms of which are attached as Exhibits C and D (the "*Equity Documents*").

3.2 Earned Royalties. In partial consideration of the Licenses, on the terms and subject to the conditions set forth herein, during the Royalty Term, Company will pay to Penn the following royalty as set forth below:

(a) on Net Sales of Licensed Pharmaceutical Products sold by Company or its Affiliates:

Licensed Pharmaceutical Products	RoyaltyPercentage	ReGenX Annual Net Sales, Cumulative (Million)
1. Using Novel AAV	****	Up to $300;

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

	****	Greater than or equal to $300 and up to $600
	****	Greater than or equal to $600
2. Using Refinement or Modification to existing AAV	****	Up to $300;
	****	Greater than or equal to $300 and up to $600
	****	Greater than or equal to $600

(b) on Net Sales of Licensed Reagents sold by Company or its Affiliates or sublicensees:

Licensed Reagents	Royalty Percentage	ReGenX Annual Net Sales, Cumulative (Million)
1. Using Novel AAV	****	Up to $10;
	****	Greater than or equal to $10 and up to $20
	****	Greater than or equal to $20
2. Using Refinement or Modification to existing AAV	****	Up to $10;
	****	Greater than or equal to $10 and up to $20
	****	Greater than or equal to $20

(c) on royalties received by Company from third parties on Net Sales of Licensed Pharmaceutical Products by such third parties:

Licensed Pharmaceutical Products	Royalty Percentage	Third Party Annual Net Sales, Cumulative (Million)
1. Using Novel AAV	****	Up to $300;
	****	Greater than or equal to $300 and up to $600
	****	Greater than or equal to $600

2.	Using Refinement or Modification to existing AAV	****	Up to $300;
		****	Greater than or equal to $300 and up to $600
		****	Greater than or equal to $600

To meet the requirements of the term "Novel AAV" (as used in Category 1), there must be neither any dominating third party patent nor any Penn-owned patent rights other than those licensed under this Agreement with respect to the vector per se (i.e., no third party patent or Penn-owned patent rights beyond those licensed under this Agreement is required in order to make, have made, use, import, offer for sale or sell the vector for the higher royalty level to apply). Licenses from Penn to ReGenX for genes used, promoters used other than those which are part of the vector as described in Penn Patent Rights and the like in the vector will not affect the royalty pursuant to this provision. If any dominating third party patent or any Penn-owned patent other than those licensed under this Agreement issues at any time during the term of this Agreement with respect to a vector licensed hereunder, then the royalty level will immediately drop to the "Refinement" level (Category 2 above) for any Licensed Product containing such vector.

Notwithstanding the foregoing (i) in no event shall the **** paid to Penn by the Company pursuant to (c) above, **** that would be payable to Penn by the Company on such Net Sales of Licensed Pharmaceutical Products sold by Company and (ii) in no event shall **** be payable in connection with any ****. No royalties other than the payments set forth herein shall be due in connection with the exercise of the rights granted herein. ****.

3.5 Sublicense Fees. In partial consideration of the Licenses, and subject to the terms and conditions set forth herein, Company will pay to Penn a sublicense fee equal to the following percentage of the sum of all fees and milestone payments received by Company from sublicensees from the grant of sublicenses (including options to obtain a sublicense) of the Licensed Intellectual Property during the Quarter ("*Sublicensing Revenues*"):

Date of Sublicense Grant	**Sublicensing Fees**
During the period commencing on the Effective Date and ending on the day prior to the fourth anniversary of the Effective Date	****
Any date on or after the fourth anniversary of the Effective Date	****

Sublicensing Revenues shall not include (a) royalties paid to Company by a sublicensee based upon Sales or Net Sales by the sublicensee; (b) equity investments in Company by a sublicensee and any other non-cash consideration; (c) loan proceeds paid to Company by a sublicensee in an arms length, full recourse debt financing to the extent that such loan is not forgiven; (d) sponsored research funding paid to Company by a sublicensee in a bona fide transaction for future research to be performed by Company.

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

****.

3.8 Related Definitions.

"*Fair Market Value*" means the cash consideration that Company or its Affiliates or sublicensees would realize from an unrelated buyer in an arms length sale of an identical item sold in the same quantity and at the time and place of the transaction. The Fair Market Value shall be determined jointly by Penn and Company based on transactions of a similar type and standard industry practice, if any.

"*Licensed Pharmaceutical Products*" means all Licensed Products other than Licensed Reagents, including, without limitation, any Licensed Product that is intended for therapeutic use.

"*Licensed Reagents*" means a Licensed Product that is intended for research uses only, excluding any research uses in humans.

"*Net Sales*" means the total cash consideration received by Company or its Affiliates ****.

"*Qualifying Costs*" means: (a) ****.

"*Quarter*" means each three-month period beginning on January 1, April 1, July 1 and October 1.

"*Royalty Term*" means, ****.

"*Sale*" means any bona fide transaction for which consideration is received by Company or its Affiliate or sublicensee for the sale, use, lease, transfer or other disposition of a Licensed

Product to an unaffiliated third party. A Sale is deemed completed at the time that Company or its Affiliate or sublicensee receives payment for the Licensed Product.

3.9 Payment Reductions. In the event that during the Royalty Period, litigation between Company and a third party commences involving the Licensed IP, and the third party has launched a competitive product, until such litigation is finally settled or adjudicated pursuant to a nonappealable final order by a court of competent jurisdiction, or a final and binding order issued pursuant to an alternative dispute resolution procedure, ****. If following such verdict, Company is permitted to continue selling Licensed Products or the competitive product is prevented from entering or further sale in the applicable country in the territory by one or more valid claims of the Licensed Patent Rights, ****. If following such verdict, Company is prohibited from continuing to sell Licensed Products, ****.

4. REPORTS AND PAYMENTS

4.1 Royalty Reports. Within **** after the end of each Quarter following the first Sale, Company will deliver to Penn a report, certified by the chief financial officer of Company, detailing the calculation of all royalties, fees and other payments due to Penn for such Quarter. The report will include the following information for the Quarter, each listed by product, by country: (a) the number of units of Licensed Products constituting Sales; (b) the gross consideration received for Sales; (c) Qualifying Costs, listed by category of cost; (d) Net Sales; (e) the gross amount of any qualifying payments and other consideration received by Company from sublicensees; (f) amounts of any deductions permitted by Section 3.9; (g) the royalties, fees and other payments owed to Penn, listed by category; and (h) the computations for any applicable currency conversions. Each royalty report will be substantially in the form of the sample report attached as Exhibit E.

4.2 Payments. Company will pay all royalties, fees and other payments due to Penn under Sections 3.2, 3.3, 3.4, 3.5, 3.6 and 3.7 within **** after the end of the Quarter in which the royalties, fees or other payments accrued.

4.3 Records. Company will maintain, and will cause its Affiliates and sublicensees to maintain, complete and accurate books, records and related background information to verify Sales, Net Sales, and all of the royalties, fees, and other payments due or paid under this Agreement, as well as the various computations reported under Section 4.1. The records for each Quarter will be maintained for at least **** after submission of the applicable report required under Section 4.1.

4.4 Audit Rights. Upon reasonable prior written notice to Company, Company and its Affiliates and sublicensees will provide Penn and its accountants with access to all of the books, records and related background information required by Section 4.3 to conduct a review or audit of Sales, Net Sales, and all of the royalties, fees, and other payments payable or paid under this Agreement. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate Penn's review or audit without unreasonable disruption to Company's business; and (c) no more than once each calendar year during the Term (as defined below) and for a period of **** thereafter. Company will promptly pay to Penn

the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Company has underpaid any payment by **** or more, then Company will also promptly pay the costs and expenses of Penn and its accountants in connection with the review or audit.

4.5 Currency. All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments will be made in United States dollars. If Company receives payment from a third party in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal as of the last business day of the Quarter in which the payment was received by Company, and (b) the conversion computation will be documented by Company in the applicable report delivered to Penn under Section 4.1.

4.6 Place of Payment. All payments by Company are payable to "The Trustees of the University of Pennsylvania" and will be made to the following addresses:

By Electronic Transfer:
Wachovia Bank, N.A.
ABA #****
Account Number: ****
Center for Technology Transfer
Attention: ****

By Check:
The Trustees of the University of Pennsylvania
c/o Center for Technology Transfer
PO Box 785546
Philadelphia, PA 19178-5546

4.7 Interest. All amounts that are not paid by Company when due will accrue interest from the date due until paid at a rate equal to one and one-half percent (1.5%) per month (or the maximum allowed by law, if less).

5. CONFIDENTIALITY AND USE OF PENN'S NAME

5.1 Confidentiality Agreement. If Company and Penn entered into one or more Confidential Disclosure Agreements prior to the Effective Date, then such agreements will continue to govern the protection of confidential information under this Agreement, and each Affiliate and sublicensee of Company will be bound to Company's obligations under such agreements. If, however, no Confidential Disclosure Agreement has been entered into between Company and Penn prior to the Effective Date, then in connection with the execution of this Agreement, the parties will enter into a Confidential Disclosure Agreement substantially similar to Penn's standard form. The term "*Confidentiality Agreement*" means all Confidential Disclosure Agreements between the parties that remain in effect after the Effective Date.

5.2 Other Confidential Matters. Penn is not obligated to accept any confidential information from Company, except for the reports required by Sections 2.1, 4.1, 4.4 and 6.6. Penn, acting through its Center for Technology Transfer and finance offices, will use reasonable efforts not to disclose to any third party outside of Penn any confidential information of Company contained in those reports, for so long as such information remains confidential. Penn bears no institutional responsibility for maintaining the confidentiality of any other information of Company. Company may elect to enter into confidentiality agreements with individual investigators at Penn that comply with Penn's internal policies.

5.3 Use of Penn's Name. Company and its Affiliates, sublicensees, employees, and agents may not use the name, logo, seal, trademark, or service mark (including any adaptation of them) of Penn or any Penn school, organization, employee, student or representative, without the prior written consent of Penn.

6. TERM AND TERMINATION

6.1 Term. This Agreement will commence on Effective Date and end upon the expiration of the Royalty Term (the "*Term*"). Earned royalties pursuant to Section 3.4 shall only be payable hereunder during the Royalty Term.

6.2 Early Termination by Company. Company may terminate this Agreement at any time effective upon completion of each of the following conditions: (a) providing at least **** prior written notice to Penn of such intention to terminate; (b) ceasing to make, have made, use, import, offer for sale and sell all Licensed Products; (c) terminating all sublicenses and causing all Affiliates and sublicensees to cease making, having made, using, importing, offering for sale and selling all Licensed Products; and (d) paying all amounts owed to Penn under this Agreement and any Sponsored Research Agreement between Penn and Company related to the Patent Rights, through the effective date of termination.

6.3 Early Termination by Penn. Penn may terminate this Agreement if: (a) Company is more than **** late in paying to Penn any amounts owed under this Agreement and does not pay Penn in full within **** after receipt of written notice indicating such default and demanding payment, including accrued interest (a "*Payment Default*"); (b) other than a Payment Default, Company or its Affiliate or sublicensee fails to achieve a diligence event on or before the applicable completion date or otherwise breaches this Agreement and does not cure such failure or breach within **** after written notice of the breach; or (c) Company or its Affiliate or sublicensee experiences a Trigger Event.

6.4 Trigger Event. The term "*Trigger Event*" means any of the following: (a) if Company or its Affiliate or sublicensee (i) becomes insolvent, bankrupt or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver or trustee for it or its property and, if appointed without its consent, not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation or the reorganization, readjustment or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Company or its Affiliate or sublicensee of any proceeding under any law related to bankruptcy, insolvency, liquidation or the reorganization, readjustment or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4(a) or (b) above; (d) the calling by Company or its Affiliate or sublicensee of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; (e) the act or failure to act by Company or its Affiliate or sublicensee indicating its consent to, approval of or acquiescence in any of the proceedings described in Section 6.4(b) — (d) above; or (f) the commencement by Company of any action against Penn, including an action for declaratory judgment, to declare or render invalid or unenforceable the Patent Rights, or any claim thereof.

6.5 Effect of Termination. Upon the valid early termination of this Agreement pursuant to Section 6.2 or 6.3: (a) the Licenses shall terminate; (b) Company and all its Affiliates and sublicensees will cease all making, having made, using, importing, offering for sale and selling all Licensed Products and practicing the Licensed Processes; (c) Company will pay to Penn all amounts, including accrued interest, owed to Penn under this Agreement and any Sponsored Research Agreement related to the Patent Rights, through the date of termination, including royalties on Licensed Products invoiced or shipped through the date of termination when such payments are received, whether or not payment is received prior to termination; (d) each Party will, at the other Party's request, return to such Party all confidential information of such Party; and (e) except as provided in Section 6.6, all rights and duties of Penn and the Company under this Agreement immediately terminate without further action required by either Penn or Company.

6.6 Survival. Company's obligation to pay all amounts, including accrued interest, owed to Penn under this Agreement will survive the termination of this Agreement for any reason. Sections 13.9 and 13.10 and Articles 4, 5, 6, 9, 10, and 11 will survive the termination of this Agreement for any reason in accordance with their respective terms.

7. PATENT PROSECUTION AND MAINTENANCE

7.1 Patent Control. Except as otherwise provided in this Section 7.1, Penn shall control the preparation, prosecution and maintenance of the Patent Rights and the selection of patent counsel, with input from Company. If, however, Company desires a greater degree of control over the Patent Rights, then Company and Penn will use good faith efforts to promptly enter into a Client and Billing Agreement with patent counsel acceptable to the Company in substantially in the form attached as Exhibit F. During the term of the Client and Billing Agreement, Company will control and manage the preparation, prosecution and maintenance of the Patent Rights, with input from Penn. In the absence of or upon termination of a Client and Billing Agreement for any reason, control reverts to Penn with input from Company. For purposes of this Article 7, the word "*maintenance*" includes any interference negotiations, claims, or proceedings, in any forum, brought by Penn, Company, a third party, or the United States Patent and Trademark Office, and any requests by Penn or Company that the United States Patent and Trademark Office reexamine or reissue any patent in the Patent Rights.

7.2 Payment and Reimbursement. Company will reimburse Penn for (i) **** of all attorneys fees, expenses, official fees and all other charges accumulated **** to the Effective Date incident to the preparation, filing, prosecution and maintenance of the ****, and (ii) **** of all attorneys fees, expenses, official fees and all other charges accumulated prior to the Effective Date incident to the preparation, filing, prosecution and maintenance of the Patent Rights other than the ****, in the case of clause (ii) to the extent that such amounts have not already been reimbursed by third parties to Penn, provided that such reimbursement obligation with respect to clauses (i) and (ii) shall not exceed ****. The reimbursement obligation shall be paid in three equal installments, with the first installment payment becoming due thirty (30) days after the Effective Date and the two remaining payments becoming due on the first and second anniversary dates of the Effective Date. Thereafter, Company will either pay directly under a Client and Billing Agreement or reimburse Penn for **** documented attorneys fees, expenses, official fees and all other charges accumulated on or after the Effective Date incident to the preparation, filing, prosecution, and maintenance of the Patent Rights, within **** after Company's receipt of invoices for such fees, expenses and

charges. Except during the term of a Client and Billing Agreement, Penn shall notify the Company promptly, and in advance to the extent reasonably practicable, of any upcoming expenditures in excess of **** in connection with the Patent Rights. Penn reserves the right to require the Company to provide a deposit in advance of incurring out of pocket patent expenses estimated by counsel to exceed ****. If Company fails to reimburse patent expenses under Paragraph 7.2, or provide a requested deposit with respect to a Patent Right, then Penn will be free at its discretion and expense to either abandon such applications or patents related to such Patent Right or to continue such preparation, prosecution and/or maintenance activities, and any patent rights associated with such patent action will be automatically excluded from the term "Patent Rights" hereunder, on a patent by patent or country by country basis, as applicable. Notwithstanding the foregoing, (i) Company shall have no obligation to pay any amounts pursuant to this Section 7.2 with respect to the GSK Licensed Patents; (ii) Company's payment obligations pursuant to this Section 7.2 with respect to the **** shall be limited to **** of the amounts otherwise required to be paid; and (iii) **** payment obligations pursuant to this Section 7.2 shall be reduced**** by any patent expense reimbursement amounts received by Penn from **** of the Patent Rights.

8. INFRINGEMENT

8.1 Notice. Company and Penn will notify each other promptly of any infringement of the Patent Rights that may come to their attention. Company and Penn will consult each other in a timely manner concerning any appropriate response to the infringement.

8.2 Prosecution of Infringement. Company may prosecute any infringement of the Patent Rights at Company's expense, including defending against any counterclaims or cross claims brought by any party against Company or Penn regarding the Patent Rights and defending against any claim that the Patent or Patent Rights are invalid in the course of any infringement action or in a declaratory judgment action. Penn reserves the right to intervene voluntarily and join Company in any such infringement litigation. If Penn chooses not to intervene voluntarily, but Penn is a necessary party to the action brought by Company, then Company may join Penn in the infringement litigation provided that Penn shall have the right to retain its own counsel, reasonably acceptable to Company, and Company will be responsible for **** of Penn's reasonable litigation expenditures including any attorney's fees, expenses, official fees and other charges incurred by Penn, even if there are no financial recoveries from the infringement action. Company will reimburse Penn within **** after receiving each invoice from Penn. If Company decides not to prosecute any infringement of the Patent Rights, then Penn may elect to prosecute such infringement independently of Company in Penn's sole discretion, and at Penn's expense.

8.3 Cooperation. In any litigation under this Article 8, either party, at the request and sole expense of the other party, will cooperate to the fullest extent reasonably possible. This Section 8.3 will not be construed to require either party to undertake any activities, including legal discovery, at the request of any third party, except as may be required by lawful process of a court of competent jurisdiction. If, however, either party is required to undertake any activity, including legal discovery, as a right of lawful process of a court of competent jurisdiction, then Company will pay all expenses incurred by Company and by Penn.

8.4 Control of Litigation. Company controls any litigation or potential litigation involving the prosecution of infringement claims regarding the Patent Rights, including the selection of counsel, all with input from Penn. Notwithstanding the foregoing, Penn shall have the right to approve all decisions that would have a materially adverse affect on the validity, scope of patent claims, or enforceability of the Patent Rights. Company must not settle or compromise any such litigation in a manner that imposes any obligations or restrictions on Penn or grants any rights to the Patent Rights, other than any permitted sublicenses, without Penn's prior written permission. In all instances in which Penn is a voluntary party, Penn reserves the right to select its own counsel, at its own expense. Penn shall have the right to control all litigation regarding the Patent Rights which is prosecuted by Penn independent of Company.

8.5 Recoveries from Litigation. Except as expressly provided in this Section 8.5, if Company prosecutes any infringement claims, Company will use the financial recoveries from such claims, if any, (a) first, to reimburse **** for its litigation expenditures; and (b) second, to retain any remainder but to treat the remainder as **** for the purpose of determining ****. If Company prosecutes any infringement claims with Penn joined as a voluntary party, then Company will use the financial recoveries from such claims, if any, (a) first, to reimburse **** and the **** for their respective litigation expenditures on a dollar-for-dollar basis; and (b) second, to retain any remainder but to ****. If Penn prosecutes any infringement claims independent of ****, then Penn will prosecute such infringement at **** expense and will retain any financial recoveries ****.

9. REPRESENTATIONS AND WARRANTIES; DISCLAIMER OF WARRANTIES

9.1 Mutual Representations and Warranties. Each party represents and warrants to the other party that Party that: (a) this Agreement is and shall be a legal and valid obligation binding upon such Party and enforceable in accordance with its terms; and (b) the execution, delivery and performance of this Agreement by such Party have been duly authorized by all necessary corporate and institutional action and do not and will not: (i) require any consent or approval of its stockholders or Trustees; or (ii) to such Party's knowledge, violate any law, rule, regulation, order, writ, judgment, decree, determination or award of any court, governmental body or administrative or other agency having jurisdiction over such Party.

9.2 Representations and Warranties of Penn. Penn represents and warrants to Company that to the knowledge of the current staff of Penn's Center for Technology ("*CTT*"):

(a) Penn has no commercial license agreements in effect as of the Effective Date, with third parties under the Patent Rights, other than to the US government;

(b) CTT has obtained from Dr. Wilson and the employees he has designated as being involved in the development of the Patent Rights an assignment of rights necessary to permit Penn to grant the Company the Licenses and make the representation set forth in Section 9.2(a) above;

(d) (i) there are no actual, pending actions, suits, claims, interferences, oppositions or governmental investigations involving Patent Rights; (ii) the Patent Rights are not subject

anywhere in the Territory to any pending re-examination, protest, opposition, interference or litigation proceeding.

9.3 Disclaimer. EXCEPT AS EXPRESSLY PROVIDED HEREIN, THE PENN PATENT RIGHTS, LICENSED PRODUCTS AND ANY OTHER TECHNOLOGY LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS. PENN MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, INCLUDING BUT NOT LIMITED TO ANY WARRANTY OF ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, PROFITABILITY, COMMERCIAL UTILITY, NON-INFRINGEMENT OR TITLE.

10. LIMITATION OF LIABILITY

10.1 Limitation of Liability. PENN WILL NOT BE LIABLE TO COMPANY, ITS AFFILIATES, SUBLICENSEES, SUCCESSORS OR ASSIGNS, OR ANY THIRD PARTY WITH RESPECT TO ANY CLAIM: ARISING FROM COMPANY'S USE OF THE PENN PATENT RIGHTS, LICENSED PRODUCTS OR ANY OTHER TECHNOLOGY LICENSED UNDER THIS AGREEMENT; OR ARISING FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE OR SALE OF LICENSED PRODUCTS. PENN WILL NOT BE LIABLE TO COMPANY, ITS AFFILIATES, SUBLICENSEES, SUCCESSORS OR ASSIGNS, OR ANY THIRD PARTY FOR LOST PROFITS, BUSINESS INTERRUPTION, OR INDIRECT, SPECIAL, INCIDENTAL, OR CONSEQUENTIAL DAMAGES OF ANY KIND.

11. INDEMNIFICATION

11.1 Indemnification. Company will defend, indemnify, and hold harmless each Indemnified Party from and against any and all Liabilities with respect to an Indemnification Event. The term "*Indemnified Party*" means each of Penn and its trustees, officers, faculty, students, employees, contractors, and agents. The term "*Liabilities*" means all damages, awards, deficiencies, settlement amounts, defaults, assessments, fines, dues, penalties, costs, fees, liabilities, obligations, taxes, liens, losses, lost profits and expenses (including, but not limited to, court costs, interest and reasonable fees of attorneys, accountants and other experts) that are incurred by an Indemnified Party or awarded or otherwise required to be paid to third parties by an Indemnified Party. The term "*Indemnification Event*" means any Claim against one or more Indemnified Parties to the extent arising out of or resulting from: ****. The term "*Claim*" means any charges, complaints, actions, suits, proceedings, hearings, investigations, claims or demands.

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

11.2 Reimbursement of Costs. Company will pay directly all Liabilities incurred for defense or negotiation of any Claim or will reimburse Penn for all documented Liabilities incident to the defense or negotiation of any Claim within **** after Company's receipt of invoices for such fees, expenses and charges.

11.3 Control of Litigation. Company controls any litigation or potential litigation involving the defense of any Claim, including the selection of counsel, with input from Penn.

11.4 Other Provisions. Company will not settle or compromise any Claim giving rise to Liabilities in any manner that imposes any restrictions or obligations on Penn or grants any rights to the Licensed IP or the Licensed Products without Penn's prior written consent. If Company fails or declines to assume the defense of any Claim within thirty (30) days after notice of the Claim, or fails to reimburse an Indemnified Party for any Liabilities pursuant to Sections 11.1 and 11.2 within the thirty (30) day time period set forth in Section 11.2, then Penn may assume the defense of such Claim for the account and at the risk of Company, and any Liabilities related to such Claim will be conclusively deemed a liability of Company. The indemnification rights of the Indemnified Parties under this Article 11 are in addition to all other rights that an Indemnified Party may have at law, in equity or otherwise.

12. INSURANCE

12.1 Coverages. Company will procure and maintain insurance policies for the following coverages with respect to personal injury, bodily injury and property damage arising out of Company's performance under this Agreement: (a) during the Term, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence and in the aggregate; (b) prior to the commencement of clinical trials involving Licensed Products, clinical trials coverage in a minimum amount of **** combined single limit per occurrence and in the aggregate; and (c) prior to the Sale of the first Licensed Product, product liability coverage, in a minimum amount of **** combined single limit per occurrence and in the aggregate. Penn may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 12.1, ****. The required minimum amounts of insurance do not constitute a limitation on Company's liability or indemnification obligations to Penn under this Agreement.

12.2 Other Requirements. The policies of insurance required by Section 12.1 will be issued by an insurance carrier with an A.M. Best rating of **** or better and will name Penn as an additional insured with respect to Company's performance under this Agreement. Company will provide Penn with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Penn in writing at least **** prior to the cancellation or material change in coverage.

13. ADDITIONAL PROVISIONS

13.1 Independent Contractors. The parties are independent contractors. Nothing contained in this Agreement is intended to create an agency, partnership or joint venture between the parties. At no time will either party make commitments or incur any charges or expenses for or on behalf of the other party.

13.2 No Discrimination. Neither Penn nor Company will discriminate against any employee or applicant for employment because of race, color, sex, sexual or affectional preference, age, religion, national or ethnic origin, handicap, or veteran status.

13.3 Compliance with Laws. Company must comply with all prevailing laws, rules and regulations that apply to its activities or obligations under this Agreement. For example, Company will comply with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the applicable agency of the United States government and/or written assurances by Company that Company will not export data or commodities to certain foreign countries without prior approval of the agency. Penn does not represent that no license is required, or that, if required, the license will issue.

13.4 Modification, Waiver & Remedies. This Agreement may only be modified by a written amendment that is executed by an authorized representative of each party. Any waiver must be express and in writing. No waiver by either party of a breach by the other party will constitute a waiver of any different or succeeding breach. Unless otherwise specified, all remedies are cumulative.

13.5 Assignment & Hypothecation. Neither Party may assign this Agreement or any part of it to any entity, other than an Affiliate, without the prior written consent of the other Party, such consent not to be unreasonably withheld or delayed. Notwithstanding the foregoing, Company shall be permitted to assign this Agreement, without the prior written consent of Penn, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates to a company in the business of developing and commercializing pharmaceutical products that has, together with its affiliates, a market value or, in the case of a publicly traded company listed on a nationally recognized exchange, market capitalization, of at least $250,000,000. As part of any permitted assignment, the assigning party will require any assignee to agree in writing to be legally bound by this Agreement to the same extent as the assigning party. The non-assigning party will not unreasonably withhold or delay its consent, provided that: (a) at least thirty (30) days before the proposed transaction, the assigning party gives the non-assigning party written notice and such background information as may be reasonably necessary to enable the non-assigning party to give an informed consent; (b) the assignee agrees in writing to be legally bound by this Agreement; and (c) the assigning party provides the non-assigning party with a copy of assignee's undertaking. Any permitted assignment will not relieve the assigning party of responsibility for performance of any obligation of the assigning party that has accrued at the time of the assignment. Further, in the event of assignment to an Affiliate, the assigning party will assume responsibility to ensure that Affiliate assignee complies fully with all of its obligations under the Agreement on an ongoing basis. Neither party will grant a security interest in the Licenses or this Agreement during the Term. Any prohibited assignment or security interest will be null and void.

13.6 Notices. Any notice or other required communication (each, a "*Notice*") must be in writing, addressed to the party's respective Notice Address listed on the signature page, and delivered: (a) personally; (b) by certified mail, postage prepaid, return receipt requested; (c) by recognized overnight courier service, charges prepaid; or (d) by facsimile. A Notice will be deemed received: if delivered personally, on the date of delivery; if mailed, five (5) days after deposit in the United States mail; if sent via courier, one (1) business day after deposit with the

courier service; or if sent via facsimile, upon receipt of confirmation of transmission provided that a confirming copy of such Notice is sent by certified mail, postage prepaid, return receipt requested.

13.7 Severability & Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then the remaining provisions of this Agreement will remain in full force and effect. Such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the parties' original intent.

13.8 Headings & Counterparts. The headings of the articles and sections included in this Agreement are inserted for convenience only and are not intended to affect the meaning or interpretation of this Agreement. This Agreement may be executed in several counterparts, all of which taken together will constitute the same instrument.

13.9 Governing Law. This Agreement will be governed in accordance with the laws of the Commonwealth of Pennsylvania, without giving effect to the conflict of law provisions of any jurisdiction.

13.10 Dispute Resolution. If a dispute arises between the parties concerning any right or duty under this Agreement, then the parties will confer, as soon as practicable, in an attempt to resolve the dispute. If the parties are unable to resolve the dispute amicably, then the parties will submit to the exclusive jurisdiction of, and venue in, the state and Federal courts located in the Eastern District of Pennsylvania with respect to all disputes arising under this Agreement.

13.11 Integration. This Agreement with its Exhibits and the Sponsored Research Agreement, the Equity Documents, and the Confidentiality Agreement, contain the entire agreement between the parties with respect to the Patent Rights and the License and supersede all other oral or written representations, statements, or agreements with respect to such subject matter, including but not limited to the Term Sheet.

Each party has caused this Agreement to be executed by its duly authorized representative.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: /s/ Mike Cleare
Name: Mike Cleare
Title: Executive Director
Center for Technology Transfer

Address: Center for Technology Transfer
University of Pennsylvania
3160 Chestnut Street, Suite 200
Philadelphia, PA 19104-6283
Attention: Executive Director

Required copy to: University of Pennsylvania
Office of General Counsel
133 South 36th Street, Suite 300
Philadelphia, PA 19104-3246
Attention: General Counsel

ReGenX, LLC

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: Chief Executive Officer

Address: ReGenX, LLC
750 17th Street, NW
Washington, DC 20006
Attention: Board of Managers

CONFIDENTIAL TREATMENT REQUESTED

EXHIBIT INDEX

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Patents and Patent Applications in Patent Rights

Penn #	Disclosure Title	US Patents	Foreign Patents
****	****	****	****
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CONFIDENTIAL TREATMENT REQUESTED

Exhibit B-1
****** Licensed Patents**

Penn #	Disclosure Title	US Patents	Foreign Patents
****	****	****	****

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CONFIDENTIAL TREATMENT REQUESTED

Exhibit B-2

GSK Licensed Patents

Penn #	Disclosure Title	US Patents	Foreign Patents
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Penn CTT
Center *for* Technology Transfer

Licensee: _____
Inventor(s): _____
Period Covered:
From _____
To _____

Agreement # _____
Patent #(s): _____
Prepared By _____
Date _____
Approved By _____
Date _____

If license covers several major product lines, please prepare a separate report for each line.
Then combine all product lines into a summary report.

Report Type:

- ☐ Single Product Line Report
- ☐ Multiple product Summary Report Page ____ of ____ pages
- ☐ Product Line Detail: Line: _____
  Trade Name _____
  Page _____

Report Currency: ☐ US Dollars ☐ Other (specify) _____

					Period Royalty Amount			
Country	Gross Sales	Allowances	Net Sales	Royalty Rate	This Quarter	This Year to Date	This Quarter - Prior Year	Year to date Prior Year
Total			0					
United States			0	7.5%	0			
Canada			0	7.5%	0			
Total US & Canada	0	0	0		0			
Net Outside US & Canada	0	0	0	15%	0			
Total	0	0	0		0	0	0	0

Conversion rate if other than US Dollars _____

Royalties in US Dollars _____

On a separate page, please indicate the reasons for returns of other adjustments, if >=5% of sales. Also, note any unusual occurrences that affected royalty amounts during the reporting period.

CONFIDENTIAL TREATMENT REQUESTED

[NOTE THAT A PMA CAN BE USED ONLY DURING THE PERIOD WHERE THERE IS ONLY ONE LICENSEE TO THE PATENT RIGHTS IN ANY FIELD]

PATENT MANAGEMENT AGREEMENT

The Trustees of the University of Pennsylvania ("Penn"), a Pennsylvania non-profit corporation doing business at 3160 Chestnut Street, Suite 200, Philadelphia, PA 19104-6283; and _____ ("Company"), a corporation doing business at _____, have entered into a License Agreement with respect to certain inventions which are the subject of the patent applications and patents listed in Appendix A hereto, including any continuations, divisions, extensions thereof, and any foreign counterpart patents, applications, or registrations ("Patent Rights").

Penn has retained the services of _____ ("Law Firm"), with offices at _____, to prepare, file and prosecute the pending patent applications constituting the Patent Rights and to maintain the patents that issue thereon.

Penn, Company and Law Firm, intending to formalize their business relationships, agree as follows:

1. Penn is the owner of the Patent Rights.

2. Company is the licensee of Penn's interest in the Patent Rights.

3. Penn shall maintain an attorney-client relationship with Law Firm in furtherance of efforts to secure and maintain the Patent Rights.

4. Law Firm will interact directly with Company on all patent prosecution and patent maintenance matters related to the Patent Rights and will copy Penn on all correspondence related thereto. Company and Law Firm agree to use all reasonable efforts to notify Penn in writing at least thirty (30) days prior to the due date or deadline for any action which could adversely affect the pending status of any patent application within the Patent Rights, the maintenance of any granted patent within the Patent Rights, Penn's right to file any continuing application or foreign counterpart application based on the Patent Rights, or the breadth of any claim within the Patent Rights. In any case, Company shall give Penn written notice of any final decision regarding the action to be taken or not to be taken on such matters prior to instructing Law Firm to implement the decision. Penn reserves the right to countermand any instruction given by Company to Law Firm.

5. Law Firm's legal services relating to the Patent Rights will be performed on behalf of Penn. Law Firm will invoice Penn for all such services. Company will reimburse Penn for all such services within thirty (30) days of Company's receipt of Penn's invoice for such services.

6. To clarify each party's position with regard to prosecution and maintenance of the Patent Rights, Company will notify Law Firm in writing of all decisions to authorize the performance of any desired service(s), which shall be subject to Penn's right to countermand, as provided in paragraph 4, above. In the event Penn countermands any decision or instruction of Company, such countermand shall be promptly communicated in writing to Law Firm.

7. Penn may terminate this agreement at any time upon notice to Law Firm and Company.

8. This agreement represents the complete understanding of each of the undersigned parties as to the arrangements defined herein. Additions or deletions of dockets identified in Appendix A will become effective only by written addendum to Appendix A. All such additions or deletions of individual patents or applications filed in the US, or as foreign counterparts thereof are considered to be within the terms of this Patent Management Agreement.

9. Notices and copies of all correspondence relating to the Patent Rights should be sent to the following:

To PENN:

Center for Technology Transfer
University of Pennsylvania
3160 Chestnut Street, Suite 200
Philadelphia, PA 19104-6283
Attn: Director, Intellectual Property

To COMPANY:

To Law Firm:

ACCEPTED AND AGREED TO:

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By: _____

Name: _____

Title: _____

Date: _____

COMPANY

By: _____

Name: _____

Title: _____

Date: _____

LAW FIRM

By: _____

Name: _____

Title: _____

Date: _____

CONFIDENTIAL TREATMENT REQUESTED

Appendix A

COMPANY LICENSED TECHNOLOGIES

PENN Docket Number	Title	Patent Numbers

CONFIDENTIAL TREATMENT REQUESTED

Penn
University of Pennsylvania

Center for Technology Transfer

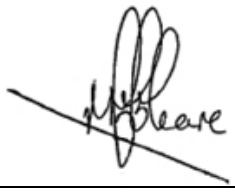
March 6, 2009

Mr. Kenneth T. Mills
Chief Executive Officer
ReGenX, LLC
750 17th Street, NW
Washington, DC 20006

Dear Ken:

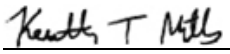
Please find attached an amended Exhibit A "Patents and Patent Applications in Patent Rights" for the License Agreement between the Trustees of the University of Pennsylvania and ReGenX, LLC with an Effective Date of February 24, 2009 (the "Agreement"). The amendment removes Penn docket #K1774 from Exhibit A in its entirety. With the exception of this single change, all other provisions of the Agreement remain in full force and effect.

Sincerely yours,



Name: Michael J. Cleare, PhD
Title: Associate Vice Provost for Research and Executive Director, Center for Technology Transfer

Acknowledged and Agreed:

Signature: 
Name: Kenneth T. Mills
Title: Chief Executive Officer, ReGenX, LLC
Date: 3/9/2009

3160 Chestnut Street, Suite 200 Philadelphia, PA 19104-6283
Tel 215.573.4500 Fax 215.898.9519 www.ctt.upenn.edu

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Patents and Patent Applications in Patent Rights

Penn #	Disclosure Title		US Patents		Foreign Patents
****	****	****		****	
****	****	****		****	
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****	****	****		****	

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CONFIDENTIAL TREATMENT REQUESTED

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CONFIDENTIAL TREATMENT REQUESTED

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CONFIDENTIAL TREATMENT REQUESTED

UNIVERSITY *of* PENNSYLVANIA

Second Amendment to License Agreement

This Second Amendment to License Agreement (this "*Second Amendment*") effective as of September 9, 2014 (this "*Second Amendment Effective Date*"), is made by and between The Trustees of the University of Pennsylvania ("*Penn*") and ReGenX Biosciences, LLC ("*Company*") (collectively, the "*Parties*") and amends the License Agreement between the Parties, which was effective as of February 24, 2009, as subsequently amended by a First Amendment dated March 6, 2009 (the "*License Agreement*"). All capitalized terms used but not defined herein shall have the meaning set forth in the License Agreement.

BACKGROUND

The License Agreement relates to certain intellectual property developed by Dr. James M. Wilson of Penn's Perelman School of Medicine, which intellectual property is the subject of patents or patent applications (the "*Penn Dockets*").

WHEREAS, the Company has elected to exercise certain of its option rights pursuant to the sponsored research agreement by and between the Parties effective as of February 24, 2009 and as subsequently amended with respect to certain Penn patentable inventions and patent rights. Penn and Company have reached agreement on terms to extend such additional Penn patent rights to Company as set forth herein;

WHEREAS, The Parties wish to amend the License Agreement to reflect these changes.

Now, therefore, in consideration of the promises and covenants contained in this Second Amendment and for other good and valuable consideration the receipt and sufficiency of which is hereby acknowledged and intending to be legally bound, the Parties agree as follows:

1) Exhibit A of the License Agreement is hereby amended and restated in its entirety by Exhibit A to this Second Amendment to add the additional Patent Rights and Know-How designated therein as "Second Amendment Patent Rights and Know-How". On the Second Amendment Effective Date, Company will reimburse Penn for all patent prosecution and maintenance expenses associated with the Second Amendment Patent Rights that have not previously been reimbursed by Company.

2) The following definitions in Section 1.2 of the License Agreement shall be amended and restated in their entirety as follows:

> "Background Know-How" means all Know-How that (a) was developed by Dr. Wilson, or other Penn researchers working under his direct supervision, at Penn, and (b) is related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn prior to February 24, 2009, or is related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn after February 24, 2009 pursuant to the SRA, arid (c) is owned by Penn and available for licensing,

and (d) is necessary or useful for the practice of the Patent Rights in connection with the manufacture, use, sale, importation and/or other exploitation of the Licensed Products or the practice of the Licensed Processes in the Territory in the Field of Use, including, without, limitation, any Know-How necessary for the Company to manufacture or have manufactured the materials produced by the Penn Vector Core or Dr. Wilson's lab at Penn.

"Field of Use" means any and all fields of use, except with respect to the Patent Rights listed in Exhibit A, Part 2 and all related Know-How and data, for which the Field of Use is limited to viral vector mediated gene therapy.

"Know-How" means any and all information, discoveries, software, methods, works of authorship, techniques, formulae, data, biological materials, processes, unpatentable inventions and other know-how, not including the Patent Rights, developed prior to the Effective Date or under the SRA.

"Patent Rights" means (i) all of Penn's patent rights represented by or issuing from the United States patents and patent applications (including provisional patent applications) listed in Exhibit A, as well as any continuations, continuations-in-part (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A), divisionals, reexaminations, renewals, re-issues, substitutions, extensions and foreign counterparts of any of the foregoing, and all other patents and patent applications that claim priority from or have common priority with any of the foregoing patents and patent applications, (to the extent the inventions claimed or disclosed in any such patent or patent applications are directed to subject matter specifically described in the patent or patent applications listed in Exhibit A) and including any patents issuing from any of the foregoing; and (ii) all patentable inventions (to the extent they are or become available for license) that (a) were discovered by Dr. Wilson, or other Penn researchers working under his direct supervision, at Penn prior to the Second Amendment Effective Date, and (b) are related to the adeno associated virus technology platform discovered by Dr. Wilson at Penn prior to the Effective Date or under the SRA, and (c) are owned by Penn and available for licensing.

"SRA" means the each of: 1) the Sponsored Research Agreement between the Company and Penn effective as of February 24, 2009, as subsequently amended and 2) the Sponsored Research Agreement between the Company and Penn effective as of November 1, 2013 and any future amendments thereof

3) Section 1.5 (a) of the License Agreement shall be amended and restated in its entirety as follows:

"1.5(a) Within **** after Company enters into a sublicense agreement, Company will deliver to Penn a complete and accurate copy of the entire executed sublicense agreement written in the English language."

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

4) Section 2.3 of the License Agreement shall be amended and restated in its entirety as follows:

"Company is deemed to have fully satisfied all of its obligations under this Section 2."

5) Section 3.2 is hereby amended to add the following language at the end:

"Notwithstanding anything herein to the contrary, in no event shall Penn receive a royalty of less than **** (****) of Net Sales of Licensed Pharmaceutical Products sold by ****, where such Licensed Pharmaceuticals Products are not also covered by GSK Licensed Patents. For clarity, **** include ****."

6) Section 6.1 of the License Agreement shall be amended and restated in its entirety as follows:

"6.1 Term. This Agreement will commence on Effective Date and end upon the expiration of the Royalty Term (the "*Term*"). Earned royalties pursuant to Section 3.4 shall only be payable hereunder during the Royalty Term. Upon expiration of the Agreement, Company's license under Section 1.1(c) will become perpetual, irrevocable, royalty-free, transferable, sublicensable, and fully paid-up."

7) Section 6.3 of the License Agreement shall be amended and restated in its entirety as follows:

"6.3 Early Termination by Penn. Penn may terminate this Agreement if: (a) Company is more than **** late in paying to Penn any amounts owed under this Agreement and does not pay Penn in full within **** after receipt of written notice indicating such default and demanding payment, including accrued interest (a "*Payment Default*"); (b) other than a Payment Default, Company or its Affiliate fails to achieve a diligence event on or before the applicable completion date or otherwise breaches this Agreement and does not cure such failure or breach within **** after written notice of the breach; or (c) Company or its Affiliate experiences a Trigger Event."

8) Section 6.4 of the License Agreement shall be amended and restated in its entirety as follows:

"6.4 Trigger Event. The term "*Trigger Event*" means any of the following: (a) if Company or its Affiliate (i) becomes insolvent, bankrupt or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver or trustee for it or its property and, if

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appointed without its consent, not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation or the reorganization, readjustment or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Company or its Affiliate of any proceeding under any law related to bankruptcy, insolvency, liquidation or the reorganization, readjustment or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4(a) or (b) above; (d) the calling by Company or its Affiliate of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; (e) the act or failure to act by Company or its Affiliate indicating its consent to, approval of or acquiescence in any of the proceedings described in Section 6.4(b) (d) above; or (f) the commencement by Company of any action against Penn, including an action for declaratory judgment, to declare or render invalid or unenforceable the Patent Rights, or any claim thereof."

9) Section 6.5 of the License Agreement shall be amended and restated in its entirety as follows:

"6.5 Effect of Termination. Upon the early termination of this Agreement pursuant to Section 6.2 or 6.3: (a) the Licenses shall terminate; (b) Company and all its Affiliates will cease all making, having made, using, importing, offering for sale and selling all Licensed Products and practicing the Licensed Processes; (c) Company shall assign and Penn will accept the assignment of all sublicenses granted to sublicensees to the extent related solely to the Patent Rights or the Licensed Products; provided, however, that any such sublicensee (i) is not in breach of any provision of this Agreement or the applicable sublicense agreement as of the effective date of such termination and (ii) upon request by Penn, agrees in writing that such sublicensee will perform all obligations of Company under this Agreement that are applicable to the sublicensed rights; (d) Company will pay to Penn all amounts, including accrued interest, owed to Penn under this Agreement and any Sponsored Research Agreement related to the Patent Rights, through the date of termination, including royalties on Licensed Products invoiced or shipped through the date of termination when such payments are received, whether or not payment is received prior to termination; (e) each Party will, at the other Party's request, return to such Party all confidential information of such Party; and (0 except as provided in Section 6.6, all rights and duties of Penn and the Company under this Agreement immediately terminate without further action required by either Penn or Company."

10) The second sentence of Section 13.5 is amended and restated as follows:

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Notwithstanding the foregoing, provided that Company is not in breach of any provisions of this Agreement, Company shalt be permitted to assign this Agreement, without the prior written consent of Penn, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates to a company in the business of developing and commercializing pharmaceutical products that has, together with its affiliates, a market value or, in the case of a publicly traded company listed on a nationally recognized exchange, market capitalization, of at least $250,000,000.

11. For clarity, Exhibit A now includes, in Part 2, all available intellectual property and technology owned and controlled by Penn and created at Penn in the laboratory of Dr. James M. Wilson, MA., Ph.D. through the Second Amendment Effective Date in the disease indications of familial hypercholesterolemia (FH) and ornithine transcarbarnylase deficiency (OTC), including any related data and know-how.

12. This Second Amendment, together with the License Agreement and First Amendment, constitute the entire agreement between the Parties. All other terms and provisions of the License Agreement, except as expressly amended by this Second Amendment, remain in full force and effect.

13. This Second Amendment may be executed in two or more counterparts, each of which shall be deemed an original and together shall be deemed one and the same instrument.

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Second Amendment to be executed by their duly authorized representatives.

THE TRUSTEES OF THE UNIVERSITY OF PENNSYLVANIA

By:	/s/ John S. Swartley, Ph.D.
Name:	John S. Swartley, Ph.D.
Title:	Associate Vice President for Research
	Executive Director, PCI
Date:	September 9, 2014

REGENX BIOSCIENCES, LLC

By:	/s/ Kenneth T. Mills
Name:	Kenneth T. Mills
Title:	President and CEO
Date:	September 9, 2014

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A

Patents and Patent Applications in the Patent Rights

Part 1; No Field of Use Limitation

Penn #	Disclosure Title		US Patents		Foreign Patents
****	****	****		****	
****	****	****		****	
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****	****	****		****	

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Docket V5085 — **Combination method to generate novel and/or hybrid coding sequences within the defined domains**

Serial No	Patent No	App Type	File Date	Country	Issue Date
****		****	****	****	
****		****	****	****	

Docket V5131 — **A domain of the adeno-associated virus stereotype 6 capsid that confers transduction of conducting airway epithelium**

Serial No	Patent No	App Type	File Date	Country	Issue Date
****		****	****	****	
****		****	****	****	
****		****	****	****	
****		****	****	****	
****		****	****	****	
****		****	****	****	

Docket X5836 — **The receptor for AAV9 and its modification in vivo to improve lung gene therapy**

Serial No	Patent No	App Type	File Date	Country	Issue Date
****		****	****	****	
****		****	****	****	
****		****	****	****	
****		****	****	****	
****		****	****	****	
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Docket Z6622	**Treatment of MPS1**				
Serial No	**Patent No**	**App Type**	**File Date**	**Country**	**Issue Date**
****		****	****	****	
****		****	****	****	

Exhibit A, Part 2
Field of Use limited to viral vector mediated delivery of gene therapy product

Docket 14-7025	**Improved AAV-LDLR for treating human disease**				
Serial No	**Patent No**	**App Type**	**File Date**	**Country**	**Issue Date**
****		****	****	****	
****		****	****	****	

Docket 14-7037	**AAV OTC for treating human disease**				
Serial No	**Patent No**	**App Type**	**File Date**	**Country**	**Issue Date**
****		****	****	****	

Penn / Wilson Lab Know-How for the Familial Hypercholesterolemia and Onithine Transcarbamylase Deficiency (OTC) Programs

FH Know-How (associated with ****)

****

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

OTC Know-How (associated with ****)

****

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Execution Version
Confidential

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of March 6, 2009 ("Effective Date") by and between ReGenX, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("ReGenX") and SmithKline Beecham Corporation, a Pennsylvania corporation doing business as GlaxoSmithKline, with offices at One Franklin Plaza, 200 North 16th Street, Philadelphia, Pennsylvania, 19102 ("GSK"). ReGenX and GSK are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, pursuant to a license agreement by and between GSK and the University of Pennsylvania ("Penn"), dated as of May 31, 2002, as amended from time to time (the "Penn License Agreement") GSK has exclusive rights under certain Penn Patent Rights (as defined herein) pertaining to various recombinant adeno-associated virus vectors; and

WHEREAS, ReGenX desires to obtain an exclusive sublicense from GSK under the Penn Patent Rights (as defined herein).

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "Affiliate" means any legal entity directly or indirectly controlling, controlled by or under common control with a Party or sublicensee. For purposes of this Agreement, "control" means the direct or indirect ownership of more than fifty percent (50%) of the outstanding voting securities of a legal entity, or the right to receive more than fifty percent (50%) of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.2 "Calendar Quarter" means each three (3) month period or any portion thereof, beginning on January 1, April 1, July 1 and October 1.

1.3 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes and other proprietary ideas, whether or not patentable or copyrightable, of either Party (a) that is identified as confidential or proprietary at the time of disclosure; or (b) whose confidential or proprietary status would be reasonably apparent under the circumstances. Notwithstanding the foregoing, Confidential Information shall not include the following:

1.3.1 information that is lawfully known to the receiving Party prior to the time of disclosure or independently developed by the receiving Party without use of the disclosing Party's Confidential Information, in each case, to the extent evidenced by written records;

1.3.2 information disclosed to a Party by a Third Party that has a right to make such disclosure;

1.3.3 information that becomes patented, published or otherwise part of the public domain as a result of acts by the Party owning such information or a Third Party obtaining such information as a matter of right; or

1.3.4 information that is required to be disclosed by order of a governmental authority or a court of competent jurisdiction; provided that the Receiving Party (as defined below) must use reasonable efforts to obtain confidential treatment of such information by the agency or court and notifies the Disclosing Party (as defined below) in the event that such information is required to be disclosed pursuant to Section 5.2.

1.4 “Domain Antibody” ****.

1.5 “GSK Collaborators” means entities (a) with which GSK has an active drug research and development agreement; and (b) from which GSK has retained substantial commercial rights to products derived from the development of drug candidates in the RNAi or antisense field.

1.6 “Licensed Product” means (a) products which are made, made for, used, sold or imported by ReGenX, its Affiliates and any of its or their sublicensees, the manufacture, use, sale or import of which, in the absence of the license granted pursuant to this Agreement, would infringe at least one Valid Claim in the country of manufacture, use, sale or import, including products manufactured by a process which would infringe at least one Valid Claim in the country of manufacture, use, sale or import; or (b) services sold by ReGenX, its Affiliates or its or their sublicensees which, in the absence of the licenses granted pursuant to this Agreement, would infringe at least one Valid Claim of the Penn Patent Rights in the country of sale.

1.7 “Muscular Dystrophy” ****.

1.8 “Net Sales” means the gross receipts from sales of a Licensed Product by ReGenX and/or its Affiliates and/or its or their sublicensees to Third Parties under this Agreement less deductions for ****.

1.9 “Penn Patent Rights” means all United States patents and patent applications, re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications listed in Exhibit 1 which cover adeno-associated vectors.

1.10 “ReGenX Materials” means those vector materials that are (a) the subject of a claim within the Penn Patent Rights; or (b) made using a process that is the subject of a claim within the Penn Patent Rights, in each case, that ReGenX or its Affiliates offer for commercial sale from time-to-time to Third Parties and that are intended and licensed for research uses only, but excluding any research uses in humans.

1.11 “Retained Rights” shall have the meaning set forth in Section 2.1

1.12 “Third Party” means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.13 “Valid Claim” means a claim of an issued and unexpired patent included within the Penn Patent Rights, which has not lapsed, been abandoned, been held revoked or deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. GSK hereby grants to ReGenX an exclusive (except as provided in Section 2.2 and in this Section 2.1), royalty-bearing, worldwide right and license, with the right to grant sublicenses, under the Penn Patent Rights to make, have made, use, import, sell and offer for sale Licensed Products anywhere in the world, including, for the avoidance of doubt, the right to conduct research and development under the Penn Patent Rights. No other rights are granted. Notwithstanding the foregoing, GSK retains the following rights (individually and collectively, the “**Retained Rights**”) with respect to the Penn Patent Rights:

2.1.1 An exclusive (even as to ReGenX), fully sublicensable right under the Penn Patent Rights to make, have made, use, sell, offer to sell and import Domain Antibodies which are expressed by an adeno-associated vector that is the subject of at least one Valid Claim or a claim in a pending patent application within the Penn Patent Rights.

2.1.2 A non-exclusive right, sublicensable only to GSK’s Affiliates and GSK Collaborators, under the Penn Patent Rights to make, have made, use, sell, offer to sell and import products that deliver RNA interference and antisense drugs using an adeno-associated vector that is the subject of at least one Valid Claim or a claim in a pending patent application within the Penn Patent Rights. GSK will provide to ReGenX reasonable written notice of any sublicense it grants to any GSK Collaborator pursuant to the Retained Rights under this Section 2.1.2, and such notice shall include the field for which such sublicense has been granted.

2.1.3 A non-exclusive right to use the Penn Patent Rights solely for internal (except as provided for in this Section 2.1.3), non-commercial research purposes and for GSK's discovery research efforts with non-profit organizations. Such right shall be sublicensable only to (a) GSK's Affiliates; and (b) GSK Collaborators; provided, however, that for GSK Collaborators, GSK shall provide written notice to ReGenX of such sublicense and such sublicense shall be limited only to the field of research identified for such collaboration; provided that ReGenX acknowledges that fee-for-service work with Third Parties will not be considered a sublicense. In addition, ReGenX acknowledges that GSK has granted a sublicense to the Penn Patent Rights solely for non-commercial research purposes to one (1) Third Party sublicensee who is not an Affiliate of GSK or a GSK Collaborator. Pursuant to the sublicense agreement between GSK and such Third Party sublicensee, such sublicensee is permitted to sublicense the rights granted pursuant to such sublicense to its Affiliates and contract research organizations who are performing specific research on behalf of such sublicensee. Upon termination or expiration of the sublicense agreement with such Third Party sublicensee, GSK shall have no right to grant any further sublicenses under this Section 2.1.3 except to GSK Affiliates and GSK Collaborators, as set forth herein.

2.1.4 In order to comply with potential and existing Third Party commitments existing as of the Effective Date, an exclusive, worldwide, sublicensable right under (a) the Penn Patent Rights which cover the rAAV serotype 8, to make, have made, use, sell, offer for sale and import Licensed Products for the treatment of all forms of hemophilia B; or (b) the Penn Patent Rights which cover rAAV serotype 9, to make, have made, use, sell, offer for sale and import Licensed Products for the treatment of (i) all forms of Muscular Dystrophy; (ii) congestive heart failure suffered by Muscular Dystrophy patients; (iii) all forms of cardiovascular disease by delivery of genes encoding I-lc and Serca2a and creatine kinase and (iv) amyotrophic lateral sclerosis, acid maltase deficiency and spinal muscular atrophies; provided, however, that with respect to category (iv), GSK agrees to use its commercially reasonable efforts to provide these rights to ReGenX within forty-five (45) days of the Effective Date; provided further, that GSK shall be under no obligation to make payments or otherwise incur financial obligations in order to provide such rights to ReGenX. In the event that GSK's existing commitments to any such Third Party cease, GSK's Retained Rights under this Section 2.1.4 shall be exclusively licensed to ReGenX.

2.1.5 In order to comply with Third Party commitments existing as of the Effective Date, a non-exclusive, sublicensable right to make, have made, and use all of the various serotypes of any adeno-associated vector that is the subject of at least one claim in the Penn Patent Rights solely for non-commercial research in the areas of Muscular Dystrophy, hemophilia B, congestive heart failure suffered by Muscular Dystrophy patients, and other cardiovascular disease. In no event shall the Retained Rights granted under this Section 2.1.5 be used for any commercial purposes. In the event that GSK's existing commitments to any such Third Party cease, GSK's Retained Rights under this Section 2.1.5 shall be exclusively

licensed to ReGenX, subject to GSK's Retained Rights under Section 2.1.1, 2.1.2 and 2.1.3.

2.2 Penn Retained Rights. Notwithstanding anything to the contrary in Section 2.1, Penn may use and permit other non-profit organizations to use the Penn Patent Rights for educational and non-commercial research purposes only.

2.3 Government Rights. ReGenX acknowledges that, pursuant to Public Laws 96-517, 97-256 and 98-620, codified at 35 U.S.C. §§ 200-212, the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant or similar agreement with a federal agency. Pursuant to these laws, the government may impose certain requirements regarding such intellectual property, including but not limited to the requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States. The license grant hereunder is expressly subject to all applicable United States government rights as provided in the above-mentioned laws and any regulations issued under those laws, as those laws or regulations may be amended from time to time.

2.4 Sublicensing. The right to sublicense granted to ReGenX under this Agreement is subject to the following conditions:

2.4.1 In each such sublicense, ReGenX must require that the sublicensee be subject to the material terms and conditions of the licenses granted to ReGenX under this Agreement.

2.4.2 Within **** after ReGenX enters into any sublicense, ReGenX must send to GSK a complete copy of the sublicense written in the English language in order for GSK to send such sublicense to Penn, pursuant to the terms of the Penn License Agreement.

2.4.3 In the event ReGenX enters into sublicenses, ****

2.5 License to GSK. ReGenX hereby grants to GSK a non-exclusive, worldwide, royalty-free license to use any patentable modifications or improvements developed by ReGenX to any vector which is the subject of a claim within the Penn Patent Rights consummate in scope to the Retained Rights set forth in Sections 2.1.1 through 2.1.3 above ("**Licensed Back Improvements**"). Such license shall be sublicensable solely as described and within the scope of the Retained Rights under Sections 2.1.1 through 2.1.3. ReGenX shall provide reasonable notice to GSK upon the filing of any patent application covering such Licensed Back Improvements.

2.6 Ownership. Subject to any licenses expressly set forth herein, ReGenX shall own and retain all right, title and interest (including all intellectual property rights) in all technical information, inventions (whether or not patentable), developments, discoveries, software, know-

how, methods, techniques, formulae, animate and inanimate materials, data, processes and other proprietary information, including without limitation the Licensed Products, that are created, discovered, conceived or reduced to practice by ReGenX, its Affiliates and Third Party collaborators using the rights granted to ReGenX hereunder.

ARTICLE 3: CONSIDERATION

3.1 ReGenX Equity. In consideration for the licenses granted to ReGenX hereunder, upon the Effective Date, ReGenX will issue to GSK ****in ReGenX.

3.2 Milestone Fees. ReGenX will pay to GSK the following milestone payments the first time a Licensed Product that fits within Category la or lb, as listed in Section 3.3, achieves such milestone event:

Milestone	Exclusive License
Phase I Entry	****
Phase III Entry	****
Product Approval	****

****

3.3 Royalties. In further consideration of the license granted to ReGenX, ReGenX shall pay to GSK the following royalty based upon Net Sales of Licensed Products, subject always to the reductions in royalty rates set forth in Sections 3.3.4 and 3.3.5:

LICENSED PRODUCTS	Royalty Percentage	Cumulative Annual NET SALES (Million $)
1a. Novel Vector	****	Up to ****;

	****	In excess of **** up to ****; and In excess of ****.
1b. i) Refinement of an existing vector or ii) a Novel Vector made by ReGenX through the material use of technology recited in a Valid Claim	****	Up to ****; In excess of **** up to ****; and In excess of ****.
2. Licensed Products that would otherwise infringe a Valid Claim covering a therapeutic use of a vector or compound.	****	Up to ****; In excess of **** up to ****; and In excess of ****.
3. Manufacturing technology that would otherwise infringe a Valid Claim covering a method or process of manufacture	****	Up to ****; In excess of **** up to ****; and In excess of ****.

3.3.1 Description of Categories:

3.3.1.1 To meet the requirements of the term “Novel Vector” (as used in Categories la and lb(ii)), there must be neither any dominating Third Party patent, including any Penn-owned patent rights other than those licensed under this Agreement with respect to the vector per se (i.e., no Third Party patent or Penn-owned patent rights beyond those licensed under this Agreement is required in order to make, have made, use, import, offer for sale or sell the vector for the higher royalty level to apply). Licenses from Penn or GSK to ReGenX for genes used, promoters used other than those which are part of the vector as described in Penn Patent Rights and the like in the vector will not affect the royalty pursuant to this provision. If any dominating Third Party patent or any Penn-owned patent other than those licensed under this Agreement issues at any time during the term of this Agreement with respect to a vector licensed hereunder, then the royalty level will immediately drop to the “Refinement” level (Category lb above) for any Licensed Product containing such vector.

3.3.1.2 As used in Category 2 of the Table in Section 3.3, a “therapeutic use” shall mean use in the prevention and/or treatment of a disease in a patient. For the

avoidance of doubt, ReGenX shall have no obligation under Section 3.3 or under any other provision of this Agreement to pay any royalties on any "reach-through" basis for any reason whatsoever, including without limitation, on the basis of the use by ReGenX of any subject matter recited in a Valid Claim in the development, selection or advancement of any Licensed Product, except only as may be expressly provided under Category lb(ii) in Section 3.3 above.

3.3.1.3 In the event that any Licensed Product does not fall into any of the Categories 1-3 above, ReGenX shall pay to GSK a royalty of **** based upon Net Sales of such Licensed Product, subject always to the reductions in royalty rates set forth in Sections 3.3.4 and 3.3.5, and subject always to the terms and conditions of this Section 3.3.

3.3.2 One Royalty: For any single Licensed Product, ****. In addition, the **** royalty shall be subject to the **** provided below.

3.3.4 Valid Claims: Except as set forth below, royalties will only apply to Net Sales of Licensed Products that are the subject of at least one Valid Claim of any issued patent in the Penn Patent Rights. ****.

3.3.5 Third Party Royalties Stacking Provision: If, in connection with the manufacture, use or commercialization of a given Licensed Product, ReGenX is obligated to pay royalties to GSK and any Third Parties that, ****, then the royalty owed to GSK for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C*(A/(A+B)))

Where
R = Reduction of GSK royalty.
A = Unreduced GSK royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****.

Example Calculation:

assume: i) all Third Party royalties = ****%
ii) unreduced GSK royalty = ****%
iii) projected total royalty = ****%

R = (****-****)*(****/(**** + ****))
R = (*********)
R = ****
GSK Stacked Royalty = ****-**** = ****%

Notwithstanding the foregoing, ReGenX will pay to GSK no less than **** of the royalties that ReGenX would otherwise pay to GSK if there were no royalties due to Third Parties.

3.3.6 Termination of Obligation to Pay Royalty: ****.

3.4 Sublicense Fees. ReGenX shall pay GSK a percentage of any sublicense fees (****) received by ReGenX for the Penn Patent Rights from any sublicensee; ****. The applicable percentage due to GSK for each sublicense shall be:

**** - if sublicensed on or before the **** of the Effective Date;

**** - if sublicensed on or before the **** of the Effective Date, but after the **** of the Effective Date; and

**** - if sublicensed after the **** of the Effective Date.

Notwithstanding anything to the contrary herein, in the event ReGenX receives non-cash consideration for any sublicense granted to a Third Party hereunder, and such non-cash consideration is liquidated ****, then, with respect to the proceeds resulting from the liquidation of such non-cash consideration, ReGenX shall pay to GSK the percentage of sublicense fees that **** if such payment had been received in cash.

3.5 Reports and Records.

3.5.1 ReGenX must deliver to GSK within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to GSK for such Calendar Quarter, including, without limitation:

3.5.1.1 Number of Licensed Products included within Net Sales, listed by country;

3.5.1.2 Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

3.5.1.3 Qualifying costs to be excluded from the gross consideration, as defined in Section 1.7, listed by category of cost;

3.5.1.4 Net Sales of Licensed Products listed by country; and

3.5.1.5 Royalties owed to GSK, listed by category, including without limitation earned and sublicensee-derived categories.

3.5.2 ReGenX shall pay the royalties due under Section 3.3 and other payments due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue or the other consideration is received. ReGenX shall send the royalty payments along with the report described in Section 3.5.1.

3.5.3 ReGenX shall maintain and require its Affiliates and its or their sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records for each Calendar Quarter must be maintained for **** after the submission of each report under Article 3. If any such verification determines that ReGenX has underpaid royalties by **** or more of the money actually owed, ReGenX shall pay the costs and expenses of GSK and its accountants in connection with their review or audit. Such audit or review shall be made by an independent certified public accountant, and only upon reasonable prior written notice to ReGenX (not less than **** notice) and during ReGenX's normal working hours; and shall not be made more than once in any given year.

3.6 Currency, Place of Payment, Interest.

3.6.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to GSK under this Agreement must be made in United States dollars.

3.6.2 If ReGenX receives revenues from the sale of Licensed Product in currency other than United States dollars, revenues shall be converted into United States dollars at the conversion rate used by GSK in producing its quarterly and annual accounts, as confirmed by GSK's auditors. If government regulations prevent remittances from a foreign country with respect to sale made in that country, the obligation to pay royalties on the sale in that country shall be suspended until such remittances are possible.

3.6.3 Notwithstanding anything herein to the contrary, in the event that any amounts due under the terms of this Agreement may be passed on by GSK to Penn or otherwise are ultimately payable to Penn under the terms of this Agreement, the Penn License Agreement or any step-through or other agreement entered into by ReGenX and Penn, ReGenX shall have the right to make such payment directly to Penn and deduct any such amounts from fees due to GSK (as applicable) hereunder.

3.7 Favored Nation Pricing to GSK. During the term of this Agreement, ReGenX agrees to provide ReGenX Materials to GSK at a price that is equal to or lower than the price **** for the same (in substance and amount and on substantially the same terms) products at the time of such GSK purchase. ****.

ARTICLE 4: DILIGENCE

4.1 ReGenX shall use commercially reasonable efforts to develop, market, promote and sell a Licensed Product ****. Without limiting the foregoing, ReGenX shall be deemed to have satisfactorily discharged its diligence obligations hereunder as long as ReGenX and/or its Affiliates and/or sublicensees are diligently pursuing the development and/or commercialization of ****.

4.2 Within **** after the Effective Date and within **** of each December 1 thereafter, ReGenX shall provide GSK with written progress reports, setting forth in such detail as GSK may reasonably request, the progress of the development, evaluation, testing and commercialization of each Licensed Product. ReGenX acknowledges that GSK is required to notify Penn within **** of the first commercial sale by ReGenX, its Affiliates, or its or their sublicensees of each Licensed Product. Such a report ("**Development Progress**

Report"), setting forth the current stage of development of Licensed Products, shall include, without limitation:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by ReGenX, its Affiliates, and its or their sublicensees relating directly to the Licensed

4.2.3 Product since the last Development Progress Report;

4.2.4 Significant research and development projects relating directly to the Licensed Product currently being performed by ReGenX, its Affiliates, and its or their sublicensees and projected dates of completion.

4.2.5 Future development activities to be undertaken by ReGenX, its Affiliates or its or their sublicensees during the next reporting period relating directly to the Licensed Product;

4.2.6 Projected total development remaining before product launch of each Licensed Product; and

4.2.7 Summary of significant development efforts using the Penn Patent Rights being performed by Third Parties including the nature of the relationship between ReGenX and such Third Parties.

4.3 In the event that Penn and GSK modify the Penn License Agreement or any of the terms or provisions thereof (whether by formal amendment, letter agreement or other binding or applicable arrangement) where such modification has a material effect on ReGenX's obligations under this Agreement (a "**PLA Modification**"), GSK shall provide notice of such PLA Modification in writing to ReGenX. The Parties shall then enter into discussions to determine whether this Agreement should be amended to reflect the PLA Modification in the Penn License Agreement so that ReGenX may benefit from all applicable terms of the PLA Modification to the extent that the PLA Modification lessens GSK's obligations or requirements thereunder or otherwise benefits GSK. ****.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "**Receiving Party**"), agrees that it will (a) treat Confidential Information of the other Party (the "**Disclosing Party**") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted by the

license grants contained herein; provided that such disclosure be under confidentiality agreements with provisions comparable to those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly. A Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those under this Agreement.

5.2 No Public Announcement. No public announcement or other disclosure to Third Parties concerning the existence of or terms of this Agreement shall be made, either directly or indirectly, by any Party to this Agreement, except as set forth in this Section 5.2 or to the extent legally required; provided that either Party may make such a disclosure of the existence of and/or terms of this Agreement to ****, who are obligated to keep such information confidential on terms no less stringent than those set forth herein. Furthermore, GSK shall be permitted to provide a copy of this Agreement to Penn, pursuant to GSK's obligations under the Penn License Agreement. In the event that the Receiving Party becomes obligated by law to disclose the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement, the Receiving Party may disclose such Confidential Information without liability hereunder; provided, that the Receiving Party shall furnish only such portion of the Confidential Information which is legally required to be disclosed and only to the extent required by law.

5.3 Treatment of Development Progress Reports. All Development Progress Reports provided by ReGenX to GSK shall be considered the Confidential Information of ReGenX. GSK shall be permitted to provide such Development Progress Reports to Penn, subject to GSK's obligations of confidentiality hereunder. Notwithstanding anything to the contrary in this Article 5., GSK shall be obligated to keep such Development Progress Reports confidential for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, terminates upon the expiration, lapse, abandonment or invalidation of the last Valid Claim to expire, lapse, become abandoned or unenforceable in all the countries of the world where Penn Patent Rights existed; provided, however, that if not one patent ever issues from the Penn Patent Rights, then this Agreement will terminate **** after the first commercial sale of the first Licensed Product in any country. Expiration of this Agreement or expiration of ReGenX's obligation to pay royalties to GSK in any country hereunder shall not preclude ReGenX from continuing to market, have marketed, sell and have sold Licensed Product in such country without further payment or obligation to GSK.

6.2 ReGenX's Right to Terminate. ReGenX may, upon **** written notice to GSK, terminate this Agreement for any reason, with or without cause.

6.3 Termination for Breach. GSK may terminate this Agreement if:

6.3.1 ReGenX is more than **** late in paying to GSK royalties, expenses, or any other monies due under this Agreement and ReGenX does not pay GSK in full within **** upon written demand from GSK; or

6.3.2 ReGenX materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach.

6.4 Termination for Insolvency. In the event that ReGenX files for protection under bankruptcy laws, makes a general assignment for the benefit of creditors, appoints or suffers appointment of a receiver or trustee over its business, files a petition under any bankruptcy or insolvency act or has any such petition filed against it which is not dismissed or stayed within **** of the filing thereof, GSK may terminate this Agreement effective immediately upon written notice to ReGenX.

6.5 Effects of Termination. The effect of termination by ReGenX pursuant to Section 6.2, or by GSK pursuant to Section 6.3 or 6.4 shall be as follows:

6.5.1 ReGenX shall cease to make, have made, use, import, sell and offer for sale all Licensed Products;

6.5.2 ReGenX shall assign to GSK all sublicenses granted to Third Parties related solely to the Penn Patent Rights or the Licensed Products;

6.5.3 ReGenX shall grant, and hereby grants to GSK a non-exclusive, perpetual, irrevocable, worldwide, royalty-free license under any patentable modifications or improvements developed by ReGenX to any vector that is the subject of a claim in any of the Penn Patent Rights, for use by GSK for the research, development and commercialization of products in any therapeutic indication. Such license shall be (a) fully sublicensable to GSK's Affiliates, GSK Collaborators, and Third Parties with respect to the research, development and commercialization of Domain Antibodies, and (b) only sublicensable to GSK's Affiliates and GSK Collaborators for all other purposes;

6.5.4 ReGenX shall transfer to GSK all remaining ReGenX Materials that have been requested by and made for GSK under Section 3.7 prior to the effective date of termination at GSK's cost and expense;

6.5.5 ReGenX shall pay all monies then-owed to GSK under this Agreement; and

6.5.6 Each Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.6 Survival. ReGenX's obligation to pay all monies due and owed to GSK under this Agreement which have matured as of the effective date of termination shall survive the termination of this Agreement. In addition, the provisions of Articles 5—Confidentiality, Article 6—Term and Termination, Article 8—Warranties; Indemnification, Article 9—Use of ReGenX's and GSK's Name; and Article 10—Additional Provisions shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Penn Licensed Patents. Pursuant to the Penn License Agreement, Penn controls the preparation, prosecution and maintenance of the Penn Patent Rights. As of the Effective Date, Penn and GSK have agreed on the selection of appropriate patent counsel to carry out Penn's prosecution responsibilities. If, after the Effective Date, Penn seeks to appoint different patent counsel, GSK will not agree to such patent counsel without consultation with ReGenX. GSK shall provide to ReGenX a copy of any paper in a reasonable time prior to submission to the Patent and Trademark Office for review and comment, and GSK shall reasonably consider any comments thereon by ReGenX and relay such comments to Penn. Further, GSK shall keep ReGenX informed of the progress of any patent filings. ReGenX shall also have the right to consult directly with Penn patent counsel. The Parties shall keep each other informed with respect to any material communications with Penn regarding the Penn Patent Rights licensed hereunder. **** upon receipt of an invoice for all documented and reasonable Third Party expenses incurred in connection with the filing, prosecution and the maintenance of the Penn Patent Rights. If, in ReGenX's opinion, the costs of preparation, prosecution and maintenance are inappropriate, then ReGenX and GSK shall discuss and agree on a resolution. If ReGenX elects not to pay for the filing, prosecution or maintenance of any patent or patent application within the Penn Patent Rights, ReGenX shall provide GSK with reasonable advance notice in order to enable GSK to assume responsibility for the filing, prosecution or maintenance of any such patent or patent application, and then such patent or patent application shall no longer be a part of the Penn Patent Rights licensed hereunder.

7.2 Infringement Actions Against Third Parties.

7.2.1 ReGenX and GSK are responsible for notifying each other promptly of any infringement of Penn Patent Rights (other than Retained Rights) which may come to their attention. ReGenX and GSK shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 To the extent permitted under the Penn License Agreement, ReGenX may prosecute such infringement at its own expense. ReGenX shall not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on GSK or Penn or grants any rights to the Penn Patent Rights other

than rights which ReGenX has the right to grant under this Agreement, without GSK's prior written permission. All monies recovered upon the final judgment or settlement of any such action shall be used (a) first, to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of ****, **** and ****; (b) second, to **** to account for lost sales or lost profits (to the extent that damages are awarded for lost sales or lost profits from the sale ****); (c) third, to **** to the extent necessary to account for the royalties that would have been payable to **** but for the lost sales or lost profits; and (d) the remainder to the account of the **** that undertake such actions to the relative extent of their financial participation therein.

7.2.3 ReGenX's rights under Section 7.2 are subject to the continuing right of Penn and GSK to intervene at Penn's or GSK's own expense and join ReGenX in any claim or suit for infringement of the Penn Patent Rights. Any consideration received by GSK or Penn in the settlement or award for any claim or suit shall be shared between ReGenX, GSK and Penn as set forth in Section 7.2.2 above.

7.2.4 If ReGenX elects to pursue an infringer under Section 7.2.2 above and ReGenX fails to prosecute such infringement, then GSK may prosecute such infringement at its own expense. In such event, financial recoveries will be entirely retained by GSK.

7.2.5 In any action to enforce any of the Penn Patent Rights, either Party, at the request and expense of the other Party shall cooperate to the fullest extent reasonably possible, including in the event that if either Party is unable to initiate or prosecute such action solely in its own name, the other Party shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. This provision shall not be construed to require either Party to undertake any activities, including legal discovery, at the request of any Third Party except as may be required by lawful process of a court of competent jurisdiction.

7.3 Defense of Infringement Claims.

7.3.1 In the event ReGenX or GSK becomes aware that ReGenX's or any of its Affiliates' or sublicensees' practice of the Penn Patent Rights is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other and the Parties shall consider the claim and the most appropriate action to take. ReGenX shall cause each of its Affiliates and sublicensees to notify ReGenX promptly in the event such entity becomes aware that its practice of the Penn Patent Rights is the subject of a claim of patent infringements by another. ReGenX shall have the right to control the defense of any such suit brought against ReGenX or any of its Affiliates or sublicensees and shall do so at its own expense. ReGenX shall have the right to require GSK's and Penn's reasonable cooperation in any such suit, upon written notice to GSK and Penn, and GSK and Penn shall have the obligation to participate and ReGenX shall bear the cost of

GSK's and Penn's participation. ReGenX must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on GSK or Penn or grants any rights to the Penn Patent Rights other than rights which ReGenX has the right to grant under this Agreement, without GSK's prior written permission.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty. GSK represents, warrants and covenants to ReGenX that it has sufficient rights in the Penn Patent Rights to grant to ReGenX the rights specified in this Agreement.

8.2 Disclaimer of Warranties.

8.2.1 Disclaimer by GSK. EXCEPT AS SET FORTH IN SECTION 8.1, THE PENN PATENT RIGHTS, LICENSED PRODUCTS AND ALL OTHER TECHNOLOGY LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS AND GSK MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, GSK MAKES NO REPRESENTATIONS OR WARRANTIES (i) OF COMMERCIAL UTILITY; (ii) OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE; OR (iii) THAT THE USE OF THE PENN PATENT RIGHTS, LICENSED PRODUCTS AND ALL TECHNOLOGY LICENSED UNDER THIS AGREEMENT WILL NOT INFRINGE ANY PATENT, COPYRIGHT OR TRADEMARK OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, GSK SHALL NOT BE LIABLE TO REGENX, REGENX'S SUCCESSORS OR ASSIGNS OR ANY THIRD PARTY WITH RESPECT TO: ANY CLAIM ARISING FROM REGENX'S USE OF THE PENN PATENT RIGHTS, LICENSED PRODUCTS AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE MANUFACTURE, USE OR SALE OF LICENSED PRODUCTS; OR ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL OR CONSEQUENTIAL DAMAGES OF ANY KIND.

8.2.2 Disclaimer by ReGenX. THE REGENX MATERIALS AND ALL LICENSED BACK IMPROVEMENTS UNDER SECTION 2.5 PROVIDED OR LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS AND REGENX MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, REGENX MAKES NO REPRESENTATIONS OR WARRANTIES (i) OF COMMERCIAL UTILITY; (ii) OF MERCHANTABILITY OR FITNESS FOR A PARTICULAR PURPOSE; OR (iii) THAT THE USE OF THE REGENX MATERIALS THE LICENSED BACK IMPROVEMENTS AND ALL TECHNOLOGY LICENSED UNDER THIS AGREEMENT WILL NOT INFRINGE ANY PATENT, COPYRIGHT OR TRADEMARK OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, REGENX SHALL NOT BE LIABLE TO GSK, GSK'S SUCCESSORS OR ASSIGNS OR ANY THIRD PARTY WITH RESPECT TO: ANY CLAIM ARISING FROM GSK'S USE OF THE REGENX MATERIALS, THE

LICENSED BACK MATERIALS AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE MANUFACTURE, USE OR SALE OF PRODUCTS BASED THEREON; OR ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL OR CONSEQUENTIAL DAMAGES OF ANY KIND.

8.3 Indemnification.

8.3.1 By ReGenX. ReGenX shall defend, indemnify and hold harmless GSK, its officers, agents and employees (individually, a "**GSK Indemnified Party**", and collectively, the "**GSK Indemnified Parties**"), from and against any and all liability, loss, damage, action, claim or expense (including attorneys' fees) (individually, a "**Liability**", and collectively, the "Liabilities") suffered or incurred by the GSK Indemnified Parties from Third Parties that results from or arises out of: ****; provided, however, that ReGenX shall not be liable for claims based on the gross negligence or intentional misconduct of any of the GSK Indemnified Parties. Without limiting the foregoing, ReGenX must defend, indemnify and hold harmless the GSK Indemnified Parties from and against any Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that was **** by ReGenX, its Affiliates, assignees, sublicensees, or vendors; and

(b) **** conducted by or on behalf of ReGenX or its Affiliates or sublicensees relating to the Penn Patent Rights or Licensed Products, including, without limitation, any claim by or on behalf of ****.

8.3.2 By GSK. GSK shall defend, indemnify and hold harmless ReGenX, its officers, agents and employees (individually, a "**ReGenX Indemnified Party**", and collectively, the "**ReGenX Indemnified Parties**"), from and against any and all Liabilities suffered or incurred by the ReGenX Indemnified Parties from Third Parties that results from or arises out of: ****; provided, however, that GSK shall not be liable for claims based on the gross negligence or intentional misconduct of any of the ReGenX Indemnified Parties. Without limiting the foregoing, GSK must defend, indemnify and hold harmless the ReGenX Indemnified Parties from and against any Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a product developed from or based on the ReGenX Materials or Licensed Back Improvements

that was **** **** by GSK, its Affiliates, assignees, sublicensees (other than ReGenX), or vendors; and

(b) **** conducted by or on behalf of GSK or its Affiliates or sublicensees (other than ReGenX) relating to a product developed from or based on the ReGenX Materials or Licensed Back Improvements, including, without limitation, any claim by or on behalf of ****.

8.4 Indemnification Procedure. Each Party, as an indemnifying party (a "**Indemnifying Party**"), shall not be permitted to settle or compromise any claim or action giving rise to Liabilities in a manner that imposes any restrictions or obligations on the other Party (the "**Indemnified Party**") (or Penn as applicable) or grant any rights to the Penn Patent Rights, Licensed Products, ReGenX Materials or Licensed Back Improvements other than those ReGenX has the right to grant under this Agreement without GSK's prior written consent. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within twenty (20) days after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Liabilities related thereto shall be conclusively deemed a Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained herein are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise.

8.5 Insurance. Prior to the first administration of a Licensed Product to a human, ReGenX shall obtain and/or maintain, at its sole cost and expense, **** insurance in amounts, which are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** per occurrence (or claim) and in the aggregate annually. Such **** insurance shall insure against all liability, including ****.

ARTICLE 9: USE OF A PARTY'S NAME

9.1 ReGenX and its employees and agents must not use and ReGenX must not permit its Affiliates or sublicensees to use GSK's or Penn's name or any adaptation thereof, or any GSK or Penn seal, logotype, trademark, or service mark, or the name, mark, or logotype of any GSK or Penn representative or organization in any way without the prior written consent of GSK or Penn; provided, however that ReGenX may acknowledge the existence and general nature of this Agreement.

9.2 GSK and its employees and agents must not use ReGenX's name or any adaptation thereof, or any ReGenX seal, logotype, trademark, or service mark, or the name, mark, or logotype of any ReGenX representative or organization in any way without the prior written consent of ReGenX; provided, however, that GSK may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between ReGenX and GSK, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of ReGenX and GSK hereunder shall inure to the benefit of, and shall be binding upon, their respective successors and assigns. Neither Party may assign its rights under this Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld; provided however, a Party may assign this Agreement to (a) any Affiliate of such Party or (b) any corporation or other entity to which such Party may transfer all or substantially all of its assets to which this Agreement relates ("Sale of Assets"). ReGenX shall notify GSK in writing at least thirty (30) days prior to the anticipated closing of any bona fide Sale of Assets it proposes to effect or any merger or consolidation pursuant to which the holders of the voting power of ReGenX immediately prior to such merger or consolidation hold, immediately after such merger or consolidation, less than 50% of the voting power of ReGenX ("Sale by Merger"). Upon ReGenX's closing of a Sale of Assets or Sale by Merger, GSK's obligations under Section 2.1.2 and Section 2.1.3 to provide written notice to ReGenX of any sublicense it grants to the Retained Rights shall cease. No assignment shall relieve such Party of responsibility for the performance of any accrued obligations which it has prior to such assignment.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date sent if sent by public courier (e.g. Federal Express) or by Express Mail, receipt requested, and addressed as follows:

If for ReGenX:

ReGenX, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer

with a copy to:

ReGenX, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel

If for GSK:

GlaxoSmithKline
Gunnels Wood Road
Mail Code 3T123

with a copy to:

GlaxoSmithKline
2301 Renaissance Blvd.
Mail Code RN0220

Stevenage
Hertfordshire SG12NY
United Kingdom
Attn: Vice President,
Business Development,
BioPharm Management and Administration

King of Prussia, PA 19406
Attn: Associate General
Counsel

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the Commonwealth of Pennsylvania, without giving effect to conflict of law provisions. In the event that a Party to this Agreement perceives the existence of a dispute with the other Party concerning any right or duty provided for herein, the Parties will, as soon as practicable, confer in an attempt to resolve the dispute. If the Parties are unable to resolve such dispute amicably, then the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the Eastern District of the Commonwealth of Pennsylvania with respect to any and all disputes concerning the subject of this Agreement.

10.6 No Discrimination. ReGenX and GSK, in their activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual or affectional preference, age, religion, national or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran of the Vietnam Era.

10.7 Compliance with Law. GSK and ReGenX must comply with all prevailing laws, rules and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes and other commodities, articles and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979, and that the Parties' obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by GSK or ReGenX that GSK or ReGenX shall not export data or commodities to certain foreign countries without prior approval of such agency. ReGenX neither represents that a license is not required nor that, if required, it will issue.

10.8 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral. This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this LICENSE AGREEMENT to be executed by their duly authorized representatives.

REGENX

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: CEO

SMITHKLINE BEECHAM CORPORATION d/b/a GLAXOSMITHKLINE

By: /s/ William J. Mosher
Name: William J. Mosher
Title: Vice President & Secretary

CONFIDENTIAL TREATMENT REQUESTED

Exhibit 1
Penn Patent Rights

Penn #	Disclosure Title	Inventors	Applicant(s)	US Patents	Foreign Patents
****	****	****	****	****	****
****	****	****	****	****	****
****	****	****	****	****	****
****	****	****	****	****	****
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****	****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Exhibit 2
Muscular Dystrophies

****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Exhibit 10.13

AMENDMENT TO LICENSE AGREEMENT

This Amendment to the License (this "Amendment") is entered into as of April 15, 2009 by and between ReGenX, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("ReGenX") and SmithKline Beecham Corporation, a Pennsylvania corporation doing business as GlaxoSmithKline, with offices at One Franklin Plaza, 200 North 16th Street, Philadelphia, Pennsylvania, 19102 ("GSK"). This Amendment amends that certain License Agreement dated March 6, 2009 by and between ReGenX and GSK (collectively, the "Parties") relating to certain patent rights covering adeno-associated vectors, as amended from time to time (the "License").

WHEREAS, the Parties wish to amend certain provisions of the License.

NOW, THEREFORE, in consideration of the promises and covenants contained in this Amendment, and intending to be legally bound, the Parties hereby agree as follows:

1. The first sentence of Section 2.1.3 is hereby amended to read as follows:

   "A non-exclusive right to use the Penn Patent Rights solely for internal (except as provided for in this Section 2.1.3) research purposes and for GSK's discovery research efforts with non-profit organizations and GSK Collaborators."

2. Section 2.1.4 is hereby deleted in its entirety and replaced with the following:

   "2.1.4 In order to comply with Third Party commitments existing as of the effective date of this Amendment, an exclusive, worldwide, sublicensable right under (a) the Penn Patent Rights which cover the rAAV serotype 8, to make, have made, use, sell, offer for sale and import Licensed Products for the treatment of all forms of hemophilia B; or (b) the Penn Patent Rights which cover rAAV serotype 9, to make, have made, use, sell, offer for sale and import Licensed Products for the treatment of (i) all forms of Muscular Dystrophy; (ii) congestive heart failure suffered by Muscular Dystrophy patients; and (iii) any and all cardiovascular diseases by delivery of any or all of genes encoding I-lc and Serca2a and creatine kinase. In the event that GSK's existing commitments to any such Third Party cease, GSK's Retained Rights under this Section 2.1.4 shall be exclusively licensed to ReGenX."

3. Except as specifically modified by this Amendment, all of the provisions of the License remain in full force and effect. The License and this Amendment constitute the entire agreement between ReGenX and GSK and supersede all other agreements and understandings between the Parties with respect to the subject matter of the License and this Amendment. This Amendment will be binding upon, and will inure to the benefit of, the parties and their respective successors and permitted assigns. This Amendment may be executed in one or more counterparts, all of which will be considered one and the same agreement. This Amendment will be governed by the

laws of the Commonwealth of Pennsylvania, without giving effect to conflict of laws provisions.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Amendment to be executed by their duly authorized representatives.

REGENX, LLC	SMITHKLINE BEECHAM CORPORATION d/b/a GLAXOSMITHKLINE
By: /s/ Kenneth T. Mills	By: /s/ Damien McDevitt
Name: Kenneth T. Mills	Name: Damien McDevitt
Title: President & CEO	Title: VP & Head Drug Discovery Transactions WWBD

CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of April 10, 2014 ("Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006, USA ("Licensor"), and AAVLife, a French simplified joint stock company (Société par actions simplifiée) whose registered office is 183/189 avenue de Choisy – 75013 Paris, France ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to certain recombinant adeno-associated virus vectors; and

WHEREAS, Licensee desires to obtain from Licensor certain licenses under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV7" means (a) the recombinant adeno-associated virus serotype 7 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 7 vector that are covered by the claims of the Licensed Patents set forth on Exhibit A-1 (or other Licensed Patents relating thereto described in Section 1.19(b) or 1.22(b), as applicable).

1.2 "AAV8" means (a) the recombinant adeno-associated virus serotype 8 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 8 vector that are covered by the claims of the Licensed Patents set forth on Exhibit A-2 (or other Licensed Patents relating thereto described in Section 1.19(b) or 1.22(b), as applicable).

1.3 "AAV9" means (a) the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector that are covered by the claims of the Licensed Patents set forth on Exhibit A-3 (or other Licensed Patents relating thereto described in Section 1.19(b) or 1.22(b), as applicable).

1.4 "AAVrhl0" means (a) the recombinant adeno-associated virus serotype rh10 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype rh10 vector that are covered by the claims of the Licensed Patents set forth on Exhibit A-4 (or other Licensed Patents relating thereto described in Section 1.19(b) or 1.22(b), as applicable).

1.5 "AAV Materials" means AAV Vectors, and any materials that are made or used for the sole purpose of making AAV Vectors, in each case, which, in the absence of the license granted

pursuant to Section 2.2, would infringe or is covered by at least one Valid Claim of the applicable Licensed Research Patents in the country of manufacture or use.

1.6 "AAV Vectors" means, collectively, AAV7, AAV8, AAV9, and AAVrh10.

1.7 "Affiliate" means any legal entity directly or indirectly, during the Term, controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity. An entity may be or become an Affiliate of an entity and may cease to be an Affiliate of an entity, in each case, during the Term.

1.8 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.9 "Commercial Field" means (a) the treatment of Friedreich's Ataxia (Systemic) in human beings by *in vivo* gene therapy with AAVrh10; and (b) if and when a Commercial Option is exercised for a Disease Indication by Licensee under Section 2.3, the treatment of such Disease Indication in human beings by in vivo gene therapy with the Specified Vector selected for such Disease Indication.

1.10 "Commercial Option" has the meaning set forth in Section 2.3.

1.11 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.7 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.11.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.11.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.11.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.11.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.11.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.12 "Disclosing Party" has the meaning set forth in Section 5.1.

1.13 "Disease Indication(s)" means Friedreich's Ataxia (CNS) and Friedreich's Ataxia (Systemic).

1.14 "Domain Antibody" ****.

1.15 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.16 "Friedreich's Ataxia (CNS)" means Friedreich's Ataxia that is treated by administration of the applicable AAV Vector directly to the central nervous system (brain and spinal cord).

1.17 "Friedreich's Ataxia (Systemic)" means Friedreich's Ataxia that is treated by administration of the applicable AAV Vector by any route except administration directly to the central nervous system (brain and spinal cord).

1.18 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.19 "Licensed Commercial Patents" means, on a Specified Vector-by-Specified Vector basis, to the extent they cover such Specified Vector, (a) all United States patents and patent applications listed in Exhibit A-1 (if the Specified Vector is AAV7), Exhibit A-2 (if the Specified Vector is AAV8), Exhibit A-3 (if the Specified Vector is AAV9), or Exhibit A-4 (if the Specified Vector is AAVrh10), including patents arising from such patent applications; and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Commercial Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.20 "Licensed Patents" means the Licensed Commercial Patents or Licensed Research Patents, as applicable.

1.21 "Licensed Product" means (a) any product using the applicable Specified Vector that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at

least one Valid Claim of the Licensed Commercial Patents in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe or is covered by at least one Valid Claim of the Licensed Commercial Patents in the country of manufacture, use, sale, offer for sale, or import; or (b) any service sold by Licensee, its Affiliates, and any of its or their Sublicensees with respect to the administration of any product using the applicable Specified Vector to patients that, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim of the Licensed Commercial Patents in the country of sale.

1.22 "Licensed Research Patents" means (a) all United States patents and patent applications listed in Exhibit A-1 (in the case of AAV7), Exhibit A-2 (in the case of AAV8), Exhibit A-3 (in the case of AAV9), and Exhibit A-4 (in the case of AAVrh10), in each case, including patents arising from such patent applications; and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Research Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.23 "Manufacturing Technology" means any and all patents, patent applications, know-how, and all intellectual property rights associated therewith that are owned or controlled by Licensor, and including all tangible embodiments thereof, that are necessary or useful for the manufacture of adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, formulations, quality control data, specifications, scale up, any and all improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided to regulatory authorities and the information contained therein are deemed Manufacturing Technology.

1.24 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.25 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****

****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.26 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.27 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.28 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences.

1.29 "Receiving Party" has the meaning set forth in Section 5.1.

1.30 "ReGenX Licensors" means SmithKline Beecham Corporation (or any successor thereto under the GSK Agreement) and The Trustees of the University of Pennsylvania (or any successor thereto under the Penn Agreement).

1.31 "Research Field" means Licensee's internal research and pre-clinical development for the treatment of either Disease Indication in humans by in vivo gene therapy using AAV Materials (excluding AAVrh10 for Friedreich's Ataxia (Systemic)). "Research Field" specifically excludes (without limitation) (a) all human clinical trial use, diagnostic use, therapeutic use, and prophylactic use, and (b) any commercial uses.

1.32 "Research Term" means the following:

(a) with respect to Friedreich's Ataxia (Systemic), a period beginning with the Effective Date and ending on the earlier of (i) **** and (ii) ****; and

(b) with respect to Friedreich's Ataxia (CNS), a period beginning with the Effective Date and ending on the earlier of (i) **** and (ii) the **** of the Effective Date.

1.33 "Retained Rights" has the meaning set forth in Section 2.4.

1.34 "Specified Vector" means the following:

(a) with respect to Friedreich's Ataxia (Systemic), (i) AAVrhl0 and (ii) if a Commercial Option is exercised with respect to Friedreich's Ataxia (Systemic), the AAV Vector that is selected by Licensee pursuant to Section 2.3, and

(b) with respect to Friedreich's Ataxia (CNS), if a Commercial Option is exercised with respect to Friedreich's Ataxia (CNS), the AAV Vector that is selected by Licensee pursuant to Section 2.3.

The Specified Vectors and applicable Disease Indication will be set forth on Exhibit B (to be amended as of the applicable Grant Date as provided in Section 2.3).

1.35 "Sublicensee" means (i) any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement; and (ii) any other Third Party or Affiliate to whom a sublicensee described in clause (i) has granted a further sublicense as permitted by this Agreement.

1.36 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.37 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANTS

2.1 Exclusive License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.6 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the Licensed Commercial Patents to make, have made, use, import, sell, and offer for sale Licensed Products using AAVrh10 solely in the Commercial Field of Friedreich's Ataxia (Systemic), including, for the avoidance of doubt, the right to conduct research and development.

2.2 Research License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, during the Research Term, Licensor hereby grants to Licensee a non-exclusive, sublicensable (as provided in Section 2.6 only), non-transferable (except as provided in Section 10.2), worldwide license under the Licensed Research Patents to make, have made, and use AAV Materials in the Research Field (including, for the avoidance of doubt, the right to conduct research and pre-clinical development) solely for purposes of selecting Specified Vector(s) for use in the Commercial Field upon exercise of a Commercial Option. For the

avoidance of doubt, the foregoing license in this Section 2.2 does not include the right to sell, offer for sale, or import any AAV Materials.

2.3 Commercial License Option. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee the option, exercisable at Licensee's sole discretion, to obtain a non-exclusive worldwide license with respect to each of the Disease Indications and a single Specified Vector for such Disease Indication (each such right with respect to a particular Disease Indication, a "Commercial Option") in accordance with the following provisions:

2.3.1 Method of Exercise. To exercise the Commercial Option for a particular Disease Indication, Licensee must provide written notice to Licensor prior to the end of the applicable Research Term, which written notice must specify the Disease Indication and Specified Vector (as further described in Section 2.3.2) with respect to which Licensee desires to exercise its Commercial Option. For Friedreich's Ataxia (CNS), such written notice must be accompanied by a wire transfer of the commercial option fee set forth in Section 3.2.

2.3.2 Specified Vector. For purposes of selecting a Specified Vector for use with a Disease Indication, the Specified Vector must be ****. Upon Licensor's receipt of the notice and, if applicable, fee described in Section 2.3.1, Exhibit B will be amended to set forth the Specified Vector for each Disease Indication with respect to which a Commercial Option is exercised.

2.3.3 License Grant Upon Exercise. If Licensee exercises the Commercial Option for a particular Disease Indication, effective upon Licensor's receipt of the notice and, if applicable, fee described in Section 2.3.1 (the "Grant Date" for such Disease Indication with respect to the applicable Specified Vector), subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor shall be deemed to have granted to Licensee a non-exclusive, sublicensable (as provided in Section 2.6 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the applicable Licensed Commercial Patents to make, have made, use, import, sell, and offer for sale Licensed Products using the Specified Vector solely in the Commercial Field of such Disease Indication, including, for the avoidance of doubt, the right to conduct research and development.

2.3.4 Disease Indications. For the avoidance of doubt, the foregoing license granted pursuant to Section 2.3.3 will be deemed granted on the Grant Date on a Disease Indication-by-Disease Indication basis, solely with respect to the Commercial Field associated with the Disease Indication for which the Commercial Option was exercised under this Section 2.3 and solely with respect to Licensed Products using the Specified Vector selected for the particular Disease Indication. The Parties acknowledge that there may be different Grant Dates for each Disease Indication, depending on when and if Licensee exercises the Commercial Option for a particular Disease Indication. As set forth above, Licensee, at its sole discretion, may exercise the Commercial Option with respect to either or both of the two Disease Indications. If Licensee exercises the Commercial Option with respect to only one of the Disease Indications but not both, the Commercial Option will terminate with respect to the unexercised Disease Indication at the end of the applicable Research Term (together with the license granted under Section 2.2), and Licensee will have no further rights under this Agreement with respect to Friedreich's Ataxia

(CNS) if it is the unexercised Disease Indication or with respect to Friedreich's Ataxia (Systemic) with respect to any Specified Vector (other than AAVrhl0) if it is the unexercised Disease Indication; provided that the termination of a Commercial Option with respect to Friedreich's Ataxia (Systemic) will not affect Licensee's rights under this Agreement with respect to the license granted under Section 2.1.

2.4 Retained Rights. Except for the rights and licenses specified in Sections 2.1, 2.2, and, if applicable, 2.3.3, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise and whether such intellectual property is subordinate, dominant, or otherwise useful for the practice of the Licensed Patents. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes inside or outside of the Commercial Field (other than to the extent of the exclusive license under Section 2.1) or the Research Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees to the following rights retained by Licensor and the ReGenX Licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Commercial Field or Research Field:

2.4.1 The rights and licenses granted in Sections 2.1, 2.2, and, if applicable, 2.3.3 shall not include any right (and Licensor and the ReGenX Licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including any Specified Vector.

2.4.2 Licensor and the ReGenX Licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including any Specified Vector; and

(b) A non-exclusive right for the ReGenX Licensors (which right is sublicensable by such licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.4.3 The rights and licenses granted in Sections 2.1, 2.2 and, if applicable, 2.3.3 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, the foregoing retained right does not give Licensor (i) the right to conduct

clinical trials in humans in the Commercial Field for Friedreich's Ataxia (Systemic) using AAVrh10 or (ii) the exclusive right to conduct clinical trials in humans in any other Commercial Field with respect to which a Commercial Option has been exercised, though Licensor retains the non-exclusive right to do so; or

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Sections 2.1 and, if applicable, 2.3.3 does include the right to provide the service of the administration of Licensed Products to patients.

2.4.4 Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Commercial Field for Friedreich's Ataxia (Systemic) using AAVrhl0 or any rights to sell products using AAVrhl0 in the Commercial Field for Friedreich's Ataxia (Systemic).

2.4.5 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational and research purposes.

2.5 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grants hereunder are expressly subject to all applicable United States government rights, including any applicable requirement that products that result from such intellectual property and are sold in the United States must be substantially manufactured in the United States.

2.6 Sublicensing.

2.6.1 The research license granted pursuant to Section 2.2 is not sublicensable by Licensee, except to its Affiliates; provided that any such sublicense to an Affiliate must comply with the provisions of this Section 2.6 (including Section 2.6.2). The license granted pursuant to Sections 2.1 and, if applicable, 2.3.3 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.6 (including Section 2.6.2).

2.6.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee; ****. Licensor must receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive an unredacted copy of the sublicense written in the English language for Licensor's records and to share with the ReGenX Licensors.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.7 Improvements.

2.7.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) for any and all purposes, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that, during the term of this Agreement, Licensor shall have no right, under the license in this Section 2.7.1(b), to practice the Licensed Back Improvements with respect to AAVrh10 in the Commercial Field of Friedreich's Ataxia (Systemic).

2.7.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any of its Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents, which modification or improvement is developed by Licensee or any of its Affiliate during the term of this Agreement or by any Sublicensee during the term of any sublicense agreement with such Sublicensee.

2.7.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement.

2.8 Covenants Regarding In-Licenses. During the term of this Agreement, without the prior written consent of Licensee, which consent shall not be unreasonably withheld, Licensor agrees not to exercise its right to terminate and will not amend either the GSK Agreement or Penn Agreement if such termination or amendment would materially, adversely affect Licensee's rights under this Agreement with respect to the Licensed Patents.

2.9 Section 365(n) of the Bankruptcy Code. All rights and licenses granted to Licensee or Licensor under or pursuant to this Agreement are and will otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code (Title 11, U.S. Code), as amended (the "Bankruptcy Code") or any comparable law outside the United States, licenses of rights to "intellectual property" as defined in Section 101(35A) of the Bankruptcy Code. The Parties will retain and may fully exercise all of their respective rights and elections under the Bankruptcy Code and any comparable law outside the United States.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor an initial fee of $600,000, of which ****will be paid upon the Effective Date and **** will be paid upon the earlier of (a) December 31, 2014 and (b) the closing of a transaction (or series of transactions) involving the issuance or sale of equity securities of Licensee pursuant to which Licensee receives proceeds of not less than US ****; provided that such **** portion of the initial fee will be immediately payable upon any termination of this Agreement prior to the earlier of those events.

3.2 Commercial Option Fee. If Licensee exercises the Commercial Option granted to Licensee under Section 2.3 with respect to Friedreich's Ataxia (CNS), Licensee shall pay Licensor a fee of $300,000. For clarity, no such fee will be required with respect to exercising the Commercial Option with respect to Friedreich's Ataxia (Systemic).

3.3 Annual Maintenance Fee. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor on-going annual maintenance fees no later than **** after each anniversary of the Effective Date. The annual maintenance fees will be as follows:

(a) **** for Friedreich's Ataxia (Systemic) and

(b) if the Commercial Option with respect to Friedreich's Ataxia (CNS) is exercised, then, following such exercise, **** for Friedreich's Ataxia (CNS).

3.4 Milestone Fees. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor the following milestone payments on a per-Disease Indication basis for the first Licensed Product to achieve such milestone event:

3.4.1 Friedreich's Ataxia (Systemic) Milestones.

Friedreich's Ataxia (Systemic) Milestone	**Milestone Payment**

Friedreich's Ataxia (Systemic) Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (i.e., first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. NDA submission in the United States	****
4. NDA submission in the European Union or the rest of the world (excluding the United States)	****
5. NDA approval in the United States	****
6. NDA approval in the European Union or the rest of the world (excluding the United States)	****
Total (per such Disease Indication):	$ 8,850,000

3.4.2 Friedreich's Ataxia (CNS).

Friedreich's Ataxia (CNS) Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (*i.e.*, first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. NDA submission in the United States	****
4. NDA submission in the European Union or the rest of the world (excluding the United States)	****
5. NDA approval in the United States	****
6. NDA approval in the European Union or the rest of the world (excluding the United States)	****
Total (per such Disease Indication):	$5,000,000

3.4.3 For clarity, the milestone payments set forth in Section 3.4.1 are payable **** with respect to Friedreich's Ataxia (Systemic), and the milestone payments set forth in Section 3.4.2 are payable **** with respect to Friedreich's Ataxia (CNS), in each case, with respect to the **** Licensed Product for such Disease Indication that achieves the milestone event, ****. To the extent that either of the two development milestones in Section 3.4.1 or 3.4.2 (*i.e.*, first treatment of human subject in a clinical trial or first treatment in Phase 3 Clinical Trial in the applicable Disease Indication) has not been paid at the time of achievement of either NDA submission milestone within the same Disease Indication, then, upon the achievement of either of such NDA submission milestones, the preceding unpaid development milestone payments within such Disease Indication shall be made in addition to the payment corresponding to the NDA submission milestone that has been achieved.

3.5 Royalties.

3.5.1 In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay to Licensor the following royalties based upon the annual Net Sales worldwide of all Licensed Products in a given calendar year, subject to the reductions in royalty rates set forth in Section 3.5.2:

Cumulative Annual Net Sales of all Licensed Products Worldwide	**Royalty Percentage**
Portion of Net Sales less than $300,000,000	****
Portion of Net Sales between (and including) $300,000,000 through (and including) $600,000,0000	****
Portion of Net Sales greater than $600,000,000	****

3.5.2 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

$$R = (C * (A / (A+B)))$$

Where
R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****

Example Calculation:

Assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R= (**** – ****)* (**** / (**** + ****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** – **** = ****%

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.5.3 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.5 on the Net Sales of Licensed Products in a given country will end on a Licensed Product-by-Licensed Product and country-by-country basis when ****

****. For clarity, only one royalty, determined in accordance with this Section 3.5, is payable on the Net Sales of any unit of a Licensed Product.

3.6 Sublicense Fees.

3.6.1 In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee will pay Licensor a percentage of any sublicense fees (****) received by Licensee or its Affiliates for the Licensed Commercial Patents from any Third Party Sublicensee or from any Third Party granted any option to obtain a sublicense. The applicable percentage due to Licensor for each sublicense (or option) in the Commercial Field of Friedreich's Ataxia (CNS) shall be **** The applicable percentage due to Licensor for each sublicense (or option) in the Commercial Field of Friedreich's Ataxia (Systemic) shall be as follows:

Friedreich's Ataxia (Systemic)

Event	Sublicense Fee Rate
If sublicensed (or optioned) on or before ****	****
If sublicensed (or optioned) on or before ****	****
If sublicensed (or optioned) on or before ****	****
If sublicensed (or optioned) after ****	****

For the avoidance of doubt, with respect to an option to obtain a sublicense in the Commercial Field of Friedreich's Ataxia (Systemic), if a sublicense is later granted as a result of the exercise of such option, the sublicense fees applicable to such sublicense will be determined by reference to ****.

3.6.2 With respect to the obligations under this Section 3.6, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement or payment, in either case, of Licensee's actual costs for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the research, development and/or manufacturing of Licensed Products pursuant to a specific agreement;

(b) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value or in the form of loans at commercially reasonable rates of interest; and

(c) Any and all amounts paid to Licensee or its Affiliates by a Third Party Sublicensee as royalties on sales of Licensed Product sold by such Sublicensee under a sublicense agreement.

3.6.3 If Licensee or its Affiliate receives sublicense fees from Third Party Sublicensees or from any Third Party granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.6 (a) in the form of the non-cash consideration received by Licensee or its Affiliates or (b) a cash payment determined based on the fair market value of such non-cash consideration. If Licensee or its Affiliate enters into any sublicense that is not an arm's length transaction, fees due under this Section 3.6 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined jointly and in good faith by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

3.6.4 To the extent Licensee or its Affiliates receives payment from a Third Party relating to one or more of the milestone events set forth in the tables in Section 3.4, then the amount of the payment made to Licensor under such Section 3.4 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.6; instead, the amounts due under this Section 3.6 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.6.1 above to the sublicense fees received by Licensee or its Affiliates from such Third Party after deducting the amount of the payment under Section 3.4.

3.7 Reports and Records.

3.7.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.25, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.5.2;

(f) Royalties owed to Licensor, listed by category; and

(g) The computations for any applicable currency conversions.

3.7.2 Licensee shall pay the royalties due under Section 3.5 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.7.1.

3.7.3 Within **** after the occurrence of a milestone event described in Section 3.4, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.4.

3.7.4 Within **** after the receipt of any fees from any Third Party as described in Section 3.6, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.6.

3.7.5 All financial reports under this Section 3.7 will be certified by the chief financial officer of Licensee.

3.7.6 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or the ReGenX Licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of five years thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and the ReGenX Licensors and their respective accountants in connection with the review or audit.

3.8 Currency, Interest.

3.8.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.8.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.7.

3.8.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.9 Taxes and Withholding.

3.9.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.9.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.9.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products for Friedreich's Ataxia (Systemic) in the Commercial Field. Furthermore, if Licensee exercises the Commercial Option granted to Licensee under Section 2.3 with respect to Friedreich's Ataxia (CNS), Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products for Friedreich's Ataxia (CNS) in the Commercial Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****. Without limiting the foregoing, Licensee will meet the following:

(a) acceptance by the FDA of an Investigational New Drug application, or acceptance by the European Medicines Agency (or any successor entity thereto) of an equivalent application, for a Licensed Product using AAVrh10 for Friedreich's Ataxia (Systemic) by no later than **** after the Effective Date; and

(b) if Licensee exercises the Commercial Option granted to Licensee under Section 2.3 with respect to Friedreich's Ataxia (Systemic), acceptance by the FDA of an Investigational New Drug application, or acceptance by the European Medicines Agency (or any successor entity thereto) of an equivalent application, for a Licensed Product using the Specified Vector selected in the exercise of such Commercial Option for Friedreich's Ataxia (Systemic) by no later than **** after the Grant Date;

provided, however, that, if Licensee expects not to achieve one of the milestones set forth in clause (a) or (b) on or before the specified deadline in such clause (a) or (b), Licensee may pay Licensor an extension fee of **** on or before such deadline and the relevant deadline in clause (a) or (b), as applicable, shall then be extended by an additional ****. Licensee will only be entitled to **** for **** of the milestones in clauses (a) and (b), ****.

4.2 Reporting. Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and good faith, but non-binding, projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with the ReGenX Licensors.

4.4 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.7.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties agree they will release a joint press release in the form attached hereto as Exhibit C. Except as provided in Section 5.2.2, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or Specified Vector program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee, but Licensor will provide Licensee with a copy of any such publications or referrals two business days prior to release.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose the other's Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and

notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder, with the ReGenX Licensors to the extent required by the GSK Agreement and Penn Agreement. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, then, to the extent legally permitted, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will, at the Disclosing Party's request and expense, provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Commercial Patents to expire, lapse, or become abandoned or unenforceable in all the countries of the world.

6.2 Termination for Failure to Exercise Option. This Agreement will terminate automatically with respect to Friedreich's Ataxia (CNS) at the end of the Research Term for Friedreich's Ataxia (CNS) if Licensee does not exercise the Commercial Option for Friedreich's Ataxia (CNS) in accordance with Section 2.3. This Agreement will terminate automatically with respect to Friedreich's Ataxia (Systemic) with respect to any Specified Vector (other than AAVrh10) at the end of the Research Term for Friedreich's Ataxia (Systemic) if Licensee does not exercise the Commercial Option for Friedreich's Ataxia (Systemic) in accordance with Section 2.3; provided that such termination will not affect Licensee's rights under this Agreement with respect to the license granted under Section 2.1.

6.3 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason, with or without cause; provided that, if such termination notice is sent prior to the first anniversary of the Effective Date, such termination notice shall be accompanied by Licensee's payment of **** in satisfaction of the remainder of the initial fee under Section 3.1. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to one or more of the Disease Indications within the Research Field or Commercial Field, as applicable.

6.4 Termination for Breach.

6.4.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4.3 If the allegedly breaching Party disputes in good faith the allegation of breach or non-cure prior to the expiration of the applicable cure period, this Agreement shall not be terminated until such dispute is resolved in favor of the non-breaching Party in accordance with Section 10.6, and the breaching Party has not cured such material breach within an additional ****, or such payment breach within an additional ****, after such resolution; provided that Licensor shall be entitled to terminate this Agreement at the end of the original **** or ****, as applicable, cure period, without waiting for resolution of the dispute in accordance with Section 10.6 if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6.5 Termination for Insolvency.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee, any of its Affiliates, or any Sublicensees experiences any Trigger Event.

6.5.2 For purposes of this Section 6.5, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.5.2(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.5.2(b) through (d) above.

6.6 Patent Challenge.

6.6.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee, any of its Affiliates, or any Sublicensee of a Patent Challenge.

6.6.2 For purposes of this Section 6.6, "Patent Challenge" means any action against Licensor, The Trustees of the University of Pennsylvania, or the ReGenX Licensors, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.7 Effects of Termination. The effect of termination pursuant to Section 6.2, by Licensee pursuant to Section 6.3, by either Party, as applicable, under Section 6.4, or by Licensor pursuant to Section 6.5 or 6.6 shall be as follows:

6.7.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.7.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all AAV Materials or Licensed Products and shall cease to otherwise practice the Licensed Patents; provided that Licensee, its Affiliates, and Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.7.2 At Licensor's request, Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate;

6.7.3 If termination is by Licensee pursuant to Section 6.3 or by Licensor pursuant to Section 6.4, 6.5, or 6.6, Licensee shall grant, and hereby grants to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee or any Affiliates (during the term of this Agreement) or by any Sublicensees (during the term of any sublicense agreement with such Sublicensee) to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.7.4 Licensee shall pay all monies then-owed to Licensor under this Agreement;

6.7.5 Each Receiving Party shall, at the Disclosing Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations; and

6.7.6 If termination is only with respect to a particular Disease Indication within the Research Field or the Commercial Field, but not all Disease Indications, then the provisions of this Section 6.7 shall only apply with respect to the terminated Disease Indications, and this Agreement shall continue with respect to the non-terminated Disease Indications.

6.8 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.4, (Retained Rights), Section 2.5 (Government Rights), Section 2.7 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.7 (Reports and Records), Section 4.3 (Confidential Information), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, but subject to any obligations of Licensor to the ReGenX Licensors, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention.

7.2.2 As between Licensor and Licensee, but subject to any obligations of Licensor to the ReGenX Licensors, Licensor shall have the sole right, but not the obligation, to prosecute any such infringement at its **** recovered in connection therewith. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action. Nothing in this Agreement obligates Licensor to bring or prosecute lawsuits against Third Parties for infringement of any Licensed Patents.

7.2.3 Licensee shall have no right to undertake prosecution of any such infringement.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another. To the extent Licensor takes any action, Licensor (or the ReGenX Licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or the ReGenX Licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION

8.1 Representations and Warranties by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the licenses specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Research Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.1.4 To Licensor's knowledge, (a) the Licensed Research Patents are solely owned by the Trustees of the University of Pennsylvania, and (b) no Third Party (other than the ReGenX Licensors) has any right, interest, or claim in or to such Licensed Research Patents with respect to the Disease Indications that are inconsistent with those granted to Licensee with respect to the Disease Indications;

8.1.5 To Licensor's knowledge, no Third Party is infringing any of the Licensed Research Patents in a manner that is inconsistent with the scope of rights granted to Licensee with respect to the Disease Indications; and

8.1.6 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Research Patents with respect to the Disease Indications.

8.2 Representations and Warranties by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the licenses granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, AAV MATERIALS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS, AAV MATERIALS, OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR OR EITHER OF THE REGENX LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS, AAV MATERIALS, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF AAV MATERIALS OR LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, the ReGenX Licensors, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party

liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties to the extent that such claims result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims to the extent based on (1) any breach by Licensor of the representations, warranties, or obligations of Licensor under this Agreement or (2) the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, but subject to clauses (1) and (2) above, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that was **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents, AAV Materials, or Licensed Products, including any claim by or on behalf of a ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties to the extent that such claims result from or arise out of: ****; provided, however, that Licensor shall not be liable for claims to the extent based on (1) any breach by Licensee of the representations, warranties, or obligations of Licensee under this Agreement or (2) the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on any indemnified party (an "Indemnified Party") without the Indemnified Party's prior written consent or, if Licensee is the Indemnifying Party, that imposes any restrictions or obligations on Licensor's direct or indirect licensors or grants any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. Upon the Indemnifying Party's reasonable request, the Indemnified Parties will reasonably cooperate with the Indemnifying Party in the defense and settlement of any such claim, at the Indemnifying Party's cost and expense. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of $3,000,000 combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage

within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement, subject to Section 5.3.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor; provided, however, that Licensee may assign this Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets of Licensee to which the Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with written notice of such assignment at least five business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. In addition, Licensee will provide Licensor with written notice of any change of control (*i.e.*, the acquisition by a person or group of "control" of Licensee, as defined in Section 1.7) of Licensee at least five business days prior to the effectiveness of such change of control. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

AAVLife
183/189 avenue de Choisy
75013 Paris
France
Attn: Amber Salzman
Telephone: 610-659-1098
Facsimile: [_____]

with a copy to:

WilmerHale
60 State Street
Boston, MA 02109
USA
Attn: Belinda M. Juran, Esq.
Telephone: 617-526-6987
Facsimile: 617-526-5000

Either Party may change its official address upon written notice to the other Party in accordance with this Section 10.4.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than 30 days following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of

biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee and its Affiliates, and Licensee shall use reasonable efforts to require that any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement dated January 9, 2014 between the Parties. All "Confidential Information" (as defined in such Mutual Non-Disclosure Agreement) disclosed by one Party to the other Party pursuant to such Mutual Non-Disclosure Agreement shall be deemed "Confidential Information" of such disclosing Party under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.11). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Commercial Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by the arbitrators or a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other reasonable acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections, Articles, and exhibits in this Agreement are to Sections, Articles, and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC	AAVLIFE
By: /s/ Kenneth Mills	By: /s/ Amber Salzman, PhD
Name: Kenneth Mills	Name: Amber Salzman, PhD
Title: President & CEO	Title: President

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A-1
Licensed Research Patents (AAV7)

Application #	Patent #	Filing Date	Country	Status
	****	****	****	****
****		****	****	****
****		****	****	****
	****	****	****	****
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****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A-2
Licensed Research Patents (AAV8)

Application #	Patent #	Filing Date*	Country	Status
****		****	****	****
****		****	****	****
****		****	****	****
****	****	****	****	****
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****		****	****	****

* International Filing Date, where national stage application or foreign divisional thereof

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A-3
Licensed Research Patents (AAV9)

Application #	Patent #	Filing Date	Country	Status
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
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****	****	****	****	****
****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A-4
Licensed Research Patents (AAVrh10)

Appin #	Title	Inventors	Nos	Docket
****	****	****	****	****

Docket	Country	Appln No	Filing Date	Patent Number	Issue Date	Pubn Number	Pub Date
****	****	****	****	****	****	****	****
****	****	****	****			****	****
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****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Specified Vectors

Specified Vector	Disease Indication
AAVrh10	Friedreich's Ataxia (Systemic)

CONFIDENTIAL TREATMENT REQUESTED

Exhibit C
Press Release

CONFIDENTIAL TREATMENT REQUESTED

REGENX BIOSCIENCES ENTERS INTO LICENSE AGREEMENT WITH AAVLIFE FOR DEVELOPMENT OF TREATMENTS FOR FRIEDREICH'S ATAXIA USING NAV® VECTORS

WASHINGTON, DC and Paris, France April 2014 — REGENX Biosciences, LLC announces that the company has entered into an agreement with AAVLife for the development and commercialization of products to treat Friedreich's ataxia (FA) using NAV technology.

Under the terms of the Agreement, REGENX granted AAVLife an exclusive worldwide license, with rights to sublicense, to deliver REGENX's NAV rAAVrh10 vector via non CNS routes to treat FA in humans. In addition, AAVLife was granted an option to obtain a non-exclusive worldwide license to additional NAV vectors for CNS delivery for the treatment of FA in humans. In return for these rights, REGENX receives payments in the form of up-front and on-going fees, certain milestone fees and royalties on net sales of products incorporating NAV vectors. REGENX would also receive a share of any sublicensing revenues.

"REGENX has been engaged with the team at AAVLife, including its stakeholders like the Friedreich's Ataxia Research Alliance (FARA), since first becoming aware of their gene therapy research results and during the company's process of formation. We are pleased to formally continue our collaboration with a team who has the leadership, expertise, resources, and commitment to patients that is required in order to develop innovative treatments for patients with FA through the application of NAV technology," said Ken Mills, President and CEO of REGENX. "We believe this license agreement will be a key component to the successful development of treatments for patients suffering with FA."

Amber Salzman, Ph.D., Chief Executive Officer and a co-founder of AAVLife, commented: "The right to the REGENX vector is a critical part of our program to advance into clinical trials a gene-therapy approach to treating Friedreich's ataxia."

Jennifer Farmer, Executive Director of FARA, added: "Heart disease accounts for most early deaths due to Friedreich's ataxia. We believe that NAV technology will enable successful clinical studies that are urgently needed for patients with Friedreich's ataxia."

About Friedreich's Ataxia (FA)

Friedreich's ataxia is a rare, degenerative, life-shortening neuro-muscular disorder that affects children and adults, and involves the loss of strength and coordination usually leading to wheelchair use. Other symptoms may include diminished vision, hearing and speech; scoliosis (curvature of the spine); and increased risk of diabetes. Also associated with the disorder is a progressive decline in cardiac function which is the most common cause of death. There are no FDA-approved treatments.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary NAV vector technology platform, for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, and AveXis. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding REGENX, please visit www.regenxbio.com.

About AAVLife

AAVLife, registered in Paris, is a privately held company dedicated to advancing gene therapy for rare diseases. Further information is available at www.aavlife.com.

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Contact:
REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista @regenxbio.com

CONFIDENTIAL TREATMENT REQUESTED

EXECUTION VERSION

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of July 9th, 2013 ("Effective Date") by and between ReGenX Biosciences, LLC (formerly known as ReGenX, LLC), a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), and Audentes Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at ****, San Francisco, California, 94115 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to adeno-associated virus serotype 8 and 9; and

WHEREAS, Licensee desires to obtain an exclusive license under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV8" means (a) the recombinant adeno-associated virus serotype 8 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 8 vector that are covered by the claims of the Licensed AAV8 Patents.

1.2 "AAV9" means (a) recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector that are covered by the claims of the Licensed AAV9 Patents.

1.3 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.4 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.5 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this

Agreement will be deemed the Confidential Information of both Parties and (ii) the records and reports referred to Section 3.6 of this Agreement will be deemed the Confidential Information of Licensee, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.5.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.5.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.5.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.5.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.5.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.6 "Disclosing Party" has the meaning set forth in Section 5.1.

1.7 "Domain Antibody" ****.

1.8 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.9 "Field" means, collectively, the XLMTM Field and the Pompe Field.

1.10 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.11 "Licensed AAV8 Patents" means (a) all United States patents and patent applications listed in part 1 of Exhibit A and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications.

1.12 "Licensed AAV9 Patents" means (a) all United States patents and patent applications listed in part 2 of Exhibit A and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications.

1.13 "Licensed Patents" means, collectively, (a) the Licensed AAV8 Patents and the Licensed AAV9 Patents and (b) any additional claims of patents and patent applications as required pursuant to Section 8.1.5.

1.14 "Licensed Product" means (a) any AAV8 or AAV9 product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service with respect to the administration of AAV8 or AAV9 to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe at least one Valid Claim of the Licensed Patents in the country of sale.

1.15 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.16 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.17 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.18 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.19 "Pompe Field" means the treatment of Pompe Disease (GAA deficiency) in humans by in vivo gene therapy in humans using AAV8 or AAV9.

1.20 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents.

1.21 "Receiving Party" has the meaning set forth in Section 5.1.

1.22 "Retained Rights" has the meaning set forth in Section 2.2.

1.23 "Sublicensee" means any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement.

1.24 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.25 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

1.26 "XLMTM Field" means the treatment of X-linked myotubular myopathy (XLMTM) in humans by in vivo gene therapy in humans using AAV8 or AAV9.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Patents to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field, including, for the avoidance of doubt, the right to conduct research and development (including by conducting clinical trials in humans and/or animal studies).

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1 or as provided in Section 8.1.5, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise, whether any such intellectual property dominates or is dominated by the Licensed Patents. Notwithstanding anything to the contrary this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes outside of the Field. Without limiting the foregoing, Licensee acknowledges and agrees to the following rights retained by Licensor and its direct and indirect licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Field:

2.2.1 Notwithstanding anything in this Agreement to the contrary, the rights and licenses granted in Section 2.1 shall not include any right (and Licensor and its direct and indirect licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including AAV8 and/or AAV9.

2.2.2 Notwithstanding anything in this Agreement to the contrary, Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including AAV8 and/or AAV9; and

(b) A non-exclusive right for Licensor's direct and indirect licensors (which right is sublicensable by such licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 Notwithstanding anything in this Agreement to the contrary, the rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, or import research reagents, including any viral vector construct (provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field); and

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Section 2.1 does include the right to provide the service of the administration of Licensed Products to patients.

2.2.4 Notwithstanding anything in this Agreement to the contrary, Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such development rights

granted by Licensor shall not include the right to conduct clinical trials in humans in the Field or any rights to sell products in the Field.

2.2.5 Notwithstanding anything to the contrary in this Agreement, the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational, research, and other non-commercial purposes.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.4 Sublicensing.

2.4.1 The license granted pursuant to Section 2.1 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses **** pursuant to a written sublicense agreement with the Sublicensee. Licensor must receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with Licensor's direct and indirect licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or any of its direct or indirect licensors') ability to ensure compliance with this Agreement; provided that, if any of Licensor's direct or indirect licensors require a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.5 Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license (a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights and (b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with AAV8 and AAV9 outside the Field, including the right to research, develop, make, have made, use, offer for sale, and sell products and services outside the Field. For purposes of this Agreement, "Licensed Back Improvements" means any **** by Licensee, any Affiliates ****, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.5.2 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement by Licensor or its direct or indirect licensors or licensees.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor an initial fee of $600,000 upon the Effective Date. One-half of the amount paid by Licensee to Licensor under this Section 3.1 may be paid by Licensee in the form of shares of Licensee's common stock, which will be issued in accordance with Section 3.8.

3.2 Annual Maintenance Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor on-going annual maintenance fees of **** on each anniversary of the Effective Date.

3.3 Milestone Fees. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor the following milestone payments on a per-Licensed Product basis:

XLMTM Field Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (*i.e.*, first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (i.e., first patient, first dose)	****

XLMTM Field Milestone	Milestone Payment
3. NDA submission in the United States	****
4. NDA submission in the European Union	****
5. NDA approval in the United States	****
6. NDA approval in the European Union	****
Total:	8.85 million

Pompe Field Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (*i.e.*, first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. NDA submission in the United States	****
4. NDA submission in the European Union	****
5. NDA approval in the United States	****
6. NDA approval in the European Union	****
Total:	$ 8.85 million

3.3.1 At Licensee's option, up to **** of the amount paid by Licensee to Licensor under the first milestones (i.e., first treatment of human study) for each of the XLMTM Field and the Pompe Field may be paid by Licensee in the form of shares of Licensee's common stock, which will be issued in accordance with Section 3.8.

3.3.2 For clarity, the milestone payments set forth in this Section 3.3 are payable **** in the XLMTM Field and once in the Pompe Field with respect to each Licensed Product that achieves the milestone event, regardless of whether the milestone is achieved by Licensee or any Sublicensee. To the extent that either of the two development milestones in this Section 3.3 with respect to a particular field (i.e., first treatment of human subject in a clinical trial or first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of either NDA submission milestone for that field, then, upon the achievement of either of such NDA submission milestones, the preceding unpaid development milestone payments with respect to that field shall be made in addition to the payment corresponding to the NDA submission milestone that has been achieved.

3.4 Royalties. In further consideration of the license granted to Licensee under Section 2.1, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products in the XLMTM Field or the Pompe Field, as applicable, subject to the reductions in royalty rates set forth in Section 3.4.1:

Cumulative Annual Net Sales of all Licensed Products in the XLMTM Field Worldwide	Royalty Percentage for XLMTM Field

Portion of Net Sales less than $300 million	****
Portion of Net Sales between (and including) $300 million through (and including) $600 million	****
Portion of Net Sales greater than $600 million	****

Cumulative Annual Net Sales of all Licensed Products in the Pompe Field Worldwide	**Royalty Percentage for Pompe Field**
Portion of Net Sales less than $300 million	****
Portion of Net Sales between (and including) $300 million through (and including) $600 Million	****
Portion of Net Sales greater than $600 million	****

3.4.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C * (A / (A+B)))

Where
R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****.

Example Calculation:

assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R = (**** - ****)* (**** / (**** + ****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** — **** = ****%

Notwithstanding the foregoing, Licensee will pay to Licensor no less than ****% of the royalties that Licensee would otherwise pay to Licensor if there were no royalties due to Third Parties.

3.4.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end on a country-by-country basis when ****.

3.5 Sublicense Fees.

3.5.1 In further consideration of the license granted to Licensee under Section 2.1, Licensee will pay Licensor a percentage of any sublicense fees (including upfront payments and milestone payments) received by Licensee for the Licensed Patents from any Sublicensee or from any person or entity granted any option to obtain a sublicense. The applicable percentage due to Licensor for each sublicense (or option) shall be as follows:

Event	Sublicense Fee Rate
If sublicensed (or optioned) on or before the first anniversary of the Effective Date	****
If sublicensed (or optioned) on or before the third anniversary of the Effective Date but after the first anniversary of the Effective Date	****
If sublicensed (or optioned) on or before the fourth anniversary of the Effective Date but after the third anniversary of the Effective Date	****
If sublicensed (or optioned) after the fourth anniversary of the Effective Date	****

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee corresponding directly to the development of Licensed Products pursuant to a specific agreement;

(b) Consideration received for the purchase of an equity interest in Licensee at fair market value or in the form of loans at commercially reasonable rates of interest; and

(c) Any and all amounts paid to Licensee by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement.

3.5.3 To the extent Licensee receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.3, then the amount of the payment made to Licensor under such Section 3.3 with respect to such milestone event shall be not be deemed sublicense fees under this Section 3.5; instead, the amounts due under this Section 3.5 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.5.1 above

to the sublicense fees received by Licensee from such Third Party after deducting the amount of the payment under Section 3.3.

3.6 Reports and Records.

3.6.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

3.6.1.1 Number of Licensed Products included within Net Sales, listed by country;

3.6.1.2 Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

3.6.1.3 Qualifying costs to be excluded from the gross consideration, as described in Section 1.16, listed by category of cost;

3.6.1.4 Net Sales of Licensed Products listed by country;

3.6.1.5 A detailed accounting of any royalty reductions applied pursuant to Section 3.4.1;

3.6.1.6 Royalties owed to Licensor, listed by category; and

3.6.1.7 The computations for any applicable currency conversions.

3.6.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.6.1.

3.6.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3. In addition, within **** after the receipt of sublicense fees from any Sublicensee as described in Section 3.5, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.5.

3.6.4 All financial reports under this Section 3.6 will be certified by the chief financial officer of Licensee.

3.6.5 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or its direct or indirect licensors (and their respective accountants) with access to all of the relevant books, records, and related background information as reasonably required to confirm the accuracy of the royalties,

fees, and payments paid to Licensor under this Agreement. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and or its direct or indirect licensors and accountants in connection with the review or audit.

3.7 Currency, Interest.

3.7.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.7.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal*, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.6.

3.7.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.8 Issuance of Common Stock. If Licensee elects to pay any amounts under Section 3.1 or 3.3.1 by the issuance of shares of Licensee's common stock, then the provisions of this Section 3.8 will apply.

3.8.1 Each share of Licensee's common stock will be valued at **** per share as of the Effective Date (the "Price Per Share") and shall be issued pursuant to the terms of the Common Stock Purchase Agreement in the form attached hereto as Exhibit C (the "Stock Purchase Agreement"). If Licensee at any time or from time to time after the Effective Date effects a subdivision, split, or combination of Licensee's outstanding common stock into a greater or lesser number of shares, then, in each such event, the Price Per Share in effect immediately prior to such subdivision, split, or combination will be increased or decreased proportionately. Licensee will provide Licensor with written notice of any such subdivision, split, or combination and the resulting Price Per Share.

3.8.2 The number of shares to be issued to Licensor will be determined by taking the amount of the payment owed under Section 3.1 or 3.3.1, as applicable, and dividing it by the Price Per Share, as calculated pursuant to Section 3.8.1. Licensee will deliver to Licensor, by no later than the date the payment (with respect to which Licensee will fulfill by the issuance of shares of Licensee's common stock) is due, (a) a copy of the Stock Purchase Agreement (executed by both Licensor and Licensee), (b) a stock certificate in the name of Licensee for the number of shares of common stock to be issued, and (c) in connection with any issuance

pursuant to Section 3.3.1, a certificate signed by an officer of Licensee attesting that Licensee's representations and warranties contained in Sections 8.2.1 (with respect to Licensee's ability to issue the common stock), 8.2.4 (with respect to Licensee's ability to issue the common stock), and 8.2.5 are true and correct as of the date of issuance of such stock with the same effect as though made on and as of such date.

ARTICLE 4: DILIGENCE

4.1 Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in each of the XLMTM Field and the Pompe Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****. Without limiting the foregoing, Licensee will meet the following:

(a) Acceptance by the FDA of an Investigational New Drug application for a Licensed Product in the XLMTM Field by no later than ****; and

(b) Acceptance by the FDA of an Investigational New Drug application for a Licensed Product in the Pompe Field by no later than ****.

Licensee will notify Licensor in writing as soon as Licensee believes in good faith that Licensee will not be able to achieve either milestone set forth in Section 4.1(a) or (b) by the relevant deadline date, and, upon the payment to Licensor of **** within **** of the original deadline date, the deadline date for such milestone set forth in Section 4.1(a) or (b), as applicable, will be extended for **** from the original deadline date; provided that Licensee will only be entitled to **** for the XLMTM Field and **** for the Pompe Field, each of which extensions will require a payment of **** as provided in this Section 4.1.

4.2 Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its direct and indirect licensors.

4.4 Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.2.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties agree they will release a joint press release in the form attached hereto as Exhibit B. Except as provided in Section 5.2.1, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or AAV8 or AAV9 program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with any of Licensor's direct and indirect licensors of the Licensed Patents. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, or become abandoned or unenforceable in all countries of the world.

6.2 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason, with or without cause. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to either the Pompe Field or the XLMTM Field.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches

6.3.3 This Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period; provided that, if termination is by Licensor as a result of Licensee's materially breaching Article 4, and if such breach only relates to either the Pompe Field or XLMTM Field, but not both, then Licensor's termination right shall only be with respect to the Pompe Field or XLMTM Field, as applicable, with respect to which the breach related and not both. Notwithstanding the above, if Licensee disputes in good faith that such material breach exists, and gives Licensor written notice of such dispute within **** following Licensee's receipt of Licensor's notice of default, then, Licensor may not terminate this Agreement until the dispute is resolved in accordance with Section 10.6; provided that Licensor shall be entitled to terminate this Agreement at the end of the original **** cure period, without waiting for resolution of the dispute in accordance with Section 10.6, if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee or any of its Affiliates experiences any Trigger Event.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives Licensor's licensor a right of termination under the Penn Agreement and such licensor provides written notice of such termination to Licensor, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) ****, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, such appointment is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings

described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives Licensor's licensor a right of termination under the Penn Agreement and such licensor provides written notice of such termination to Licensor, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee commences a Patent Challenge.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor, the University of Pennsylvania, or any direct or indirect licensor of Licensor (including an action for declaratory judgment) to declare, or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The licenses granted by Licensor hereunder shall terminate, and Licensee and its Affiliates shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Patents; provided that Licensee shall have the right to continue to sell its existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.6.2 All sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee shall be assigned to Licensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5, Licensee shall grant, and hereby grants to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates (excluding any such modifications or improvements developed by a Third Party that acquired Licensee or its Affiliates, whether by merger, acquisition or assets sale, prior to the date of such acquisition), or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.6.4 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.6.5 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

If termination is only with respect to either the Pompe Field or the XLMTM Field, but not both, then the provisions of this Section 6.6 shall only apply with respect to the terminated Field, and this Agreement shall continue with respect to the non-terminated Field.

6.7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2, (Retained Rights), 2.3 (Government Rights), 2.5 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.6 (Reports and Records), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that the University of Pennsylvania controls Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees the rights and obligations under this Section 7.1 are subject to the rights of Licensor's direct and indirect licensors with respect to the Licensed Patents and Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under its agreements with its direct and indirect licensors.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 As between Licensor and Licensee, Licensor shall have the first right, but not the obligation, to prosecute any such infringement at its own expense. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action.

7.2.3 If Licensor elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by any such License Patent and such Licensed Patent is being infringed by another product in the Field (such infringement, the "Competitive Infringement"), Licensee shall have the second right, but not the obligation, to prosecute such Competitive Infringement with respect to such other product in the Field, at Licensee's own expense. In any such action to enforce any of the Licensed Patents, Licensor, at the request and expense of Licensee, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensee is unable to initiate or prosecute such action solely in its own name, Licensor shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such Competitive Infringement, Licensee (a) shall not take any actions that would be detrimental to the Licensed Patents and Licensor's rights with respect thereto outside the Field and (b) shall not settle any such Competitive Infringement without the prior consent of Licensor.

7.2.4 Any recovery of damages by Licensor for any infringement other than a Competitive Infringement shall be ****. Any recovery of damages by the Party undertaking enforcement or defense of a suit for Competitive Infringement shall be applied, as between Licensor and Licensee but subject to the obligations to Licensor's direct and indirect licensors, first to reimburse each such Party for costs and expenses (including reasonable attorneys' fees and costs) incurred by such Party in connection with such suit, and the balance remaining, if any, from any such recovery shall be ****.

7.2.5 Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.2 are subject to the rights of Licensor's direct and indirect licensors of the Licensed Patents (including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Licensor's obligations under this Agreement only apply to the

extent that Licensor has any rights with respect to enforcing the Licensed Patents under its agreements with its direct and indirect licensors. Furthermore, Licensee acknowledges the following:

7.2.5.1 All monies recovered upon the final judgment or settlement of any action with respect to Competitive Infringement will also need to be allocated to Licensor's direct and indirect licensors (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

7.2.5.2 Licensor's direct and indirect licensors retain the continuing right to intervene at their own expense and join Licensor or Licensee in any claim or suit for infringement of the Licensed Patents.

7.2.5.3 In any infringement prosecuted by Licensors' direct and indirect licensors, all financial recoveries will be ****.

7.2.5.4 In any infringement prosecuted by Licensor's direct and indirect licensors, Licensee agrees, at the request and expense of such licensors, to cooperate to the fullest extent reasonably possible, to the same extent as though Licensor were prosecuting such suit (as provided in this Section 7.2, including Section 7.2.2).

7.2.5.5 The written consent of Licensor's direct and indirect licensors will be required (a) for any decision that would have a materially adverse affect on the validity, scope of patent claims, or enforceability of the Patent Rights and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on any of its direct or indirect licensors, or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringements by another. To the extent Licensor takes any action, Licensor (or its direct or indirect licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or any of its direct or indirect licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensor's knowledge, threatened against Licensor relating to the Licensed Patents that would impact activities under this Agreement;

8.1.4 To Licensor's knowledge, (a) the Licensed Patents are solely owned by the University of Pennsylvania, and (b) no Third Party (other than Licensor's direct and indirect licensors) has any right, interest, or claim in or to such Licensed Patents in the Field that are inconsistent with those granted to Licensee herein;

8.1.5 To Licensor's knowledge, Licensor does not Control as of the Effective Date any patent or patent application (other than the Licensed Patents (as defined in Section 1.13(a)) that would necessarily be infringed by the use or sale of AAV8 or AAV9 in the Field. If it is determined, in accordance with the procedure of this Section 8.1.5, that Licensor Controls as of the Effective Date a patent or patent application (other than the Licensed Patents) that would necessarily be infringed by the use or sale of AAV8 or AAV9 in the Field, then Licensee's sole remedy shall be the inclusion of the applicable patent or patent application as a "Licensed Patent" hereunder but solely to the extent of the claim(s) that would necessarily be infringed by the use or sale of AAV8 or AAV9. At any time during the term of this Agreement, Licensee may notify Licensor in writing of any such patent or patent application that Licensee believes should be included as a "Licensed Patent" pursuant to this Section 8.1.5. Such written notice shall identify the relevant patent or patent application and relevant claim(s) and shall explain briefly why Licensee, in good faith, believes it should be included as a "Licensed Patent." Licensor has **** following Licensor's receipt of Licensee's written notice to dispute the inclusion of such patent or patent application or the scope of the remedy; in which event, such dispute will be resolved in accordance with Section 10.6. Upon the Parties' agreement (or a resolution, in favor of Licensee, of the dispute pursuant to Section 10.6), the applicable claim(s) of the applicable patent or patent application will be deemed a "Licensed Patent" hereunder. For the avoidance of doubt, Licensor makes no representation or warranty under this Section 8.1.5 as to any claim of a patent or patent application covering the manufacture of AAV8 or AAV9, and Licensee acknowledges that manufacturing claims of any patents or patent applications will not be added as "Licensed Patents" pursuant to the procedure set forth in this Section 8.1.5. For the purpose of this Section 8.1.5, "Control" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent or patent application on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any Third Party;

8.1.6 To Licensor's knowledge, no Third Party is infringing any of the Licensed Patents in the Field; and

8.1.7 Licensor has not received any written notice from any Third Party patentee alleging infringement of, and to Licensor's knowledge Licensor has not been sued for patent infringement of, Third Party technology by the practice of the Licensed Patents in the Field.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement, to grant the rights granted by it hereunder, and to issue Licensee's common stock to Licensor in accordance with this Agreement;

8.2.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement;

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement; and

8.2.5 Licensee's common stock, when issued and delivered in accordance with the terms of Article 3, (a) will be duly and validly authorized and issued, fully paid and non-assessable, and free from all taxes, liens, and charges created by Licensee in respect of the issuance thereof, (b) will be issued in compliance with all applicable federal and state securities laws, and (c) will be free of transfer restrictions (other than the transfer restrictions imposed by any federal or state securities laws and liens or encumbrances created by or imposed by Licensor).

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR OR ANY OF LICENSOR'S DIRECT OR INDIRECT LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS,

LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its direct and indirect licensors of the Licensed Patents, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that results from or arises out of: ****; provided, however, that Licensee shall not be liable for claims based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

8.4.1.1 any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

8.4.1.2 any claim by a Third Party that the ****; and

8.4.1.3 **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents or Licensed Products, including any claim by or ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that results from or arises out of: ****; provided, however, that Licensor shall not be liable for claims based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (a "Indemnifying Party" , shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on any indemnified party (a "Indemnified Party") without the other Party's prior written consent or, if Licensee is the Indemnifying Party, that grants any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the

adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Upon Licensor's written request, each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor; provided, however, that Licensee may assign this Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with notice of such assignment at least 5 business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
50 17th Street, NW
Suite 1100
Washington, DC 20006
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Audentes Therapeutics, Inc.
****
San Francisco, California, ****
Attn: Matthew Patterson, President & CEO
Telephone: 646-712-1001
Email: mpatterson@audentestx.com

with a copy to:

Fenwick and West, LLP.
1191 Second Avenue, 10th Floor
Seattle, WA 98101
Attn: Effie Toshav

Telephone: 206.389.4510
Facsimile: 206-389-4511

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of Delaware with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent

irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement between the Parties dated December 3, 2012. All "Confidential Information" disclosed by the Parties pursuant to such Mutual Non-Disclosure Agreement shall be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.5). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections and exhibits in this Agreement are to Sections and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President & CEO

AUDENTES THERAPEUTICS, INC.

By: /s/ Matthew Patterson
Name: Matthew Patterson
Title: President & CEO

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A

Licensed Patents

Part 1, Licensed AAV8 Patents

Application #	Patent #	Filing Date*	Country	Status
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
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****	****	****	****	****

* International Filing Date, where national stage application or foreign divisional thereof

Part 2, Licensed AAV9 Patents

Application #	Patent #	Filing Date	Country	Status
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Press Release

REGENX Biosciences and Audentes Therapeutics Enter into Exclusive License Agreement for Development of Treatments for Serious, Rare Muscle Diseases Using NAV™ Vectors

WASHINGTON & SAN FRANCISCO—(BUSINESS WIRE)—REGENX Biosciences, LLC and Audentes Therapeutics, Inc. announce that they have entered into an agreement for the development and commercialization of products to treat X-Linked Myotubular Myopathy (XLMTM) and Pompe disease using **NAV** vectors.

Under the terms of the Agreement, REGENX granted Audentes an exclusive worldwide license, with rights to sublicense, to REGENX's **NAV** rAAV8 and rAAV9 vectors for treatment of XLMTM and Pompe disease in humans. In return for these rights, REGENX receives an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAV8 and rAAV9.

"We believe this exclusive license agreement is important to the successful development of NAV-based gene delivery treatments for patients with XLMTM and Pompe disease," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to be cooperating with the team at Audentes in its pursuit of developing innovative treatments for patients with serious, rare muscle diseases through the application of **NAV** technology. REGENX has a continued interest to provide commercial partners that evidence outstanding leadership, expertise, resources and a strong commitment to patients, such as Audentes, with access to our **NAV** technology."

"Audentes is committed to the development of new treatments for patients with XLMTM and Pompe disease using AAV gene therapy technology and we feel rAAV8 and rAAV9 are the most promising vectors to achieve this goal," said Matthew R. Patterson, President and CEO of Audentes. "We are very pleased to enter into this agreement with REGENX, which we believe offers us the best path to expeditiously develop novel therapies for patients."

About X-Linked Myotubular Myopathy (XLMTM)

X-Linked Myotubular Myopathy (XLMTM) is a rare, inherited disorder characterized by severe muscle weakness and respiratory impairment. It is caused by mutations in the MTM1 gene, which encodes an enzyme called myotubularin. Myotubularin is thought to be involved in the development and maintenance of muscle cells. XLMTM affects approximately 1 in 50,000 newborn males worldwide.

About Pompe Disease

Pompe Disease is a rare, inherited disorder characterized by progressive muscle weakness and respiratory impairment. It is caused by mutations in a gene that encodes an enzyme called acid alpha-glucosidase (GAA), which is needed by the body to break down glycogen — a stored form of sugar used for energy. Pompe Disease affects approximately 1 in every 40,000 births.

About REGENX Biosciences

REGENX Biosciences is leading the effort to translate promising gene delivery applications into a pipeline of next generation personalized therapies for a range of severe diseases with serious unmet needs. We believe that the **NAV** technology to which we have exclusive rights represents the potential promise of curing the root cause of disease rather than the symptoms, and we are committed to establishing best in class standards for our **NAV** vectors. Our intent is to initially develop treatments for a number of rare, genetic diseases including hypercholesterolemias, the mucopolysaccharidoses, and retinitis pigmentosa and ensure continuing access for our **NAV** technology through innovative partnerships, license opportunities and the expansion of our growing team of global collaborators. REGENX holds exclusive rights to a portfolio of over 100 patents and patent applications pertaining to its **NAV** technology and related applications.

For more information regarding REGENX, please visit www regenxbio.com.

About Audentes Therapeutics, Inc.

Audentes™ is a biotechnology company committed to the development and commercialization of innovative new treatments for people with serious, rare muscle diseases through the application of adeno-associated virus (AAV) gene therapy technology. The company consists of a focused, experienced, and passionate team driven by the goal of improving the lives of patients. Audentes takes pride in strong, global relationships with the patient, research, and medical communities.

For more information regarding Audentes, please visit www.audentestx corn.

Contacts

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio com
or
Audentes Therapeutics, Inc.
Matthew Patterson, 646-712-1001
mpatterson@audentestx com

CONFIDENTIAL TREATMENT REQUESTED

EXHIBIT C

****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

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CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of March 21, 2014 ("Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), and AveXis, Inc. (formerly known as BioLife Cell Bank, Inc.), a corporation organized under the laws of the State of Delaware, with offices at 4925 Greenville Avenue, Suite 604, Dallas, TX 75206 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to adeno-associated virus serotype 9; and

WHEREAS, Licensee desires to obtain an exclusive license under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV9" means (a) the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype 9 vector that are covered by the claims of the Licensed Patents.

1.2 "Affiliate" means any legal entity directly or indirectly, during the term of this Agreement, controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity. An entity may be or become an Affiliate of an entity and may cease to be an Affiliate of an entity, in each case, during the term of this Agreement.

1.3 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.4 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.6 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the

provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.4.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.4.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.4.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.4.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.4.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.5 "Disclosing Party" has the meaning set forth in Section 5.1.

1.6 "Domain Antibody" ****.

1.7 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.8 "Field" means the treatment of spinal muscular atrophy in humans by in vivo gene therapy using AAV9.

1.9 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.10 "Licensed Patents" means, to the extent they cover AAV9, (a) all United States patents and patent applications listed in Exhibit A, and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications.

1.11 "Licensed Product" means (a) any AAV9 product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured

by a process that would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service sold by Licensee, its Affiliates, and any of its or their Sublicensees with respect to the administration of any AAV9 product to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of sale.

1.12 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.13 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.14 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.15 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.16 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences.

1.17 "Receiving Party" has the meaning set forth in Section 5.1.

1.18 "ReGenX Licensors" means SmithKline Beecham Corporation (or any successor thereto under the GSK Agreement) and The Trustees of the University of Pennsylvania (or any successor thereto under the Penn Agreement).

1.19 "Retained Rights" has the meaning set forth in Section 2.2.

1.20 "Sublicensee" means (i) any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement; and (ii) any other Third Party or Affiliate to whom a sublicensee described in clause (i) has granted a further sublicense as permitted by this Agreement.

1.21 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.22 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Patents to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field, including, for the avoidance of doubt, the right to conduct research and development.

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise and whether such intellectual property is subordinate, dominant, or otherwise useful for the practice of the Licensed Patents. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes outside of the Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees that the following rights are retained by Licensor and the ReGenX Licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Field:

2.2.1 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor and the ReGenX Licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including AAV9.

2.2.2 Licensor and the ReGenX Licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including AAV9; and

(b) A non-exclusive right for the ReGenX Licensors (which right is sublicensable by the ReGenX Licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such ReGenX Licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field; or

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Section 2.1 does include the right to provide the service of the administration of Licensed Products to patients.

2.2.4 Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Field or any rights to sell products in the Field.

2.2.5 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational and research purposes.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.4 Sublicensing.

2.4.1 The license granted pursuant to Section 2.1 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee; ****. Licensor must receive written notice as soon as practicable following execution of any such sublicenses. Any further sublicenses granted by any Sublicensees (to the extent permitted hereunder) must comply with the provisions of this Section 2.4 (including Section 2.4.2) to the same extent as if Licensee granted such sublicense directly.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with the ReGenX Licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or the ReGenX Licensors') ability to ensure compliance with this Agreement; provided that, if either of the ReGenX Licensors requires a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.5 Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with AAV9, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that Licensor shall have no right, under the license in this Section 2.5.1(b), to practice the Licensed Back Improvements in the Field.

2.5.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.5.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor an initial fee of $2,000,000, which shall be payable as follows: (i)**** upon the Effective Date, (ii) **** within **** after the Effective Date, and (iii) **** within **** after the Effective Date; provided that any unpaid portion of the initial fee will be immediately payable upon any termination of this Agreement.

3.2 Annual Maintenance Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor on-going annual maintenance fees of **** on each anniversary of the Effective Date.

3.3 Milestone Fees. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor the following milestone payments:

Milestone	**Milestone Payment**
1. First treatment of the **** human subject in a clinical trial (*i.e.*, **** patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. First NDA submission for a Licensed Product in the United States	****
4. First NDA submission for a Licensed Product in the European Union	****
5. First NDA approval for a Licensed Product in the United States	****

6. First NDA approval for a Licensed Product in the European Union	****
Total:	$12,250,000.00

For clarity, the milestone payments set forth in this Section 3.3 are payable **** with respect to each milestone event, ****.

3.4 Royalties. In further consideration of the license granted to Licensee under Section 2.1, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.4.1:

Cumulative Annual Net Sales of all Licensed Products Worldwide	**Royalty Percentage**
Portion of Net Sales in a calendar year less than ****	****
Portion of Net Sales in a calendar year between (and including) **** through (and including) ****	****
Portion of Net Sales in a calendar year greater than ****	****

By way of example only, if Licensee receives $700,000,000 in cumulative Net Sales of all Licensed Products in a calendar year, then the royalties payable by Licensee to Licensor under this Section 3.4 during such calendar year would be calculated as follows:

= (****)(****) + (****)(****) + (****)(****)

= (****) + (****) + (****)

= ****

3.4.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C * (A / (A+B)))
Where
R = reduction of Licensor royalty,

A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****

Example Calculation:

Assume i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii): projected total royalty = ****

R = (**** – ****) * (**** / (**** + ****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** – **** = **** (but subject to the cap described below)

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.4.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end on a country-by-country basis when ****.

3.5 Sublicense Fees.

3.5.1 In further consideration of the license granted to Licensee under Section 2.1, Licensee will pay Licensor **** of any sublicense fees (****) received by Licensee or its Affiliates from a Third Party for the Licensed Patents from any Sublicensee or from any Third Party granted any option to obtain a sublicense.

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the development of Licensed Products pursuant to a specific agreement;

(b) Any and all amounts paid to Licensee or its Affiliates by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement; and

(c) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value.

3.5.3 If Licensee or its Affiliates receives sublicense fees from Third Party Sublicensees or from any Third Party granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.5 (a) in the form of the non-cash consideration received by Licensee or its Affiliates or (b) a cash payment determined based on the fair market value of such non-cash consideration. If Licensee or its Affiliate enters into any sublicense that is not an arm's length transaction, fees due under this Section 3.5 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

3.5.4 To the extent Licensee receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.3, then the amount of the payment made to Licensor under such Section 3.3 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.5; instead, the amounts due under this Section 3.5 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.5.1 above to the sublicense fees received by Licensee from such Third Party after deducting the amount of the payment under Section 3.3.

3.6 Reports and Records.

3.6.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.13, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.4.1;

(f) Royalties owed to Licensor; and

(g) The computations for any applicable currency conversions.

3.6.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.6.1.

3.6.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3.

3.6.4 All financial reports under this Section 3.6 will be certified by the chief financial officer of Licensee or Licensee's qualified financial representative.

3.6.5 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or the ReGenX Licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of five years thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and the ReGenX Licensors and their respective accountants in connection with the review or audit. If the review or audit determines that Licensee has overpaid any payment, then Licensor shall refund the overpayment to Licensee.

3.7 Currency, Interest.

3.7.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.7.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.6.

3.7.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.8 Taxes and Withholding.

3.8.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in

Section 3.8.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.8.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****.

4.2 Reporting. Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with the ReGenX Licensors.

4.4 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) protect the Confidential Information of the Disclosing Party with at least the same degree of care as it protects its own confidential and proprietary information, and in any event with not less than a reasonable degree of care; (c) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (d) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties agree they will release a joint press release in the form attached hereto as Exhibit B. Except as provided in Section 5.2.2, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results

related to Licensee's Licensed Product or AAV9 program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with the ReGenX Licensors. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, or become abandoned or unenforceable in all countries of the world.

6.2 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason, with or without cause; provided that, if such termination notice is sent prior to payment in full of the initial fee under Section 3.1, such termination notice shall be accompanied by Licensee's payment of all unpaid amounts in satisfaction of the remainder of the initial fee under Section 3.1.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay

Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee or any of its Affiliates experiences any Trigger Event.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives a ReGenX Licensor a right of termination under the Penn Agreement or GSK Agreement, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee, any of its Affiliates, or any Sublicensee of a Patent Challenge.

6.5.2 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor or the ReGenX Licensors, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.6.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Patents; provided that Licensee shall have the right to continue to sell its existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.6.2 If termination is by Licensor pursuant to Section 6.3, 6.4, or 6.5, then, at Licensor's request, Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate. If termination is for any other reason, then all sublicenses shall terminate;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5, Licensee shall grant, and hereby grants, to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.6.4 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.6.5 Each Receiving Party shall, at the Disclosing Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2, (Retained Rights), 2.3 (Government Rights), 2.5 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.6 (Reports

and Records), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that The Trustees of the University of Pennsylvania control Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of the ReGenX Licensors set forth in the GSK Agreement and Penn Agreement with respect to the Licensed Patents, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under the GSK Agreement and the Penn Agreement.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention. However, Licensee is under no obligation to search for potential infringers. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 As between Licensor and Licensee, Licensor shall have the first right, but not the obligation, to prosecute any such infringement ****. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action.

7.2.3 If Licensor elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by any such Licensed Patent and such Licensed Patent

is being infringed by another product in the Field (such infringement, the "Competitive Infringement"), Licensee shall have the second right, but not the obligation, to prosecute such Competitive Infringement with respect to such other product in the Field, at Licensee's own expense. In any such action to enforce any of the Licensed Patents, Licensor, at the request and expense of Licensee, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensee is unable to initiate or prosecute such action solely in its own name, Licensor shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such Competitive Infringement, Licensee (a) shall not take any actions that would be detrimental to the Licensed Patents and Licensor's rights with respect thereto outside the Field and (b) shall not settle any such Competitive Infringement without the prior consent of Licensor.

7.2.4 The Party not controlling the action under this Section 7.2 shall be entitled to independent counsel in such proceedings but at its own expense, not subject to reimbursement by the other Party and not subject to any offset against any damages received by the Party bringing suit under Section 7.2.5. The controlling Party shall keep the cooperating party reasonably informed of the progress of the action proceedings.

7.2.5 Any recovery of damages by Licensor for any infringement other than a Competitive Infringement shall be ****. Any recovery of damages by the Party undertaking enforcement or defense of a suit for Competitive Infringement shall be applied, as between Licensor and Licensee but subject to the obligations to the ReGenX Licensors set forth in the GSK Agreement and the Penn Agreement, as follows: (a) first to reimburse each such Party for costs and expenses (including reasonable attorneys' fees and costs) incurred by such Party in connection with such suit, and (b) the balance remaining, if any, from any such recovery shall be ****.

7.2.6 Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.2 are subject to the rights of the ReGenX Licensors under the GSK Agreement and Penn Agreement (including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Licensor's obligations under this Agreement only apply to the extent that Licensor has any rights with respect to enforcing the Licensed Patents under the GSK Agreement and the Penn Agreement. Furthermore, Licensee acknowledges the following:

7.2.6.1 All monies recovered upon the final judgment or settlement of any action with respect to Competitive Infringement will also need to be allocated to the ReGenX Licensors (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

7.2.6.2 The ReGenX Licensors retain the continuing right to intervene at their own expense and join Licensor or Licensee in any claim or suit for infringement of the Licensed Patents.

7.2.6.3 In any infringement prosecuted by the ReGenX Licensors, all financial recoveries will be ****.

7.2.6.4 In any infringement prosecuted by the ReGenX Licensors, Licensee agrees, at the request and expense of such licensors, to cooperate to the fullest extent reasonably possible, to the same extent as though Licensor were prosecuting such suit (as provided in this Section 7.2, including Section 7.2.2).

7.2.6.5 The written consent of the ReGenX Licensors will be required (a) for any decision that would have a materially adverse affect on the validity, scope of patent claims, or enforceability of the Patent Rights and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on either of the ReGenX Licensors, or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringements by another. To the extent Licensor takes any action, Licensor (or the ReGenX Licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, which shall not be unreasonably denied, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or either of the ReGenX Licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Representations and Warranties by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.1.4 To Licensor's knowledge, (a) the Licensed Patents are solely owned by The Trustees of the University of Pennsylvania, and (b) no Third Party (other than the ReGenX Licensors) has any right, interest, or claim in or to such Licensed Patents in the Field that are inconsistent with those granted to Licensee in the Field under this Agreement; and

8.1.5 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Patents in the Field.

8.2 Representations and Warranties by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, TITLE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR AND THE REGENX LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, EXEMPLARY, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, the ReGenX Licensors, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims to the extent based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents or Licensed Products, including any claim by or on behalf of a ****.

8.4.2 Indemnification Procedure. Licensee, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on Licensor, the ReGenX Licensors, or any indemnified party (an "Indemnified Party") without Licensor's prior written consent or that grants any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any

such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

9.1 Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written

consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable.

9.2 Licensor and all of its employees and agents must not use Licensee's name, seal, logo, trademark, or service mark (or any adaptation thereof) in any way without the prior written consent of Licensee; provided, however that Licensor may acknowledge the existence and general nature of this Agreement, subject to Section 5.2 or 5.3, as applicable, and refer to Licensee as a licensee of Licensor.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor. Such prohibition on assignment of this Agreement shall apply even with respect to a sale or merger of Licensee, the transfer of substantially all of Licensee's business assets, or the sale of a majority of the capital stock of Licensee. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438

Facsimile: 202-785-7439 Facsimile: 202-785-7439

If for Licensee:

AveXis, Inc.
4925 Greenville Avenue, Suite 604
Dallas, TX 75206
Attn: Chief Executive Officer
Telephone: 972-725-7797
Facsimile: 516-619-0412

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior

approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement, dated February 6, 2014, between Licensor and Licensee and that certain Mutual Non-Disclosure Agreement, dated March 29, 2013, between Licensor and Licensee (who was then known as BioLife Cell Bank, Inc.). All "Confidential Information" disclosed by the Parties pursuant to such Confidential Disclosure Agreement shall be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.4). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections, Articles, and exhibits in this Agreement are to Sections, Articles, and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has

been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC		AVEXIS, INC.	
By:	/s/ Kenneth Mills	By:	/s/ John A. Carbona
Name:	Kenneth Mills	Name:	John A. Carbona
Title:	President & CEO	Title:	CEO

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A

Licensed Patents

Application #	Patent #	Filing Date	Country	Status
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B

Press Release

CONFIDENTIAL TREATMENT REQUESTED

DRAFT SUBJECT TO FINAL REVIEW AND APPROVAL

REGENX BIOSCIENCES AND AVEXIS ENTER INTO LICENSE AGREEMENT FOR DEVELOPMENT OF TREATMENTS FOR SPINAL MUSCULAR ATROPHY USING NAV® rAAV9 VECTORS

Washington, DC and Dallas, TX — REGENX Biosciences, LLC (REGENX) and AveXis, Inc. (AveXis) announce that they have entered into an agreement for the development and commercialization of products to treat Spinal Muscular Atrophy (SMA) using NAV rAAV9 vectors.

Under the terms of the agreement, REGENX granted AveXis an exclusive worldwide license, with rights to sublicense, to REG ENX's NAV rAAV9 vector for treatment of SMA disease in humans. In return for these rights, REGENX receives an up-front payment, certain milestone fees and royalties on net sales of products incorporating NAV rAAV9.

"We believe this exclusive license agreement is important to the successful development of NAV-based gene delivery treatments for patients with SMA," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to be formally collaborating with AveXis which has assembled a world class team of scientific and clinical experts in SMA, led by Brian Kaspar, Ph.D. and his colleagues at Nationwide Children's Hospital and The Ohio State University, who have demonstrated tremendous dedication to the development of innovative gene therapy treatments for patients with SMA."

"AveXis is committed to the development of new treatments for patients with SMA using NAV-vector technology and we feel rAAV9 is the most promising vector to achieve this goal, something we like to call our 'special snowflake'. We believe the unique properties of rAAV9 will allow us to effectively develop novel treatments, and is at the center of research being done at the Kaspar Laboratory in Columbus, Ohio," said John A. Carbona, CEO of AveXis. "Everyone associated with our SMA program is very pleased to establish this agreement withREGENX, which provides an important foundation for our team to continue to develop novel therapies for patients with all types of SMA."

CONFIDENTIAL TREATMENT REQUESTED

DRAFT SUBJECT TO FINAL REVIEW AND APPROVAL

About Spinal Muscular Atrophy

Spinal muscular atrophy (SMA) is an autosomal-recessive genetic disorder characterized by progressive weakness of the lower motor neurons. SMA is caused by a genetic defect in the SMN1 gene which codes SMN, a protein necessary for survival of motor neurons. SMA kills more infants than any other genetic disease in today's world.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary NAV vector technology platform, for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Audentes Therapeutics, Lysogene, and Esteve. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases. For more information regarding REGENX, please visit www.regenxbio.com.

About AveXis

Based in Dallas, Texas, AveXis is a clinic-ready synthetic biology platform company establishing unique industry alliances to create innovative treatments for people with unmet medical needs. Spinal muscular atrophy is the company's first focus.

For more information regarding AveXis, please visit www.avexisinc.com.

Contacts:

REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com

CONFIDENTIAL TREATMENT REQUESTED

DRAFT SUBJECT TO FINAL REVIEW AND APPROVAL

AveXis

Corporate Contact:

John A. Carbona, Chief Executive Officer
972-725-7797 or jcPavexisinc.com

Media Contact:

Jillian Bowman, Administrative Specialist
972-725-7797 or iillianb@avexisinc.com

CONFIDENTIAL TREATMENT REQUESTED

AGREEMENT

This AGREEMENT ("Agreement") is entered into as of November 22, 2010 ("Effective Date") by and between ReGenX Biosciences, LLC (formerly known as ReGenX, LLC), a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), Chatham Therapeutics, LLC, a North Carolina limited liability company with offices at 45 Chatham Parkway, Chapel Hill, NC 27517 ("Licensee"), and, for purposes of Article 10, Asklepios Biopharmaceutical, Inc., a North Carolina corporation with offices at 45 Chatham Parkway, Chapel Hill, NC 27517 ("Guarantor"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has exclusive rights under certain Licensed Patents (as defined herein) pertaining to recombinant adeno-associated virus vector serotype 8; and

WHEREAS, Licensee is an Affiliate (as defined below) of Guarantor; and

WHEREAS, Licensee desires to obtain a non-exclusive research right under the Licensed Patents to conduct certain research with an option to obtain an exclusive license from Licensor in a specified field under the Licensed Patents;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV8 Materials" means materials that are made, made for (except by Licensor) or used by Licensee, its Affiliates, and any of its or their sublicensees, the manufacture or use of which, in the absence of the license to be granted pursuant to Section 2.1 hereof, would infringe at least one Valid Claim in the country of manufacture or use, including materials manufactured by a process that would infringe at least one Valid Claim in the country of such manufacture.

1.2 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.3 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.4 "Commercial Field" means (i) a Therapeutic as described in Exhibit B (as such Exhibit B may be modified pursuant to Section 2.2.3) (collectively, the "Licensee Therapeutic") for treatment of Hemophilia A disorder in human being, or (ii) the treatment of Hemophilia A disorder in human beings.

1.5 "Commercial Option" has the meaning set forth in Section 2.2.

1.6 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, and other proprietary ideas, whether or not patentable or copyrightable, of either Party (a) that is identified as confidential or proprietary at the time of disclosure; or (b) whose confidential or proprietary status would be reasonably apparent under the circumstances. The Parties acknowledge that the terms and conditions of this Agreement shall be deemed the Confidential Information of both Parties. Notwithstanding the foregoing, Confidential Information shall not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.6.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.6.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.6.3 information that became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement;

1.6.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.6.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.7 "Disclosing Party" has the meaning set forth in Section 5.1.

1.8 "Domain Antibody" ****.

1.9 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.10 "Grant Date" has the meaning set forth in Section 2.2.2.

1.11 "Licensed Patents" means all United States patents and patent applications, re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications listed in Exhibit A that cover recombinant adeno-associated virus serotype 8 vectors. Upon Licensee's reasonable request, from time to time during the term of this Agreement, Licensor will update Exhibit A to reflect updated information with respect to the Licensed Patents.

1.12 "Licensed Product" means (a) any product the manufacture, use, sale, offer for sale, or import of which, in the absence of the license granted pursuant to this Agreement, would infringe

at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service that, in the absence of the licenses granted pursuant to this Agreement, would infringe at least one Valid Claim of the Licensed Patents in the country of sale.

1.13 "Licensee Collaborators" means entities and persons with which Licensee has an active, written research and development, collaboration or funding agreement relating to the Research Field.

1.14 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.15 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product by Licensee and/or its Affiliates and/or its or their sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or its or their sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or its or their sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or its or their sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.16 "Option Expiration Date" has the meaning set forth in Section 2.2.

1.17 "Phase 3 Clinical Trial" means a non-pivotal human clinical trial initiated by or on behalf of Licensee, its Affiliates, or its or their sublicensees in any country in the Territory that would satisfy the requirements of 21 C.F.R. § 312.21(c) or corresponding regulations in jurisdiction outside the United States.

1.18 "Prosecute" means preparation, filing; and prosecuting patent applications and maintaining patents.

1.19 "Receiving Party" has the meaning set forth in Section 5.1.

1.20 "Retained Rights" has the meaning set forth in Section 2.3.

1.21 "Research Field" means Licensee's internal research and pre-clinical development of adeno-associated vectors agents that deliver any DNA, RNA, or other sequence or reagent, other than Domain Antibodies, for the prevention or treatment of Hemophilia A in humans. "Research Field" specifically excludes (without limitation) any human clinical trial use, diagnostic use, therapeutic use, prophylactic use, and commercial use.

1.22 "Selected Commercial Field" has the meaning set forth in Section 2.2.

1.23 "Therapeutic" means a composition that contains a genetic construct encoding either a B-domain deleted Factor VIII and/or other sequences, each as described in Exhibit B.

1.24 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.25 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration or the like) or claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked or deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 Research License Grant. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee a non-exclusive, non-transferable (except as provided in Section 11.2), worldwide right and license, with the right to grant sublicenses only to Licensee Collaborators as provided in this Section 2.1 and subject to Section 2.5, under the Licensed Patents to use the AAV8 Materials in the Research Field and to make AAV8 Materials for use in the Research Field. For the avoidance of doubt, the foregoing license does not include the right to sell, offer for sale, or import AAV8 Materials. The foregoing license is subject to the following:

2.1.1 Only up to a total of **** of Licensee in the aggregate may exercise such rights.

2.1.2 Licensee may extend its rights under Section 2.1 to Licensee's Affiliates and to Licensee Collaborators pursuant to a written sublicense agreement with such Affiliates and Licensee Collaborators; provided that Licensee must comply with Section 2.5 hereof with respect to any such sublicense. If Licensee grants any such sublicense to its Affiliates and Licensee Collaborators, the total **** of Licensee, its Affiliates, and all Licensee Collaborators that are permitted to practice the Licensed Patents as provided in Section 2.1 is **** in the aggregate. Licensee shall establish and maintain an up-to-date list of all Licensee Collaborators who are sublicensed the rights granted pursuant to Section 2.1, which list Licensee shall provide Licensor upon Licensor's request.

2.1.3 The foregoing license rights to make AAV Materials may only be practiced at Licensee's, its Affiliates', or Licensee Collaborators' primary places of business (or any such other location agreed to in advance and in writing by the Parties).

2.1.4 Upon reasonable prior written notice to Licensee, not more than once per calendar year, Licensor shall be entitled to audit Licensee's, its Affiliates', and the Licensee Collaborator's compliance with the terms of the license in this Section 2.1 (including the limitation that not more than **** in the aggregate that may exercise such rights). Licensee shall, and shall cause its Affiliates and its Licensee Collaborators to, permit such audit, including permitting Licensor to review the records of Licensee, its Affiliates, and the Licensee Collaborators reasonably necessary to verify such compliance.

2.1.5 The license granted under this Section 2.1 shall automatically terminate on the Grant Date.

2.2 Commercial License Option. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee the exclusive right, exercisable at Licensee's sole discretion, to elect to obtain an exclusive worldwide license in one of the Commercial Fields but not both Commercial Fields (such right, the "Commercial Option") in accordance with the following provisions.

2.2.1 Method of Exercise. To exercise the Commercial Option, Licensee shall provide written notice to Licensor at any time from and after the Effective Date until the earlier of (i) **** after an IND filing by Licensee (directly or through its Affiliates or a Licensee Collaborator to whom Licensee has sublicensed its rights under Section 2.1) for the first Licensed Product and (ii) the third anniversary of the Effective Date (the earlier of such dates, the "Option Expiration Date"). Such written notice shall specify the Commercial Field with respect to which Licensee is exercising its Commercial Option (such specified field, the "Selected Commercial Field") and shall be accompanied by a wire transfer of the initial fee set forth in Section 3.2.1.1 or 3.2.2.1, as applicable, for the Selected Commercial Field.

2.2.2 License Grant upon Exercise. Effective upon Licensor's receipt of the notice and fee described in Section 2.2.1 above (the "Grant Date"), subject to the terms and conditions of this Agreement, Licensor shall be deemed to have granted Licensee an exclusive (except as provided in Section 2.3), non-transferable (except as provided in Section 11.2), royalty-bearing, worldwide right and license, with the right to grant sublicenses only as provided in Section 2.5, under the Licensed Patents to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Selected Commercial Field.

2.2.3 Amendment to Exhibit B. If Licensee exercises the Commercial Option by specifying the Commercial Field described in Section 1.4(i) as the Selected Commercial Field, simultaneously with such exercise, Licensee may elect to substitute another of Licensee's Therapeutics in place of the Therapeutic currently set forth on Exhibit B by providing Licensor with an amended Exhibit B; provided that the Commercial Field described in Section 1.4(i) shall only apply to a single Therapeutic. If Licensee makes such substitution by providing Licensor with such amended Exhibit B, this Agreement will be deemed to be amended by replacing such amended Exhibit B for the Exhibit B attached hereto as of the Effective Date, and such substituted Therapeutic shall be deemed the Licensee Therapeutic for purposes of Section 1.4 and this Agreement. If no such amended Exhibit B is provided with Licensee's notice to exercise the Commercial Option, Licensee's right to substitute Licensee Therapeutic shall terminate.

2.3 Retained Rights. Except for the rights and licenses specified in Sections 2.1 and 2.2, no other rights are granted. Licensee acknowledges and agrees to the following rights (individually and collectively, the "Retained Rights").

2.3.1 Notwithstanding anything herein to the contrary, the rights and licenses granted in Sections 2.1 and 2.2 shall not include any right under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies which are expressed by an adeno-associated vector.

2.3.2 Furthermore, notwithstanding anything herein to the contrary, Licensor and its licensors retain the following Retained Rights with respect to the Licensed Patents:

2.3.2.1 A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector; and

2.3.2.2 A non-exclusive, sublicensable right to use the Licensed Patents solely for educational, research, development and other non-commercial purposes, including for discovery research efforts with non-profit organizations and including the right to permit non-commercial entities to use the Licensed Patents for educational and research purposes.

2.4 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States. Upon Licensee's reasonable request and at Licensee's sole expense, Licensor shall assist Licensee in Licensee's attempt to obtain a waiver of any such requirement.

2.5 Sublicensing. The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

2.5.1 Licensee may only grant sublicenses pursuant to a written sublicense agreement with such sublicensee, ****. Licensee shall provide Licensor written notice as soon as practicable following execution of such sublicenses.

2.5.2 In each sublicense agreement, Licensee will require the sublicensee to comply with the terms and conditions of this Agreement.

2.5.3 The official language of any sublicense agreement shall be English.

2.5.4 Within **** after Licensee enters into any sublicense, Licensee must send to Licensor a complete copy of the sublicense written in the English language for Licensor's records and to share with Licensor's licensors.

2.5.5 Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is primarily liable to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or sublicensee of Licensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.6 Research Collaboration. In order to help facilitate development and commercialization of Licensed Product, each Party agrees, upon the reasonable request of the other Party, to meet from time to time to discuss the possibility of sharing any relevant and necessary proof of concept, preclinical, clinical, and regulatory documents, data (including animal data and data regarding immunogenicity), and information in either Party's possession or to which either Party has the right to use or access, related to AAV8 Materials as may be useful for the development and commercialization of the Licensed Product; provided that the sharing of such documents, data, and information shall only be on terms and conditions agreed to by the Parties.

ARTICLE 3: CONSIDERATION

3.1 Research Collaboration Fee. In consideration for the research collaboration, Licensee shall pay Licensor a research collaboration fee of $100,000 upon the Effective Date and an on-going annual fee of **** upon each anniversary date of the Effective Date; provided that such annual fees shall terminate on the Grant Date.

3.2 Commercial Milestone Fees. If Licensee exercises the Commercial Option in accordance with Section 2.2, in consideration of the license granted to Licensee under Section 2.2, Licensee shall pay Licensor the following fees:

3.2.1 If the Selected Commercial Field elected by Licensee is the field described in Section 1.4(i), Licensee shall pay:

3.2.1.1 An initial fee of **** by wire transfer in accordance with Section 2.2.1;

3.2.1.2 On-going annual maintenance fees of **** upon each anniversary of the Grant Date; and

3.2.1.3 The following milestone payments on a per-Licensed Product candidate basis:

(a)**** upon the acceptance of a Investigational New Drug application for a Licensed Product;

(b)**** upon initiation (i.e., first patient, first dose) of a Phase 3 Clinical Trial for a Licensed Product;

(c)**** upon the approval of an NDA for the Licensed Product in the United States; and

(d)**** upon the approval of an NDA for the Licensed Product outside the United States;

provided that the Parties acknowledge that the Selected Commercial Field described in Section 1.4(i) will result in only one Licensed Product being developed, manufactured, and commercialized by Licensee, but, if the provisions of Section 3.2.3 apply such that the foregoing milestones would apply to the Selected Commercial Field described in Section 1.4(ii), such Selected Commercial Field may result in more than one Licensed Product being developed, manufactured, and commercialized by Licensee.

3.2.2 If the Selected Commercial Field elected by Licensee is the field described in Section 1.4(ii), Licensee shall pay:

3.2.2.1 An initial fee of $2,000,000 by wire transfer in accordance with Section 2.2.1;

3.2.2.2 On-going annual maintenance fees of **** upon each anniversary of the Grant Date; and

3.2.2.3 The following milestone payments on a per-Licensed Product candidate basis:

(a)**** upon the acceptance of a Investigational New Drug application for a Licensed Product;

(b)**** upon initiation (i.e., first patient, first dose) of a Phase 3 Clinical Trial for a Licensed Product;

(c)**** upon the approval of an NDA for the Licensed Product in the United States; and

(d)**** upon the approval of an NDA for the Licensed Product outside the United States.

3.2.3 Notwithstanding Section 3.2.2 above, if Licensor or any of its Affiliates or its sublicensees files an Investigational New Drug application that is accepted by the applicable regulatory authority for a product that delivers RNA interference or antisense drugs using an adeno-associated virus serotype 8 vector that would compete with Licensed Products, then the fees and milestone payments if the Selected Commercial Field elected by Licensee is the field described in Section 1.4(ii) shall be those set forth in Section 3.2.1 (in place of those set forth in Section 3.2.2).

3.2.4 For purposes of this Section 3.2, acceptance of an Investigational New Drug application shall be deemed to have occurred **** following the filing of an Investigational New Drug application with the FDA or other regulatory authority; provided that, if the FDA or such regulatory authority provides any comments to such submission, such acceptance shall not be deemed to have occurred until such comments have been addressed to the satisfaction of the FDA or regulatory authority.

3.2.5 For clarity, the milestone payments set forth in Sections 3.2.1.3 and 3.2.2.3 are payable **** with respect to each Licensed Product that achieves the milestone event. If development of a particular Licensed Product ceases (a "Failed Product") prior to the approval of an NDA for that Licensed Product and development of another Licensed Product subsequently commences (a "Substitute Product"), then any of the development milestone payments previously made by Licensee (i.e., payments set forth in Sections 3.2.1.3(a) or (b) or Sections 3.2.2.3(a) or (b)) in connection with such Failed Product shall be fully creditable against the repeated achievement of such milestone event by the Substitute Product and shall be deemed to have been paid with respect to such Substitute Product (and will not be deemed to have been paid with respect to such Failed Product). To the extent that any development milestone has not been paid at the time of achievement of an NDA approval milestone, then upon the achievement of such NDA approval milestone all preceding unpaid development milestone payments shall be made in addition to the payment corresponding to the NDA approval milestone that has been achieved.

3.3 Royalties. If Licensee exercises the Commercial Option in accordance with Section 2.2, in further consideration of the license granted to Licensee under Section 2.2, Licensee shall pay to Licensor the following royalty based upon Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.3.1:

Royalty Percentage	Cumulative Annual Net Sales of all Licensed Products Worldwide
****	Up to and including ****;
****	In excess of **** and up to and including ****; and
****	In excess of ****.

3.3.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

$$R = (C * (A / (A+B)))$$

Where
R = Reduction of Licensor royalty,
A = Unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****

Example Calculation:

assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R = (**** – ****)* (**** / (**** + ****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** – **** = ****%

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor if there were no royalties due to Third Parties.

3.3.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under Section 3.3.1 on the Net Sales of Licensed Products in a given country will end on a country by country basis when ****.

3.4 Sublicense Fees.

3.4.1 If Licensee exercises the Commercial Option in accordance with Section 2.2, in further consideration of the license granted to Licensee under Section 2.2, Licensee shall pay Licensor a percentage of any sublicense fees (****) received by Licensee for the Licensed Patents from any sublicensee; provided, however, that Licensee shall have no obligation to pay Licensor any portion of non-cash consideration received from a sublicensee including any equity interests. The applicable percentage due to Licensor for each sublicense shall be ****. Notwithstanding anything to the contrary herein, in the event Licensee receives non-cash consideration for any sublicense granted to a Third Party hereunder, and such non-cash consideration is liquidated within one year of the effective date of such sublicense, then, with respect to the proceeds resulting from the liquidation of such non-cash consideration, Licensee shall pay to Licensor the percentage of sublicense fees that would have been payable on the date such consideration was received by Licensee if such payment had been received in cash. Notwithstanding anything to the contrary herein, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development and/or manufacturing activities performed by Licensee corresponding directly to the development of Licensed Products pursuant to a specific agreement, including a performance plan and commensurate budget;

(b) Proceeds derived from debt financing and any loans to Licensee by a sublicensee;

(c) Consideration received for the purchase of an equity interest in Licensee at fair market value; and

(d) Any and all amounts paid to Licensee by a sublicensee as royalties on sales of Licensed Product sold by the sublicensee under a sublicense agreement.

3.4.2 Amounts paid by Licensee to Licensor as sublicensee fees pursuant to Section 3.4.1 shall be **** creditable against the ****, ****, and **** paid under **** and the **** paid under ****.

3.5 Reports and Records.

3.5.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

3.5.1.1 Number of Licensed Products included within Net Sales, listed by country;

3.5.1.2 Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

3.5.1.3 Qualifying costs to be excluded from the gross consideration, as described in Section 1.15, listed by category of cost;

3.5.1.4 Net Sales of Licensed Products listed by country;

3.5.1.5 Royalties owed to Licensor, listed by category; and

3.5.1.6 the computations for any applicable currency conversions.

3.5.2 Licensee shall pay the royalties due under Section 3.3 and other payments due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue or the other consideration is received. Licensee shall send the royalty payments along with the report described in Section 3.5.1.

3.5.3 In addition, within **** after the end of each Calendar Quarter, Licensee must deliver to Licensor a report setting forth the amounts if any due pursuant to Section 3.2 or 3.4, together with a payment of the applicable amount.

3.5.4 Licensee shall maintain and require its Affiliates and its or their sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records for each Calendar Quarter must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and sublicensees will provide Licensor and its accountants with access to all of the books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more

than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and its accountants in connection with the review or audit.

3.6 Currency, Interest.

3.6.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.6.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal* as of the last business day of the Calendar Quarter in which the payment was received by Licensee, and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.5.1.

3.6.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to **** per month (or the maximum allowed by law, if less).

ARTICLE 4: DILIGENCE

4.1 If Licensee exercises the Commercial Option and the license grant in Section 2.2.2 becomes effective, Licensee shall use commercially reasonable efforts to develop, market, promote, and sell a Licensed Product in the Selected Commercial Field. Commercially reasonable efforts means efforts consistent with those utilized by ****.

4.2 If Licensee exercises the Commercial Option, then within **** after the date that the license grant in Section 2.2.2 becomes effective, and within **** of each **** thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or its or their sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and its or their sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and its or their sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or its or their sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its licensors.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted herein; provided that such disclosure be under confidentiality agreements with provisions comparable to those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly herein. A Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those under this Agreement.

5.2 No Public Announcement. No public announcement or other disclosure to Third Parties concerning the existence of or terms of this Agreement shall be made, either directly or indirectly, by any Party to this Agreement, except with the prior written consent of the other Party.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth herein. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided by Licensee to Licensor hereunder with any of Licensor's licensors of the Licensed Patents. In the event that the Receiving Party becomes

obligated by law to disclose the Confidential Information of the other Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, become abandoned, or unenforceable in all countries of the world. The license granted to Licensee under Section 2.2.2 shall become a fully paid-up, non-exclusive, royalty-free license, on a country-by-country basis, upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, become abandoned, or unenforceable in the applicable country where Licensed Patents existed.

6.2 Automatic Termination. This Agreement automatically terminates upon the Option Expiration Date if Licensee does not exercise the Commercial Option in accordance with Section 2.2 hereof.

6.3 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason, with or without cause.

6.4 Termination for Breach.

6.4.1 Licensor may terminate this Agreement, effective immediately upon the expiration of the applicable cure period described below, if Licensee is more than **** late in paying to Licensor royalties, expenses, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor; or

6.4.2 Either Party may terminate this Agreement, effective immediately upon the expiration of the applicable cure period described below, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach.

6.5 Termination for Insolvency. Either Party may terminate this Agreement, effective immediately upon written notice to the other Party, if the other Party experiences any of the following: (a) if the other Party (i) becomes insolvent, bankrupt or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its

inability to pay its debts, (iv) suffers the appointment of a custodian, receiver or trustee for it or its property and, if appointed without its consent, not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by the other Party of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.5(a) or (b) above; (d) the calling by the other Party of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by the other Party indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.5(b) through (d) above. Furthermore, Licensor may terminate if any of the events described in this Section 6.5 occur with respect to (x) Guarantor, (y) any of Licensee's Affiliates to whom Licensee has granted any rights under this Agreement, or (z) Licensee's or its Affiliate's sublicensee; provided that, with respect to Licensee's or its Affiliates' sublicensees, such termination shall not be effective until **** after written notice to Licensee if Licensee has not terminated such sublicensee's sublicense agreement prior to the end of such **** period.

Notwithstanding the foregoing, in the event of termination of this Agreement pursuant to this Section 6.5, all licenses and rights under this Agreement are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code, licenses of rights to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The Parties agree that they shall retain and may fully exercise all of their respective rights and elections under the U.S. Bankruptcy Code. Each Party further agrees that, in the event of a rejection of this Agreement by or on behalf of the other Party in any bankruptcy proceeding by or against such other Party under the U.S. Bankruptcy Code the non-bankrupt Party shall be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of intellectual property, which, if not already in Licensee's possession, shall be promptly delivered to it upon Licensee's written request therefor. The term "embodiments" of intellectual property includes all tangible, intangible, electronic, or other embodiments of rights and licenses required to be delivered by the non-bankrupt Party hereunder.

6.6 Patent Challenge. Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee, its Affiliate, or its or its Affiliates' sublicensee of any action against the University of Pennsylvania, including an action for declaratory judgment, to declare, or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.7 Effects of Termination. The effect of termination by pursuant to Section 6.2, 6.3, 6.4, 6.5, or 6.6 shall be as follows:

6.7.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates and (unless the sublicense agreement is assigned pursuant to Section 6.7.2) its and their sublicensees shall cease to make or use the AAV8 Materials and/or to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to practice the Licensed Patents;

6.7.2 Any or all sublicenses granted to Third Parties related solely to the Licensed Patents or the Licensed Products shall survive this Agreement and be assigned to Licensor to the extent of the rights licensed to Licensee hereunder and sublicensed to the sublicensee by Licensee; provided that (i) such sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and (ii) Licensor shall not be liable to such sublicensee with respect to any obligations of Licensee to the sublicensee;

6.7.3 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.7.4 Each Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.8 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination shall survive the termination of this Agreement. In addition, the provisions of Section 3.5 — Reports and Records, Article 5 — Confidentiality, Article 6 — Term and Termination, Section 8.2 — Disclaimer of Warranties, Damages, Sections 8.3 and 8.4 — Indemnification, Article 9 — Use of Name, and Article 11 — Additional Provisions shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee but subject to any obligations of Licensor to its licensors of the Licensed Patents, the Parties agree as follows:

7.1.1 Licensor shall have the obligation to Prosecute patent applications and issued patents within Licensed Patents, at Licensor's expense and in the exercise of Licensor's reasonable business judgment. From and after Licensee's exercise of the Commercial Option set forth in Section 2.2 hereof, (a) Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and (b) Licensor shall keep Licensee reasonably informed as to all material developments with respect to such patent applications and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such patent applications.

7.1.2 Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents. If Licensor elects not to undertake any such inter-party proceedings, Licensor shall provide Licensee with reasonable advance notice and shall consider any reasonable request from Licensee that Licensee assume filing and financial responsibility for such inter-party proceedings.

7.1.3 Licensee acknowledges that the University of Pennsylvania controls Prosecution of the Licensed Patents. Licensee acknowledges and agrees the rights and obligations under this Section 7.1 are subject to the rights of Licensor's licensors Licensed Patents and Licensor's obligations only apply to the extent that Licensor has any rights with respect to Prosecuting the Licensed Patents under its agreements with its licensors.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee and Licensor are responsible for notifying each other promptly of any infringement of Licensed Patents (other than Retained Rights) which may come to their attention. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 Licensor shall have the sole right, but not the obligation, to prosecute any such infringement at its own expense prior to the Grant Date. Following the Grant Date, as between Licensor and Licensee, Licensor shall have the first right, but not the obligation, to prosecute any such infringement at its own expense. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action.

7.2.3 If, following the Grant Date, Licensor elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by any such License Patent and such Licensed Patent is being infringed by another product in the Selected Commercial Field (such infringement, the "Competitive Infringement"), Licensee shall have the second right, but not the obligation, to prosecute such Competitive Infringement with respect to such other product in the Selected Commercial Field, at Licensee's own expense. In any such action to enforce any of the Licensed Patents, Licensor, ****, shall cooperate to the fullest extent reasonably possible, including in the event that if Licensee is unable to initiate or prosecute such action solely in its own name, Licensor shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such Competitive Infringement, Licensee (a) shall not take any actions that would be detrimental to the Licensed Patents and Licensor's rights with respect thereto outside the Selected Commercial Field and (b) shall not settle any such Competitive Infringement without the prior consent of Licensor.

7.2.4 Any recovery of damages by Licensor prior to the Grant Date or after the Grant Date for any infringement other than a Competitive Infringement ****. Any recovery of damages by the Party undertaking enforcement or defense of a suit for Competitive Infringement following the Grant Date shall be applied, as between Licensor and Licensee but subject to the obligations to Licensor's licensors, first to reimburse each such Party for expenses and reasonable attorneys' fees incurred by such Party in connection with such suit, and the balance remaining, if any, from any such recovery shall be ****.

7.2.5 Licensee acknowledges and agrees the rights and obligations under this Section 7.2 are subject to the rights of Licensor's licensors Licensed Patents and Licensor's obligations only apply to the extent that Licensor has any rights with respect to enforcing the Licensed Patents under its agreements with its licensors.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or its or their sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify

the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and its or their sublicensees to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringements by another.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 To its knowledge, (a) the Licensed Patents are solely owned by the University of Pennsylvania, and (b) no Third Party (other than Licensor's licensors) has any right, interest or claim in or to such Licensed Patents in the Research Field or Commercial Field that are inconsistent with those granted to Licensee herein;

8.1.4 To its knowledge, no Third Party is infringing any of the Licensed Patents in the Research Field;

8.1.5 Licensor's license from the University of Pennsylvania is in full force and effect, and all payments to date required to be made thereunder by Licensor have been made;

8.1.6 Licensor is not in breach, and the University of Pennsylvania has not made any claim of breach by Licensor that has not been cured or otherwise resolved, of Licensor's license from the University of Pennsylvania;

8.1.7 Exhibit A contains a correct list of all United States and Patent Cooperation Treaty patents that are licensed to or controlled by Licensor or any of its Affiliates relating to the manufacture, use or sale of AAV8 Materials;

8.1.8 Licensor has not received any written notice or written warning letters from any Third Party patentee alleging infringement of, and to Licensor's knowledge Licensor has not been sued for patent infringement of, Third Party technology by the practice of the Licensed Patents in the Research Field.•

8.1.9 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensor's knowledge, threatened against Licensor, or any of its Affiliates, in each case, relating to the Licensed Patents that would impact activities under this Agreement.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee is an Affiliate of Guarantor;

8.2.4 Licensee has the ability arid the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.5 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee, or any of its Affiliates, in each case, that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS EXPRESSLY PROVIDED HEREIN, THE LICENSED PATENTS, LICENSED PRODUCTS, AND ANY OTHER TECHNOLOGY LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS. EXCEPT AS EXPRESSLY PROVIDED HEREIN, NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, AND HEREBY DISCLAIMS ALL IMPLIED WARRANTIES, INCLUDING BUT NOT LIMITED TO ANY WARRANTY OF ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, PROFITABILITY, COMMERCIAL UTILITY, NON-INFRINGEMENT, OR TITLE. NEITHER PARTY HERETO SHALL BE LIABLE FOR SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES ARISING OUT OF THIS AGREEMENT OR THE EXERCISE OF ITS RIGHTS HEREUNDER, INCLUDING LOST PROFITS ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.2 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its licensors of the Licensed Patents, and their respective shareholders, members, officers, trustees, faculty, students, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that results from or arises out of: ****; provided, however, that Licensee shall not be liable for claims based on the gross negligence or intentional

misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that was **** by Licensee, its Affiliates, their respective assignees, sublicensees, or vendors;

(b) any claim by a Third Party that the ****; provided, however, that Licensee shall not be liable for any such claim if such claim is based on Licensor's breach of any representation or warranty set forth in Section 8.1; and

(c) ****, its Affiliates, their respective assignees, sublicensees, or vendors ****, including any claim by or on behalf of a ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that results from or arises out of: ****; provided, however, that Licensor shall not be liable for claims based on the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.5 Indemnification Procedure. Each Party, as an indemnifying party (a "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on the indemnified party (a "Indemnified Party") without the other Party's prior written consent or, if Licensee is the Indemnifying Party, grant any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Article 8, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained herein are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise.

8.6 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and its and their sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence and in the aggregate; (b) prior to the commencement of clinical trials involving Licensed Products, clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence and in the aggregate; and (c) prior to the first commercial sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence and in the aggregate. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and its and their sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause its sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, its and their sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however, that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: GUARANTEE

10.1 Guarantor agrees that it shall be jointly and severally liable with Licensee with respect to the obligations of Licensee and its Affiliates and its and their sublicensees under this Agreement. Furthermore, Guarantor irrevocably guarantees each and every representation, warranty, covenant, agreement and other obligation of Licensee, and/or any of its permitted assigns (and where any such representation or warranty is made to the knowledge of Licensee, such representation or warranty shall be deemed made to the knowledge of Guarantor), and the full and timely performance of their respective obligations under the provisions of this Agreement. This is a guarantee of payment and performance, and not of collection, and Guarantor acknowledges and agrees that this guarantee is full and unconditional, and no release or extinguishment of Licensee's obligations or liabilities (other than in accordance with the terms of

this Agreement), whether by decree in any bankruptcy proceeding or otherwise, shall affect the continuing validity and enforceability of this guarantee, as well as any provision requiring or contemplating performance by Guarantor.

10.2 Guarantor hereby waives, for the benefit of Licensor, (1) any right to require Licensor, as a condition of payment or performance by Guarantor, to proceed against Licensee or pursue any other remedy whatsoever and (2) to the fullest extent permitted by law, any defenses or benefits that may be derived from or afforded by law that limit the liability of or exonerate guarantors or sureties, except to the extent that any such defense is available with respect to claims directly against Guarantor.

10.3 Warranty by Guarantor. Guarantor represents and warrants to Licensor as of the Effective Date that:

10.3.1 Guarantor has the right, power, and authority to enter into this Agreement and to perform its obligations hereunder;

10.3.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms; and

10.3.3 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee, or any of its Affiliates, in each case, that would impact activities under this Agreement.

ARTICLE 11: ADDITIONAL PROVISIONS

11.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

11.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective successors and assigns. Neither Party may assign its rights under this Agreement without the prior written consent of the other Party, such consent not to be unreasonably withheld; provided, however, a Party may assign this Agreement (a) to any Affiliate of such Party, (b) to any corporation or other entity to which such Party may transfer all or substantially all of its assets to which this Agreement relates ("Sale of Assets"), or (c) in connection with any merger or consolidation pursuant to which the holders of the voting power of the assigning Party immediately prior to such merger or consolidation hold, immediately after such merger or consolidation, less than 50% of the voting power of the assigning Party ("Sale by Merger"). The assigning Party shall notify the other Party in writing as soon as practicable following (but not more than **** after) the closing of any bona fide Sale of Assets or bona fide Sale by Merger; provided that notice shall, in all events, be provided by the assigning Party to the other Party prior to any public announcement of such Sale of Assets or Sale by Merger. No assignment shall relieve such Party of responsibility for the performance of any accrued obligations which it has prior to such assignment.

11.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

11.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee or Guarantor:
Chatham Therapeutics, LLC
45 N. Chatham Pkwy
Chapel Hill, NC 27517
Attn: Jade Samulski
Telephone: (919) 968-2727
Facsimile: (919) 968-2724

with a copy to:
Chatham Therapeutics, LLC
45 N. Chatham Pkwy
Chapel Hill, NC 27517
Attn: Jade Samulski
Telephone: (919) 968-2727
Facsimile: (919) 968-2724

Either Party may change its official address upon written notice to the other Party.

11.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of Delaware, without giving effect to conflict of law provisions. Subject to Section 11.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of Delaware with respect to any and all disputes concerning the subject of this Agreement.

11.6 Dispute Resolution. In the event of any controversy, claim or counterclaim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration in accordance with the Commercial Arbitration Rules of the American Arbitration Association ("AAA") then in effect. The arbitration shall be conducted as follows:

11.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration, it shall be conducted in English and held in New York, New York.

11.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be

resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

11.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 11.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 11.6.2 above and there is no agreed extension of time, the AAA may appoint the chairperson.

11.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 11.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of Delaware, without giving effect to conflict of law provisions. The decision and/or award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

11.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

11.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

11.6.7 Compliance with this Section 11.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 11.6 will prevent a Party from seeking interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property or other rights of that Party.

11.7 No Discrimination. Licensee, its Affiliates and its and their sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran of the Vietnam Era.

11.8 Compliance with Law. Licensee (and its Affiliates' and its and their sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations

under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 arid that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

11.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral. This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

11.10 Marking. Licensee, its Affiliates, and its and their sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, or offered for sale.

11.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

11.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

11.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; and (h) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each

Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

CHATHAM THERAPEUTICS, LLC

By: /s/ Jude Samulski
Name: Jude Samulski
Title: Partner

For purposes of Article 10,
ASKLEPIOS BIOPHARMACEUTICAL, INC.

By: /s/ Jude Samulski
Name: Jude Samulski
Title: President/Chairman

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Patents

Title	Inventors	Subject Matter	Ref nos.
****	****	****	****

Publication No.	Publication date	Country	Filing No.
****	****	****	****
****	****	****	****
****	****	****	****
****	****	****	****
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****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Licensee Therapeutic

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CONFIDENTIAL TREATMENT REQUESTED

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Exhibit 10.18

CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of October 30, 2013 ("Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), and Dimension Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at 1 Main Street, 13th Floor, Cambridge, MA 02142 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has exclusive rights under certain patents pertaining to various recombinant adeno-associated virus vectors; and

WHEREAS, Licensee desires to obtain an exclusive license under the Licensed Technology under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.2 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.3 "Collaboration" means an arrangement between Licensee and a Sublicensee under which research and development activities are performed on a shared basis for the purpose of the parties jointly developing and exploiting Licensed Products in the Field; provided that a Collaboration will not include an arrangement whereby Licensee is compensated solely for performing research or development activities.

1.4 "Commercial License" means a license agreement between Licensor and a Third Party pursuant to which Licensor grants a license to the Licensed Technology and which license agreement meets the following: (a) the agreement contains provisions substantially comparable to Section 2.6 with respect to improvements of the Third Party that are substantially similar to "Licensed Back Improvements" as defined in this Agreement; (b) the Third Party grants to Licensor a sublicensable license to such "Licensed Back Improvements" of the Third Party; and (c) Licensor is not required to pay any royalties, milestones, or other fees in connection with the exploitation of such sublicensable license.

1.5 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, Know-How, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other

proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.5 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.5.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.5.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.5.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.5.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.5.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.6 "Control" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent, patent application, Know-How, or other intellectual property on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any Third Party.

1.7 "Disclosing Party" has the meaning set forth in Section 5.1.

1.8 "Domain Antibody" ****.

1.9 "Existing Licenses" means the GSK Agreement and Penn Agreement.

1.10 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.11 "Field" means each of the following: (a) the treatment of hemophilia A in human beings by *in vivo* gene therapy administration; (b) the treatment of hemophilia B in human beings by *in vivo* gene therapy administration; and (c) the treatment of the specific disease indication(s) included within the "Field" pursuant to Section 2.4 in human beings by *in vivo* gene therapy administration.

1.12 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.13 "Know-How" means any and all ideas, information, know-how, data, research results, writings, inventions, discoveries, and other technology (including any proprietary materials), whether or not patentable or copyrightable.

1.14 "Licensed Know-How" means

(a) any Know-How that, as of the Effective Date, (i) is Controlled by Licensor pursuant to the Existing Licenses or the Penn Sponsored Research Agreement or pursuant to Licensor's ownership thereof and (ii) is reasonably necessary for the use, sale, offer for sale, or import of Licensed Products in the Field, including that which is set forth on Exhibit B; and

(b) if a specific disease indication is added to the Field pursuant to Section 2.4, any Know-How that, as (x) of the Effective Date or (y) if the added disease indication is one of the indications set forth on Exhibit D, as of the date on which such disease indication is added, (i) is Controlled by Licensor pursuant to the Existing Licenses or the Penn Sponsored Research Agreement or pursuant to Licensor's ownership thereof, (ii) is directed to the specific disease indication that is added, and (iii) is reasonably necessary for the use, sale, offer for sale, or import of Licensed Products in the added specific disease indication in the Field;

provided that "Licensed Know-How" will not include any Manufacturing Technology other than Triple-Transfection Know-How that otherwise falls within clause (a) or (b) above; provided further that "Licensed Know-How" will not include any patents or patent applications.

1.15 "Licensed Patents" means (a) all United States patents and patent applications listed in Exhibit A, as modified pursuant to Section 2.7.1, including patents arising from such patent applications; (b) any additional claims of patents and patent applications as required pursuant to Section 8.1.7; and (c) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.16 "Licensed Product" means (a) any product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the

country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service with respect to the administration of any product to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of sale.

1.17 "Licensed Technology" means, collectively, the Licensed Patents and Licensed Know-How.

1.18 "Licensor Improvements" means any patent or patent application that meets all of the following criteria:

(a) is directed to any of: the composition of recombinant adeno-associated virus vectors, methods of use of such vectors, or methods of developing such vectors, but, in each case, only to the extent of such claims;

(b) is reasonably necessary for any of: the use, sale, offer for sale, or import of Licensed Products in the Field; and

(c) prior to the 18 month anniversary of (i) the Effective Date, with respect to the disease indications of the Field set forth in Section 1.11(a) or (b), or (ii) the date on which a disease indication is added to the Field pursuant to Section 2.4, with respect to the disease indications of the Fields set forth in Section 1.11(c), is (x) is developed by Licensor or (y) becomes Controlled by Licensor pursuant to a Commercial License;

provided that "Licensor Improvements" will not include any Manufacturing Technology.

1.19 "Manufacturing Technology" means any and all patents, patent applications, Know-How, and all intellectual property rights associated therewith, and including all tangible embodiments thereof, that are necessary or useful for the manufacture of adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, formulations, quality control data, specifications, scale up, any and all improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided to regulatory authorities and the information contained therein are deemed Manufacturing Technology.

1.20 "Muscular Dystrophy" ****.

1.21 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.22 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.23 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.24 "Penn Sponsored Research Agreement" means that certain Sponsored Research Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended from time to time, including by Amendment No. 1, effective February 24, 2010, Amendment No. 2, dated March 31, 2010, Amendment No. 3, dated December 31, 2010, Amendment No. 4, effective December 31, 2011, Amendment No. 5, effective April 1, 2012, and Amendment No. 6, effective December 31, 2012.

1.25 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, and interferences.

1.26 "Receiving Party" has the meaning set forth in Section 5.1.

1.27 "Retained Rights" has the meaning set forth in Section 2.2.

1.28 "Sublicensee" means any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement.

1.29 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.30 "Triple Transfection Know-How" means unpatented Know-How that, as of the Effective Date, (a) is Controlled by Licensor pursuant to the Existing Licenses or the Penn Sponsored

Research Agreement or pursuant to Licensor's ownership thereof, (b) is directed to the triple-transfection method for making adeno-associated virus vectors, and (c) is set forth on Exhibit B; provided that, notwithstanding the scope of the license grant in Section 2.1, any rights granted to Licensee under this Agreement with respect to the Triple Transfection Know-How will be limited to use of such Know-How in the Field through Phase 2 clinical trials.

1.31 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive, sublicensable (as provided in Section 2.5 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field, including, for the avoidance of doubt, the right to conduct research and development, including conducting pre-clinical and clinical trials.

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1, or as provided in Section 8.1.7, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise, whether any such intellectual property dominates or is dominated by the Licensed Technology. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Technology for any research, development, commercial, or other purposes, outside of the Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees to the following rights retained by Licensor and its direct and indirect licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Field:

2.2.1 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor and its direct and indirect licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector.

2.2.2 Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Technology:

(a) A non-exclusive, sublicensable right under the Licensed Technology to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector; and

(b) A non-exclusive right for Licensor's direct and indirect licensors (which right is sublicensable by such licensors) to use the Licensed Technology for non-commercial research purposes and to use the Licensed Technology for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under (a) the Licensed Technology that cover the rAAV serotype 8, to make, have made, use, sell, offer for sale, and import products for the treatment of all forms of hemophilia B; or (b) the Licensed Technology that cover the rAAV serotype 9, to make, have made, use, sell, offer for sale, and import products for the treatment of (i) all forms of Muscular Dystrophy; (ii) congestive heart failure suffered by Muscular Dystrophy patients; and (iii) any and all cardiovascular diseases by delivery of any or all of genes encoding I-lc and Serca2a and creatine kinase.

2.2.4 Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Technology: a non-exclusive, sublicensable right to make, have made, use, sell, offer for sale, and import all of the various serotypes of any adeno-associated vector that is the subject of at least one claim in the Licensed Patents solely for non-commercial research in the areas of Muscular Dystrophy, hemophilia B, congestive heart failure suffered by Muscular Dystrophy patients, and other cardiovascular disease.

2.2.5 Licensor retains the following rights with respect to the Licensed Technology: to the extent Licensed Technology pertains to recombinant adeno-associated virus serotype 8, an exclusive, sublicensable right to make, have made, use, sell, offer for sale, and import products for the treatment of hemophilia A.

2.2.6 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field; or

(b) to use the Licensed Technology to provide services to any Third Parties; provided that, for clarity, Licensee's license under Section 2.1 does include the right to administer Licensed Products to patients. For clarity, activities conducted by Licensee for a Sublicensee as part of a Collaboration are not intended to be deemed services under this Section 2.2.6(b).

2.2.7 Licensor retains the fully sublicensable right under the Licensed Technology to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided

that such development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Field or any rights to sell products in the Field.

2.2.8 The University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Technology solely for educational, research, and other non-commercial purposes.

2.2.9 The Parties acknowledge that the Retained Rights included in Sections 2.2.3 and 2.2.4 are excluded from this Agreement because they were retained by the licensor under the GSK Agreement and that the Retained Rights included in Section 2.2.5 are excluded from this Agreement because of rights granted by Licensor to other licensees or Third Parties. If Licensor is granted the rights described in Section 2.2.3 or 2.2.4 or regains the rights described in Section 2.2.5, Licensor will notify Licensee of such event, together with a description of the rights granted or regained, in which case, the applicable Retained Rights granted or regained will no longer be considered Retained Rights, and the license granted to Licensee under Section 2.1 will no longer be subject to such granted or regained rights.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States absent, with respect to such manufacturing requirement, a waiver of such requirement obtained by Licensee from the applicable governmental agency.

2.4 Additional Disease Indications.

2.4.1 At any time prior to the ****of the Effective Date (the "Election Term"), Licensee may nominate in writing to Licensor a specific disease indication for inclusion in the "Field" under this Agreement. Within **** of Licensor's receipt of such notice, Licensor will inform Licensee in writing whether the nominated disease indication is available for licensing based on whether it: (a) is a disease indication set forth on Exhibit D; provided that the indications so listed do not constitute a limitation on the indications Licensee may nominate; (b) is the subject of a conflicting license with a Third Party (or the subject of a license being negotiated with a Third Party, as to which (i) there has been a written request for license terms from such Third Party, (ii) such Third Party or Licensor has submitted a written proposal for terms for a license (which may be limited to financial terms), (iii) Licensor and such Third Party have entered into a confidentiality agreement for purposes of such Third Party conducting a due diligence review, and (iv) a "writing" for purposes of the foregoing clauses includes e-mail correspondence); or (c) is part of an existing Licensor program (*i.e.*, a program that is the subject of on-going advanced preclinical study (*e.g.*, there has been a pre-IND meeting) or is in clinical development or at a later stage of development or commercialization). If the nominated disease indication is subject to a conflicting Third Party license or subject to an existing Licensor program, then the disease indication will be deemed rejected. Otherwise, the specific disease indication will be deemed available for licensing, and the Field will automatically be deemed to include the nominated specific disease indication immediately upon Licensor's delivery of a confirmatory written notice. For purposes of nominating a disease indication for inclusion in the

"Field," the indication must be a specific type of condition and not a general disease class, for instance "mucopolysaccharidosis (MPS) VI" and not "mucopolysaccharidosis (MPS)" and "hemophilia A" not "hemophilia." If Licensor determines that a disease indication nominated by Licensee pursuant to this Section 2.4 is not specific, Licensor will notify Licensee within ****of Licensor's receipt of the notice of nomination, and the Parties will negotiate in good faith as to the proposed scope and definition of the nominated disease indication.

2.4.2 Licensee will be entitled to nominate a reasonable number of specific disease indications during the Election Term until two additional specific disease indications are included in the Field.

2.4.3 During the Election Term, Licensor will not license (or enter into negotiations to license) a Third Party or initiate a Licensor program in any of the specific disease indications listed on Exhibit D, without the prior consent of Licensee. Except for the foregoing, nothing in this Agreement will prevent Licensor from granting licenses to any Third Parties for any disease indications or from initiating Licensor's own programs for any disease indications, in either case, other than the specific disease indications within the Field.

2.4.4 Notwithstanding Section 2.4.3 or anything herein to the contrary, nothing in this Agreement will prevent Licensor from (a) granting non-exclusive research licenses to Third Parties in any field; or (b) maintaining Licensor's commercial reagent and services business.

2.5 Sublicensing.

2.5.1 The license granted pursuant to Section 2.1 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.5 (including Section 2.5.2).

2.5.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses **** pursuant to a written sublicense agreement with the Sublicensee. Licensor must receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement; provided that nothing shall prevent Licensee from granting sublicenses of more limited scope than Licensee's rights, e.g. in a more limited territory, field of use, or term.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's

records and to share with Licensor's licensors under the Existing Licenses. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or any of its licensors') ability to ensure compliance with this Agreement; provided that, if any of Licensor's licensors require a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.6 Licensee's Improvements.

2.6.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, ****, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights; and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with any recombinant adeno-associated virus vectors, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that, during the term of this Agreement, Licensor shall have no right under the license in this Section 2.6.1(b) to practice the Licensed Back Improvements in the Field.

2.6.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.6.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement.

2.7 Licensor Improvements.

2.7.1 Licensor agrees to provide notice within **** to Licensee upon the filing of any patent application covering any Licensor Improvement, together with a reasonably detailed description of or access to such Licensor Improvement to permit the practice of any such

improvement. Upon the filing of any patent application covering any Licensor Improvement, Exhibit A attached hereto will be modified to add such patent application.

2.7.2 If Licensor files any patent or patent application that would constitute a Licensor Improvement but for the temporal limitation in Section 1.18(c), Licensor will within **** so inform Licensee, and, upon Licensee's written request, Licensor will, on a non-exclusive basis, discuss in good faith licensing such patent or patent application to Licensee for use in connection with the Licensed Products in the Field.

2.7.3 To the extent that the scope of Licensor's rights to any Licensor Improvements Controlled by Licensor pursuant to a Commercial License, as described in Section 1.18(c)(y), are less than or more restrictive than the license rights granted to Licensee pursuant to Section 2.1, then Licensee's rights with respect to such Licensor Improvements will be limited to the lesser or more restrictive rights Licensor can sublicense pursuant to the terms of the Commercial License. Examples of more restrictive provisions include Licensor's rights being limited to the following: (a) non-exclusive rights, (b) use in connection with only specific recombinant adeno-associate virus vectors, (c) use only in specific territories or specific fields, and (d) use only for research but not commercial purposes.

2.8 Transfer of Licensed Know-How.

2.8.1 During the **** period following the Effective Date, at Licensee's sole expense, to the extent not previously disclosed to Licensee, (a) Licensor will deliver to Licensee copies of Licensed Know-How set forth on Exhibit B in the form that such Licensed Know-How then exists; (b) Licensor will use commercially reasonable efforts to deliver, in the form that such Licensed Know-How then exists, such additional Licensed Know-How not listed on Exhibit B that is reasonably requested in writing by Licensee; and (c) Licensor will otherwise disclose, through not more than two meetings with Licensee personnel, other Licensed Know-How, which meetings will be at such times and in such places as are agreed to by the Parties.

2.8.2 During the **** period following the date on which a disease indication is added to the Field pursuant to Section 2.4, at Licensee's sole expense, to the extent not previously disclosed to Licensee, (a) Licensor will use commercially reasonable efforts to deliver, in the form that such Licensed Know-How then exists, such Licensed Know-How described in Section 1.14(b) that relates to such added disease indication that is reasonably requested in writing by Licensee; and (b) Licensor will otherwise disclose, through not more than two meetings with Licensee personnel, other Licensed Know-How described in Section 1.14(b) with respect to such added disease indication, which meetings will be at such times and in such places as are agreed to by the Parties.

2.8.3 Notwithstanding the foregoing, with respect to any Licensed Know-How not in Licensor's possession, Licensor's obligation will be limited to using reasonable efforts to cause such copies to be delivered to Licensee. Licensee acknowledges and agrees that all Licensed Know-How disclosed pursuant to this Section 2.8 will be deemed "Confidential Information" of Licensor, regardless of whether such information is marked or identified as confidential and without an obligation to summarize oral information.

2.9 Covenants Related to Existing Licenses. During the term of this Agreement, without the prior written consent of Licensee, which consent shall not be unreasonably withheld, Licensor agrees not to exercise its right to terminate and will not amend either of the Existing Licenses if such termination or amendment would materially, adversely alter the rights of Licensee under this Agreement. During the term of this Agreement, if Licensor receives a notice of termination under Section 6.3 of the Penn License, Licensor will so notify Licensee no later than **** before expiration of the applicable cure period and provide the particulars of the alleged breach.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall issue to Licensor 10,000 shares of Common Stock of Licensee pursuant to that certain Dimension Therapeutics, Inc. Common Stock Purchase Agreement of even date herewith.

3.2 Annual Maintenance Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor on-going annual maintenance fees of **** for each disease indication within the Field, which fees will be due on each anniversary of the Effective Date.

3.3 Royalties. In further consideration of the license granted to Licensee under Section 2.1, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.3.1:

Cumulative Annual Net Sales of all Licensed Products Worldwide	Royalty Percentage
Portion of Net Sales less than ****	****
Portion of Net Sales between (and including) **** through (and including) ****	****
Portion of Net Sales greater than ****	****

3.3.1 Adjustment of Royalties. The Parties acknowledge that the royalties set forth in this Section 3.3 have been set at an ****.

(a) If, after the Effective Date, ****, which amendment the Parties will negotiate in good faith.

(b) If, after the Effective Date, Licensee determines that (x) one or more Licensed Products (i) would fall within **** or (ii) would be entitled to a ****, or (y) Licensor would be entitled to ****

****, Licensee may provide Licensor with written notice thereof and reasonable documentation supporting Licensee's determination. Upon receipt thereof, Licensor will negotiate in good faith regarding whether Licensee's determination is correct and, if the Parties agree, an appropriate amendment to the royalties set forth in this Section 3.3.

(c) Negotiations for any adjustments to royalties under this Section 3.3.1 will take into account the royalties **** in the aggregate, as well as any ****.

3.3.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.3 on the Net Sales of Licensed Products in a given country will end on a country-by-country basis ****.

3.4 Third Party Obligations. In consideration of the license granted to Licensee under Section 2.1, Licensee agrees to the following:

3.4.1 Assumption of Obligations. Licensee acknowledges that certain Licensed Technology is licensed to Licensor pursuant to the Existing Licenses and will be sublicensed to Licensee hereunder. In addition to the obligations set forth herein, Licensee expressly agrees to be bound by and comply with all applicable provisions of the Existing Licenses to the extent such provisions apply to Licensee's or any of its Affiliates' or any Sublicensees' exploitation of Licensed Technology under this Agreement. To the extent that (a) any Licensed Technology is Controlled by Licensor pursuant to the Existing Licenses and sublicensed to Licensee under this Agreement and (b) the scope of rights granted under such Existing Licenses are less than the rights granted hereunder (such as Licensor's rights under the Existing Licenses being limited to non-exclusive rights), Licensee acknowledges that Licensee's rights and licenses hereunder with respect to such Licensed Technology are limited to such lesser scope.

3.4.2 Third Party Reports and Payments.

(a) Licensee will pay any milestone amounts owed under Section 3.2 of the GSK Agreement that are owed with respect to activities of Licensee in exercising its license under this Agreement. Licensee's obligation under this Section 3.4.2(a) for payments shall continue for so long as any payment obligations are due during the term of this Agreement under the Existing Licenses. For the avoidance of doubt, Licensee will not be deemed to owe a milestone amount if Licensor or a different licensee of Licensor achieves the milestone for which a payment is due prior to Licensee achieving such milestone.

(b) To the extent that any payment under Section 3.3 or 3.4.2(a) is deemed "Sublicensing Revenues" under Section 3.5 of the Penn Agreement or sublicensee fees under Section 3.4 of the GSK Agreement (collectively, "Sublicensing Fees"), Licensee will gross up such payments to ensure that Licensor receives exactly the amount that is owed for such payment under this Agreement and the Sublicensing Fees under the Existing Licenses.

(c) Licensee will make all payments due with respect to the Existing Licenses to Licensor not less than **** prior to the date on which such amounts must be paid by Licensor to its licensors under the applicable Existing Licenses.

(d) Licensee agrees to submit and to require its Affiliates and Sublicensees to submit to Licensor (or as otherwise directed by Licensor) all reports, including development and diligence reports, that Licensor is required to submit or pay pursuant to the Existing Licenses and all payments that Licensee has agreed to make as set forth in Section 3.4.2(a), in each case, to the extent such reports or payments are triggered by or otherwise result from Licensee's and its Affiliates' and any Sublicensees' exploitation of Licensed Technology under this Agreement. Unless otherwise agreed, with respect to any reporting and payment obligations under the Existing Licenses, Licensee (or its Affiliates or any Sublicensees) will provide the required reports to Licensor in sufficient time for Licensor to provide them to the applicable licensor within the time periods required by the applicable Existing License; provided that such reports will be provided to Licensor by not less than **** prior to the date on which such reports must be delivered by Licensor to its licensors under the applicable Existing License. All financial reports required to be delivered will be certified by the chief financial officer of Licensee.

(e) Without limiting the foregoing, within **** after the occurrence of a milestone event requiring a payment under either Existing License, Licensee will deliver to Licensor a report describing the milestone event that occurred and the date on which it occurred.

3.5 Reports and Records.

3.5.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.22, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) Royalties owed to Licensor, listed by category; and

(f) The computations for any applicable currency conversions.

3.5.2 Licensee shall pay the royalties due under Section 3.3 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.5.1.

3.5.3 All financial reports under this Section 3.5 will be certified by the chief financial officer of Licensee.

3.5.4 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records that enable the royalties, fees, and payments payable under this Agreement (directly or through the Existing Licenses) to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor (and its accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and accountants in connection with the review or audit. Without limiting the foregoing, Licensee acknowledges that its books and records will also be subject to the separate audit right of Licensor's licensors in accordance with the terms of the Existing Licenses.

3.6 Currency, Interest.

3.6.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.6.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal, N.Y.* edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.5.

3.6.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in each of the disease indications within the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****.

4.2 Specific Milestones. Without limiting Section 4.1, Licensee will meet the following milestones:

Event	Date
(a) Closing of $**** in financing	**** from Effective Date
(b) Milestones will be set forth in the initial Development Plan for the hemophilia A indication described in Section 1.11(a) and agreed upon by the Parties	****
(c) Milestones will be set forth in the initial Development Plan for the hemophilia B indication described in Section 1.11(b) and agreed upon by the Parties	****
(d) Filing of an investigational new drug application with the FDA for the proposed initial clinical trial of a Licensed Product targeting the first additional specific disease indication (as set forth in Section 1.11(c))	**** from the date on which the specific disease indication is added to the Field pursuant to Section 2.4
(e) Filing of an investigational new drug application with the FDA for the proposed initial clinical trial of a Licensed Product targeting the second additional specific disease indication (as set forth in Section 1.11(c))	**** from the date on which the specific disease indication is added to the Field pursuant to Section 2.4

Licensee will provide Licensor written notice within **** of the accomplishment of each of the foregoing milestones. For the avoidance of doubt, a breach of the milestone in (a) above will be deemed a breach with respect to all disease indications within the Field. If Licensee fails to meet a milestone for a particular disease indication within the Field, the date of the milestone (****) may, at Licensee's option, be extended for a period of **** from the original deadline date upon a payment to Licensor of **** within **** of the original deadline date; provided that Licensee will be entitled only to **** for each disease indication within the Field, and each ****

will require a separate payment of ****.

The Parties agree that the failure of Licensee to achieve a specific milestone contained in this Section 4.2 or in a Development Plan described in Section 4.3 for reasons beyond Licensee's reasonable control **** will not be considered a material breach hereunder; ****.

4.3 Development Plans

4.3.1 For each disease indication and corresponding Licensed Product in the Field, Licensee will prepare and deliver to Licensor a development plan and budget (each a "Development Plan"). The initial Development Plans for the initial two indications set forth in Section 1.11(a) or (b) will be delivered within **** after the Effective Date, and the Development Plan for each of the subsequent indications set forth in Section 1.11(c) will be delivered within **** of the date on which the applicable indication is added to the Field pursuant to Section 2.4.

4.3.2 Each Development Plan will cover the next two years, and will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period under Section 4.4 relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy.

4.3.3 Following receipt by Licensor of each Development Plan, Licensor will promptly notify Licensee of any comments or requested revisions, and the Parties will thereupon negotiate any appropriate revisions in good faith. With respect to developmental milestones to be set forth in the initial Development Plans for each of the initial two indications set forth in Section 1.11(a) or (b), the Parties will agree upon reasonable milestones and completion dates to be set forth in the Development Plan (and any amendments thereto).

4.4 Reporting. Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product pursuant to each Development Plan. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.4.1 Date of Development Progress Report and time covered by such report;

4.4.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.4.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.4.4 Updates to each Development Plan, including coverage of the next two years;

4.4.5 Projected total development remaining before product launch of each Licensed Product; and

4.4.6 Summary of significant development efforts using the Licensed Technology being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.5 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its licensors under the Existing Licenses.

4.6 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.6.3.

4.7 Exclusivity. ****

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements. The Parties agree they will release a joint press release in the form attached hereto as Exhibit E. Except as provided in Section 5.3, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other

Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with any of Licensor's direct and indirect licensors of the Licensed Technology. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, or become abandoned or unenforceable in all countries of the world. Upon expiration of this Agreement (but not early termination), Licensee's license to Licensed Know-How under Section 2.1 will become non-exclusive, perpetual, irrevocable, royalty-free with respect to the Licensed Know-How owned by Licensor and will continue with respect to all other Licensed Know-How for so long as Licensor's rights continue under the Existing Licenses (subject to Licensee paying any ongoing amounts due under the Existing Licenses and complying with any applicable ongoing obligations under the Existing Licenses); but, for the avoidance of doubt, such license will remain limited to the Field and subject to the Retained Rights.

6.2 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to one or more of the disease indications within the Field.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period; provided that, if termination is by Licensor as a result of Licensee's materially breaching Article 4, and if such breach only relates to one disease indication within the Field, but not all, then Licensor's termination right shall only be with respect to the disease indication with respect to which the breach related and not the remaining disease indications.

6.3.3 Notwithstanding the foregoing, if Licensee disputes in good faith that a payment is due or that such material breach exists, and gives Licensor written notice of such dispute within ****, in the case of payment, or ****, in the case of a material breach, following Licensee's receipt of Licensor's notice of default, then, Licensor may not terminate this Agreement until the dispute is resolved in accordance with Section 10.6 (and a payment is found to be due or a breach found to have occurred); provided that Licensor will be entitled to terminate this Agreement at the end of the original **** or **** cure period, as applicable, without waiting for resolution of the dispute in accordance with Section 10.6, if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee any of its Controlling Affiliates experiences any Trigger Event. "Controlling Affiliate" means an Affiliate that directly or indirectly controls Licensee within the meaning of Section 1.1.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives Licensor's licensor a right of termination under the Penn Agreement and such licensor threatens to terminate the Penn Agreement, then, upon receipt of notice to such effect, Licensor may terminate this

Agreement, effective immediately upon written notice to Licensee, if the Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives Licensor's licensor a right of termination under the Penn Agreement and such licensor threatens to terminate the Penn Agreement, then, upon receipt of notice to such effect, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if the Sublicensee commences a Patent Challenge.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor or the University of Pennsylvania or SmithKline Beecham Corporation (or their successors under the Existing Licenses), including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.6.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Technology; provided that Licensee, its Affiliates, and Sublicensees, shall have the right to continue to sell their existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.6.2 Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5,

(a) Licensee shall grant, and hereby grants (effective only upon any such termination of this Agreement), to Licensor a non-exclusive, perpetual, irrevocable, worldwide, ****, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

(b) Licensee shall grant, and hereby grants (effective only upon any such termination of this Agreement), to Licensor an exclusive (even as to Licensee), worldwide, ****, transferable, perpetual license, with the right to grant sublicenses, under the Licensee Technology to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field. For this purpose, the "Licensee Technology" means Licensee's patents, Know-How, and other intellectual property that improvements or modifications to or that are based on or derived in whole or in part from or that otherwise relate to any Licensed Technology to the extent such patents, Know-How, or other intellectual property pertains to (i) a recombinant adeno-associated virus vector or (ii) any expression construct provided by Licensor to Licensee as part of the Licensed Technology. To effectuate such license, upon any such termination of this Agreement, Licensee will promptly disclose to Licensor all Licensee Technology not already known to Licensor; and

(c) Licensee will transfer to Licensor ownership of any regulatory approvals then in Licensee's, its Affiliate's, or any Sublicensee's name related to Licensed Products containing any expression construct provided by Licensor to Licensee as part of the Licensed Technology and notify the appropriate regulatory authorities and take any other action reasonably necessary to effect such transfer of ownership. If ownership of any such regulatory approval cannot be transferred to Licensor in any country, Licensee hereby grants (effective only upon any such termination of this Agreement) to Licensor a permanent, exclusive (even as to Licensee), and irrevocable right of access and reference to such regulatory approvals for Licensed Products containing any expression construct provided by Licensor to Licensee as part of the Licensed Technology in such country in the Field.

6.6.4****

6.6.5 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.6.6 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

If termination is only with respect to a particular disease indication within the Field, but not all disease indications, then the provisions of this Section 6.6 shall only apply with respect to the terminated disease indications, and this Agreement shall continue with respect to the non-terminated disease indications.

6.7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2 (Retained Rights), 2.3 (Government Rights), 2.6 (Licensee's Improvements), 3.4 (if this Agreement expires and there are any continuing obligations under the Existing Licenses applicable to Licensee's continuing activities following expiration), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.5 (Reports and Records), Article 5 (Confidentiality), Article 6 (Term and Termination) except for Section 6.5, Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that the University of Pennsylvania controls Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of Licensor's licensors under the Existing Licenses, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under the Existing Licenses.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 As between Licensor and Licensee, **** shall have the first right, but not the obligation, to prosecute any such infringement ****. In any action to enforce any of the Licensed Patents, ****, at the request and expense of ****, shall cooperate to the fullest extent reasonably possible, including in the event that, if **** is unable to initiate or prosecute such action solely in its own name, **** shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action.

7.2.3 If **** elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by any such License Patent and such Licensed Patent is being infringed by another product **** (such infringement, the "**** Infringement"), **** shall have the second right, but not the obligation, to prosecute such **** Infringement with respect to such other product ****, at **** own expense. In any such action to enforce any of the Licensed Patents, ****, at the request and expense of ****, shall cooperate to the fullest extent reasonably possible, including in the event that, if **** is unable to initiate or prosecute such action solely in its own name,

**** shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such **** Infringement, **** (a) shall not take any actions that would be detrimental to the Licensed Patents and **** rights with respect thereto **** and (b) shall not settle any such **** Infringement without the prior consent of ****.

7.2.4 Any recovery of damages by **** for any infringement other than a **** Infringement shall be ****. Any recovery of damages by the Party undertaking enforcement or defense of a suit for **** Infringement shall be applied, as between Licensor and Licensee but subject to the obligations to Licensor's licensors under the Existing Licenses, first to reimburse each such Party for costs and expenses (including reasonable attorneys' fees and costs) incurred by such Party in connection with such suit, and the balance remaining, if any, from any such recovery shall be ****.

7.2.5 Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.2 are subject to the rights of Licensor's licensors under the Existing Licenses (including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Licensor's obligations under this Agreement only apply to the extent that Licensor has any rights with respect to enforcing the Licensed Patents under the Existing Licenses. Furthermore, Licensee acknowledges the following:

7.2.5.1 All monies recovered upon the final judgment or settlement of any action with respect to **** Infringement will also need to be allocated to Licensor's licensors under the Existing Licenses (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

7.2.5.2 Licensor's licensors under the Existing Licenses retain the continuing right to intervene at their own expense and join Licensor or Licensee in any claim or suit for infringement of the Licensed Patents.

7.2.5.3 In any infringement prosecuted by Licensors' licensors under the Existing Licenses, all financial recoveries will be ****.

7.2.5.4 In any infringement prosecuted by Licensor's licensors under the Existing Licenses, **** agrees, at the request and expense of such licensors, to cooperate to the fullest extent reasonably possible, to the same extent as though **** were prosecuting such suit (as provided in this Section 7.2, including Section 7.2.2).

7.2.5.5 The written consent of **** under the **** will be required (a) for any decision that would have a materially adverse effect on the validity, scope of patent claims, or enforceability of the Patent Rights and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on any of its ****, or grants any rights to the Licensed Patents other than rights that ****.

7.3 Defense of Infringement Claims.

7.3.1 In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringements by another.

7.3.2 To the extent Licensor takes any action, Licensor (or its licensors under the Existing Licenses) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, ****. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or any of its licensors under the Existing Licenses or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensor's knowledge, threatened against Licensor relating to the Licensed Technology that would impact activities under this Agreement;

8.1.4 To Licensor's knowledge, (a) the Licensed Patents are solely owned by the University of Pennsylvania, and (b) no Third Party has any right, interest, or claim in or to such Licensed Patents in the Field that are inconsistent with those granted to Licensee herein;

8.1.5 Licensor has not received any written notice from any Third Party patentee alleging infringement of, and to Licensor's knowledge Licensor has not been sued for patent infringement of, Third Party technology by the practice of the Licensed Patents in the Field;

8.1.6 Licensor has not received any written notice from any of its licensors under the Existing Licenses informing Licensor that there are any actions, suits, proceedings, or arbitrations pending against such licensors relating to the Licensed Patents that would impact activities under this Agreement;

8.1.7 To Licensor's knowledge, Licensor does not Control (through ownership or Control pursuant to the Existing Licenses) as of the Effective Date any patent or patent application (other than the Licensed Patents set forth on Exhibit A) that would necessarily be infringed by Licensee's practice of the Licensed Patents set forth on Exhibit A in connection with using, importing selling, and offering for sale of adeno-associated virus vectors claimed in such Licensed Patents in the Field. If it is determined, in accordance with the procedure of this Section 8.1.7, that Licensor Controls (through ownership or Control pursuant to the Existing Licenses) as of the Effective Date a patent or patent application (other than the Licensed Patents) that would necessarily be infringed by Licensee's practice of the Licensed Patents set forth on Exhibit A in connection with using, importing selling, and offering for sale of adeno-associated virus vectors claimed in such Licensed Patents in the Field, then Licensor shall include the applicable patent or patent application as a "Licensed Patent" hereunder but solely to the extent of the claim(s) that would necessarily be infringed by such practice of such Licensed Patents by Licensee, which inclusion shall be Licensee's sole remedy. At any time during the term of this Agreement, Licensee may notify Licensor in writing of any such patent or patent application that Licensee believes should be included as a "Licensed Patent" pursuant to this Section 8.1.7. Such written notice shall identify the relevant patent or patent application and relevant claim(s) and shall explain briefly why Licensee, in good faith, believes it should be included as a "Licensed Patent." If Licensor does not agree with Licensee, Licensor shall have **** following Licensor's receipt of Licensee's written notice to notify Licensee that Licensor disputes the inclusion of such patent or patent application or the scope of the remedy; in which event, such dispute will be resolved in accordance with Section 10.6. Upon the Parties' agreement (or a resolution, in favor of Licensee, of the dispute pursuant to Section 10.6), the applicable claim(s) of the applicable patent or patent application will be deemed a "Licensed Patent" hereunder. For the avoidance of doubt, Licensor makes no representation or warranty under this Section 8.1.7 as to any claim of (a) a patent or patent application covering Manufacturing Technology or (b) a patent or patent application that is not Controlled by Licensor pursuant to the Existing Licenses or pursuant to Licensor's ownership thereof, and Licensee acknowledges that (i) Manufacturing Technology claims of any patents or patent applications or (ii) claims of any patents or patent applications not Controlled by Licensor pursuant to the Existing Licenses or pursuant to Licensor's ownership thereof will not be added as "Licensed Patents" pursuant to the procedure set forth in this Section 8.1.7; and

8.1.8 To Licensor's knowledge, the Existing Licenses are in full force and effect and Licensor is not in breach of any provisions thereof.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTIONS 8.1 AND 8.2, THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED BY EITHER PARTY TO THE OTHER UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF ANY RIGHTS LICENSED BY EITHER PARTY TO THE OTHER, AND PROFITABILITY; OR (ii) THAT THE USE OF ANY RIGHTS GRANTED BY EITHER PARTY TO THE OTHER, INCLUDING ANY PRODUCTS RESULTING THEREFROM, WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NEITHER PARTY OR ANY OF SUCH PARTY'S DIRECT OR INDIRECT LICENSORS SHALL BE LIABLE TO THE OTHER PARTY, ITS SUCCESSORS OR ASSIGNS, OR ANY SUBLICENSEES OF EITHER PARTY, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF PRODUCTS ARISING THEREFROM; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER Article 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its Affiliates, sublicensees, the licensors under the Existing Licenses, and their respective shareholders, members, partners, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****

****; provided, however, that Licensee shall not be liable for claims based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Technology or Licensed Products, including any claim by or on behalf of a ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its Affiliates and Sublicensees and their respective shareholders, members, partners, officers, trustees, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensor shall not be liable for claims based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner (i) that imposes any restrictions or obligations on the indemnified party (an "Indemnified Party") or, if Licensee is the Indemnifying Party, on Licensor's licensors under the Existing Licenses, without the other Party's prior written consent, (ii) if Licensee is the Indemnifying Party, that grants any rights to the Licensed Technology or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent, or (iii) if Licensor is the Indemnifying Party, that grants any rights that are inconsistent with those granted to Licensee under this Agreement without

Licensee's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Within **** of the Effective Date, Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from **** of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensee acknowledges that Licensor's licensors under the Existing Licenses may review periodically the adequacy of the minimum amounts of insurance for each coverage required by the Existing Licenses, and Licensor reserves the right to require Licensee to adjust the limits set forth in this Section 8.5 to conform to any adjustments made by Licensor's licensors under the Existing Licenses. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor; provided, however, that Licensee may assign this Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with notice of such assignment at least five business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. In addition, Licensee will provide Licensor with notice of any change of control (i.e., the acquisition by a person or group of "control" of Licensee, as defined in Section 1.1) of Licensee at least five business days prior to the effectiveness of such change of control. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (*e.g.*, Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Dimension Therapeutics, Inc.
1 Main Street, 13th Floor
Cambridge, MA 02142
USA
Attn: President and CEO
Telephone: 617-231-2403
Facsimile: 617-231-2425

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations between senior executives of each Party with authority to resolve the dispute for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be

resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral. All "Confidential Information" disclosed by Licensor to Fidelity Biosciences Corp. (and then disclosed by Fidelity Biosciences Corp. to Licensee) pursuant to that certain Confidentiality Agreement dated September 10, 2012 between Licensor and Fidelity Biosciences Corp. or pursuant to any other agreements between them will be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.5). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall

include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words “herein” or “hereunder” relate to this Agreement; (e) “or” is disjunctive but not necessarily exclusive; (f) the word “will” shall be construed to have the same meaning and effect as the word “shall”; (g) all references to “dollars” or “$” herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections and exhibits in this Agreement are to Sections and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

DIMENSION THERAPEUTICS, INC.

By: /s/ Don Hayden
Name: Don Hayden
Title: President and CEO

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Patents

App #	Title	Inventors	Nos.	Penn Docket #
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Licensed Know-How

****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit C
Muscular Dystrophies

****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit D
Disease Indications

****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit E
Press Release

Exhibit 10.19

CONFIDENTIAL TREATMENT REQUESTED

FIRST AMENDMENT TO LICENSE AGREEMENT

This FIRST AMENDMENT TO LICENSE AGREEMENT (this "Amendment") is entered into as of June 18, 2014 (the "Amendment Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), and Dimension Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at 1 Main Street, 13th Floor, Cambridge, MA 02142 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor and Licensee entered into that certain License Agreement dated October 30, 2013 (the "Original Agreement"); and

WHEREAS, the Parties desire to make certain amendments to the Original Agreement;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

1. Definitions. Capitalized terms not defined in this Amendment have the meanings given such terms in the Original Agreement.

2. Amendments.

(a) The introductory paragraph of Section 3.5.1 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

3.5.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(b) Section 3.5.2 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

3.5.2 Licensee shall pay the royalties due under Section 3.3 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.5.1.

(c) Section 4.2 of the Original Agreement is hereby amended by replacing clause (b) of the milestone chart in such section with the following:

(b) ****

(d) Section 6.3.1 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

(e) Section 6.3.2 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within 30 days after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period; provided that, if termination is by Licensor as a result of Licensee's materially breaching Article 4, ****, but not all, then Licensor's termination right shall only be with respect to the disease indication with respect to which the breach related and not the remaining disease indications; provided further that, if termination is by Licensor as a result of Licensee materially breaching Section 3.5.1, such cure period will be **** (in place of ****).

(f) Section 8.5 of the Original Agreement is hereby amended by replacing the last sentence thereof with the following:

Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee; provided that, with Licensor's prior written consent, a Sublicensee may self-insure all or parts of the limits described above.

3. Incorporation. Article 10 of the Original Agreement is hereby incorporated *mutatis mutandis* into this Amendment.

4. Effect on Original Agreement. Except as specifically amended by this Amendment, the Original Agreement will remain in full force and effect and is hereby ratified and confirmed. Each future reference to the Original Agreement will refer to the Original Agreement as amended by this Amendment. To the extent a conflict arises between the terms of the Original Agreement and this Amendment, the terms of this Amendment shall prevail but only to the extent necessary to accomplish their intended purpose.

[*Remainder of Page Intentionally Left Blank*]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this First Amendment to License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

DIMENSION THERAPEUTICS, INC.

By: /s/ Thomas R. Beck
Name: Thomas R. Beck
Title: President & CEO

CONFIDENTIAL TREATMENT REQUESTED

SECOND AMENDMENT TO LICENSE AGREEMENT

This SECOND AMENDMENT TO LICENSE AGREEMENT (this "Second Amendment") is entered into as of September 29, 2014 (the "Second Amendment Effective Date") between REGENXBIO Inc. (f/k/a ReGenX Biosciences, LLC), a corporation organized under the laws of the State of Delaware, with offices at 1701 Pennsylvania Avenue, NW, Suite 900, Washington, DC 20006 ("Licensor"), and Dimension Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at 840 Memorial Drive, 4th Floor, Cambridge, MA 02139 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor and Licensee entered into that certain License Agreement dated October 30, 2013, as amended by that First Amendment to License Agreement dated June 18, 2014 (collectively, the "Original Agreement");

WHEREAS, by letter dated September 10, 2014, Licensee notified Licensor of its desire to exercise its option under Section 2.4 to nominate "ornithine transcarbamylase deficiency (OTCD)" as one of Licensee's two additional specific disease indications to be included in the Field, which nomination, by letter dated September 22, 2014, Licensor confirmed was available for licensing; and

WHEREAS, ****.

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

1. Definitions. Capitalized terms not defined in this Second Amendment have the meanings given such terms in the Original Agreement.

2. Acknowledgements & Amendments.

(a) The Parties acknowledge that, effective as of September 23, 2014, pursuant to the provisions of Section 2.4, the following has been added to the Field set forth in Section 1.11(c): "the treatment of ornithine transcarbamylase deficiency (OTCD) in human beings by in vivo gene therapy administration."

(b) The Parties agree that the "Election Term" is hereby ****. As such, the first sentence of Section 2.4.1 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

> At any time prior to the **** of the Effective Date (the "Election Term"), Licensee may nominate in writing to Licensor a specific disease indication for inclusion in the "Field" under this Agreement.

(c) Clause (a) of the second sentence of Section 2.4.1 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

(a) [Intentionally omitted]

(d) Section 2.4.1 of the Original Agreement is hereby further amended to add the following as a new sentence at the end of such section:

Notwithstanding the foregoing, without Licensor's prior written consent (which may be withheld in its sole discretion), Licensee may not nominate any of the following disease indications for inclusion in the "Field" under this Agreement: ****.

(e) Sections 2.4.3 and 2.4.4 of the Original Agreement are hereby amended and restated in their entirety to read as follows:

2.4.3 Nothing in this Agreement will prevent Licensor from granting licenses to any Third Parties for any disease indications or from initiating Licensor's own programs for any disease indications, in either case, other than the specific disease indications within the Field.

2.4.4 Notwithstanding anything herein to the contrary, nothing in this Agreement will prevent Licensor from (a) granting non-exclusive research licenses to Third Parties in any field; or (b) maintaining Licensor's commercial reagent and services business.

(f) Licensor and Licensee agree that the provisions of Section 1.14(b)(y) will apply with respect to ornithine transcarbamylase deficiency (OTCD), the specific disease indication added as of September 23, 2014, ****.

(g) In consideration of the ****, Licensee will pay Licensor ****, which fee will be due within **** of the Second Amendment Effective Date.

(h) Section 10.4 of the Original Agreement is hereby amended and restated in its entirety to read as follows:

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile or electronic mail (with a copy of such facsimile or electronic mail also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900

with a copy to:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900

Washington, DC 20006
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439
E-mail: kmills@regenxbio.com

Washington, DC 20006
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439
E-mail: sberl@regenxbio.com

If for Licensee:

Dimension Therapeutics, Inc.
840 Memorial Drive, 4th Floor
Cambridge, MA 02139
Attn: President and CEO
Telephone: 617-231-2403
Facsimile: 617-231-2425
E-mail: annalisa.jenkins@dimensiontx.com

Either Party may change its official address upon written notice to the other Party.

General communications required under this Agreement (including notices under Sections 2.2.9, 2.4, 2.5.2, 2.6.3, 2.7, 3.3.1, 3.5.4, 4.2, 4.3.3, 4.4, 7.1, 7.2, 7.3, 8.1.7, 8.5, and 10.2 and notices of changes of address under this Section 10.4) may be sent by any of the means outlined in the first sentence of this Section 10.4 or a copy of the notice letter may be sent by electronic mail (without the requirement of a copy being sent by another means, provided that the receiving Party has confirmed receipt of such electronic mail); however, communications related to termination of the Penn Agreement, requests for disclosures of Confidential Information, breaches or termination of this Agreement, indemnification, and dispute resolution (including notices under Sections 2.9, 5.3, 6.2, 6.3, 6.4, 6.5, 8.4, and 10.6) must be sent by one of the means outlined in the first sentence of this Section 10.4.

3. Incorporation. Article 10 of the Original Agreement is hereby incorporated *mutatis mutandis* into this Second Amendment.

4. Effect on Original Agreement. Except as specifically amended by this Second Amendment, the Original Agreement will remain in full force and effect and is hereby ratified and confirmed. Each future reference to the Original Agreement will refer to the Original Agreement as amended by this Second Amendment. To the extent a conflict arises between the terms of the Original Agreement and this Second Amendment, the terms of this Second Amendment shall prevail but only to the extent necessary to accomplish their intended purpose.

[*Remainder of Page Intentionally Left Blank*]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Second Amendment to License Agreement to be executed by their duly authorized representatives.

REGENXBIO, LLC

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

DIMENSION THERAPEUTICS, INC.

By: /s/ A. Jenkins
Name: A. Jenkins
Title: CEO

Exhibit 10.21

CONFIDENTIAL TREATMENT REQUESTED

OPTION AND LICENSE AGREEMENT

This OPTION AND LICENSE AGREEMENT ("Agreement") is entered into as of March 10, 2015 (the "Execution Date"), with effectiveness as of February 18, 2014 (the "Effective Date"), by and between REGENXBIO Inc., a limited liability company organized under the laws of the State of Delaware, with offices at 1701 Pennsylvania Avenue, NW, Suite 900, Washington, DC 20006 ("Licensor"), and Dimension Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at 840 Memorial Drive, 4th Floor, Cambridge, MA 02139 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has exclusive rights under certain patents pertaining to various recombinant adeno-associated virus vectors;

WHEREAS, Licensor and Licensee are parties to that certain License Agreement, dated October 30, 2013, as amended by the First Amendment to License Agreement, dated June 18, 2014, and the Second Amendment to License Agreement, dated September 29, 2014 and as amended from time to time (collectively, the "2013 License Agreement"), pursuant to which Licensor granted Licensee an exclusive license under certain technology of Licensor related to hemophilia A, hemophilia B, and additional disease indications to be selected as provided therein;

WHEREAS, Licensor, Licensee, and certain other persons are parties to that certain Series A Preferred Stock Purchase Agreement, dated October 30, 2013 (the "Series A SPA"); and

WHEREAS, Licensee, having met the conditions set forth in Section 6.16 of the Series A SPA on the Effective Date, desires to obtain an option for an exclusive license under the Licensed Technology under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.2 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.3 "Collaboration" means an arrangement between Licensee and a Sublicensee under which research and development activities are performed on a shared basis for the purpose of the parties jointly developing and exploiting Licensed Products in the Field; provided that a Collaboration

will not include an arrangement whereby Licensee is compensated solely for performing research or development activities.

1.4 "Commercial License" means a license agreement between Licensor and a Third Party pursuant to which Licensor grants a license to the Licensed Technology and which license agreement meets the following: (a) the agreement contains provisions substantially comparable to Section 2.5 with respect to improvements of the Third Party that are substantially similar to "Licensed Back Improvements" as defined in this Agreement; (b) the Third Party grants to Licensor a sublicensable license to such "Licensed Back Improvements" of the Third Party; and (c) Licensor is not required to pay any royalties, milestones, or other fees in connection with the exploitation of such sublicensable license.

1.5 "Commercial Option" has the meaning set forth in Section 2.1.

1.6 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, Know-How, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.7 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.6.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.6.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.6.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.6.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.6.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.7 "Control" or "Controlled" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent, patent application, Know-How, or other intellectual property on the terms and conditions set forth in this Agreement without violating the terms of any agreement or other arrangement with any Third Party.

1.8 "Disclosing Party" has the meaning set forth in Section 5.1.

1.9 "Domain Antibody" ****.

1.10 "Existing Licenses" means the GSK Agreement and Penn Agreement.

1.11 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.12 "Field" means, if and when a Commercial Option(s) is exercised pursuant to Section 2.1 and the license set forth in Section 2.1.4 is effective for a particular Licensed Indication(s), the treatment of such Licensed Indication(s) in human beings by in vivo gene therapy administration. Each Licensed Indication for the Field will be set forth on Exhibit D (to be amended as of the applicable Grant Date as provided in Section 2.1.3.4).

1.13 "First Commercial Sale" means, on a Licensed Product-by-Licensed Product and country-by-country basis, the date of the first arm's length transaction, transfer, or disposition for value by or on behalf of Licensee, its Sublicensees, or their respective Affiliates to a Third Party of such Licensed Product for end use or consumption of such Licensed Product after regulatory approval of such Licensed Product has been granted, or such marketing and sale is otherwise permitted, by the applicable regulatory authority of such country. First Commercial Sale excludes any sale or other distribution for use in a clinical trial or other development activity, promotional use (including samples), or for compassionate use or on a named patient basis.

1.14 "Grant Date" has the meaning set forth in Section 2.1.4.

1.15 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.16 "Know-How" means any and all ideas, information, know-how, data, research results, writings, inventions, discoveries, and other technology (including any proprietary materials), whether or not patentable or copyrightable.

1.17 "Licensed Back Improvements" has the meaning set forth in Section 2.5.2.

1.18 "Licensed Indication" has the meaning set forth in Section 2.1.3.4.

1.19 "Licensed Know-How" means, on a specific Licensed Indication-by-Licensed Indication basis, any Know-How that, as of the Grant Date for the applicable Licensed Indication, (i) is Controlled by Licensor pursuant to the Existing Licenses or the Penn Sponsored Research Agreement or pursuant to Licensor's ownership thereof, (ii) is directed to the applicable Licensed Indication, (iii) is not generally available to the public or otherwise part of the public domain, other than through any act or omission of Licensee in breach of this Agreement, and (iv) is reasonably necessary for the use, sale, offer for sale, or import of Licensed Products in the applicable Licensed Indication in the Field, to be generally described in Exhibit B pursuant to Section 2.1.3.4(iv); provided that "Licensed Know-How" will not include any Manufacturing Technology; provided further that "Licensed Know-How" will not include any Know-How disclosed in patents or patent applications.

1.20 "Licensed Patents" means (a) all United States patents and patent applications listed in Exhibit A, as modified pursuant to Section 2.6.1, including patents arising from such patent applications; and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.21 "Licensed Product" means (a) any product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, (i) the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import or (ii) that incorporates, was developed using, or is produced or manufactured through the use of, or with respect to which Licensee otherwise acquired a license to, Licensed Know-How; or (b) any service with respect to the administration of any product to patients that (i) in the absence of the licenses granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of sale or (ii) that incorporates, was developed using, or is produced or manufactured through the use of, or with respect to which Licensee otherwise acquired a license to, Licensed Know-How.

1.22 "Licensed Technology" means, collectively, the Licensed Patents and Licensed Know-How.

1.23 "Licensor Improvements" means any patent or patent application that meets all of the following criteria:

(a) is directed to any of: the composition of recombinant adeno-associated virus vectors, methods of use of such vectors, or methods of developing such vectors, but, in each case, only to the extent of such claims;

(b) is reasonably necessary for any of: the use, sale, offer for sale, or import of Licensed Products in the Field; and

(c) on a Licensed Indication-by-Licensed Indication basis, prior to the 18-month anniversary of the Grant Date for the applicable Licensed Indication, (i) is developed by Licensor or (ii) becomes Controlled by Licensor pursuant to a Commercial License;

provided that "Licensor Improvements" will not include any Manufacturing Technology.

1.24 "Manufacturing Technology" means any and all patents, patent applications, Know-How, and all intellectual property rights associated therewith, and including all tangible embodiments thereof, that are necessary or useful for the manufacture of adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, formulations, quality control data, specifications, scale up, any and all improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided to regulatory authorities and the information contained therein are deemed Manufacturing Technology.

1.25 "Muscular Dystrophy" ****.

1.26 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.27 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.28 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and by that certain Second Amendment to License Agreement effective on September 9, 2014, and as amended from time to time.

1.29 "Penn Sponsored Research Agreement" means (a) that certain Sponsored Research Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by Amendment No. 1, effective February 24, 2010, Amendment No. 2, dated March 31, 2010, Amendment No. 3, dated December 31, 2010, Amendment No. 4, effective December 31, 2011, Amendment No. 5, effective April 1, 2012, Amendment No. 6, effective December 31, 2012, Amendment No. 7, effective January 1, 2014, and Amendment No. 8, effective March 15, 2014; (b) that certain Sponsored Research Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective November 1, 2013; and (c) any additional amendments to either (a) or (b) effective prior to the Grant Date for a Licensed Indication.

1.30 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.31 "Program Costs" means all documented costs incurred by Licensor prior to the applicable Grant Date in researching or developing the applicable disease indication, as determined in accordance with Licensor's normal procedures, as accounted for and consistently applied according to U.S. generally accepted accounting principles. Program Costs will include all out-of-pockets costs, time of scientific, technical, or other personnel (which may be billed on a full-time-equivalent basis at Licensor's normal full-time-equivalent rate, taking into account the reasonable costs of employment of personnel, including salaries and benefits), and reasonable overhead and indirect costs allocated to such disease indication. Costs paid to a Third Party will equal 100% of the amounts invoiced by the Third Party.

1.32 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, interferences, and any post-grant proceedings including supplemental examination, post-grant review, and inter parties review.

1.33 "Receiving Party" has the meaning set forth in Section 5.1.

1.34 "Retained Rights" has the meaning set forth in Section 2.2.

1.35 "Sublicensee" means any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement.

1.36 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.37 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: OPTION GRANT

2.1 Option Grant. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee four distinct options, exercisable at Licensee's sole discretion, each to obtain an exclusive, worldwide license (under the terms described in Section 2.1.4 and this Agreement) with respect to a single disease indication (each such option with respect to a particular disease indication, a "Commercial Option") in accordance with the following provisions:

2.1.1 Election Term. Licensee may exercise each Commercial Option at any time prior to the **** of the Effective Date (the "Election Term"); provided that Licensee may extend the Election Term for an additional **** at any time prior to the **** of the Effective Date by providing written notice to Licensor of such extension and simultaneously paying Licensor a fee of ****, which notice and payment must be received by Licensor at least **** prior to the **** of the Effective Date. If Licensee does extend the Election Term by timely providing such notice and payment, the Election Term will automatically be extended until the **** of the Effective Date.

2.1.2 Transferability. The Commercial Options shall not be sublicensable or transferable, except in the case of any assignment of this Agreement pursuant to Section 10.2.

2.1.3 Method of Exercise. To exercise the Commercial Option for a particular disease indication:

2.1.3.1 Licensee must provide written notice to Licensor at least **** prior to the expiration of the Election Term, which written notice must specify the disease indication(s) with respect to which Licensee desires to exercise a Commercial Option (the "Nomination Notice").

2.1.3.2 Within **** of Licensor's receipt of such Nomination Notice, Licensor will inform Licensee in writing (the "Availability Notice") of whether the nominated disease indication is available for licensing based on whether it is the subject of any of the following:

(i) a conflicting license with a Third Party (such license, a "Conflicting License"),

(ii) a license being negotiated with a Third Party, as to which ****

****, a "Conflicting Negotiation"); in which event, the Availability Notice will describe whether the license under negotiation would be exclusive or non-exclusive, the disease indication and territory subject to the Conflicting Negotiation, the applicable adeno-associated virus vector(s) being discussed, and any other exclusions that would apply to Licensee's exercise of its Commercial Option for the nominated disease indication (collectively, the "Excluded Rights"); or

(iii) an existing Licensor program (i.e., a program that is the subject of on-going advanced preclinical study (e.g., there has been a pre-IND meeting) or is in clinical development or at a later stage of development or commercialization by Licensor or its Affiliates) (such program, a "Conflicting Program").

2.1.3.3 If Licensor states in the Availability Notice that the nominated disease indication is subject to a Conflicting License or a Conflicting Program, then no Commercial Option will be deemed exercised with respect to such nominated disease indication, in which event Licensee will have the continuing right, until at least **** prior to the expiration of the Election Term, to nominate another disease indication with respect to which Licensee desires to exercise such Commercial Option. If Licensor states in the Availability Notice that the nominated disease indication is subject to a Conflicting Negotiation, then such nominated disease indication will be deemed available for licensing, but such license shall be subject to any Excluded Rights that are being negotiated with the Third Party as part of the Conflicting Negotiation, and the Availability Notice sent by Licensor to Licensee will include a statement of Program Costs, if any, associated with the nominated disease indication. If the nominated disease indication is not subject to a Conflicting License, Conflicting Program, or Conflicting Negotiation, Licensor will so state in the Availability Notice, and such nominated disease indication will be deemed available for licensing, and the Availability Notice sent by Licensor to Licensee will include a statement of (i) Program Costs, if any, associated with the nominated disease indication as of the date of such Availability Notice, plus Licensor's reasonable, good faith estimate for the anticipated Program Costs for the **** period following the date of such Availability Notice, and (ii) to Licensor's knowledge, a general description of any Know-How Controlled by Licensor that is applicable to the nominated disease indication and proposed to be included in Exhibit B as Licensed Know-How; provided that Licensor will not be required to disclose any such Licensed Know-How prior to the Grant Date.

2.1.3.4 If the nominated disease indication set forth in the Nomination Notice is available (in whole or, in the case of a Conflicting Negotiation, subject to the Excluded Rights), Licensee will have **** from receipt of the Availability Notice to notify Licensor whether it wishes to include in the license any Licensed Know-How identified by Licensor pursuant to 2.1.3.3 and to pay Licensor, by wire transfer, (a) the commercial option fee set forth in Section 3.1 and (b) if applicable, an amount equal to **** the Program Costs for such nominated disease indication; provided that Licensee will not be required to pay, on a Commercial Option-by-Commercial Option basis, more than **** in the aggregate under this clause (b). If Licensee fails to deliver such payment

within such **** period, the nominated disease indication will be deemed rejected by Licensee, and no Commercial Option will be deemed exercised with respect to such indication, in which event Licensee will have the continuing right, at least until **** prior to the expiration of the Election Term, to nominate another disease indication with respect to which Licensee desires to exercise a Commercial Option. If Licensee makes such payment within such **** period, (i) the license grant in Section 2.1.4 will become effective (subject to any Excluded Rights, if applicable), (ii) Exhibit D will be amended to set forth the applicable disease indication with respect to which the license in Section 2.1.4 has been granted (a "Licensed Indication") and, if applicable, any Excluded Rights, (iii) the additional representation and warranty by Licensor as set forth in Exhibit F shall become effective as of the Grant Date for the applicable Licensed Indication (unless Licensor has otherwise disclosed to Licensee in the Availability Notice any exceptions to such representation and warranty), (iv) the Parties shall promptly amend Exhibit A to include, subject to Section 2.6, any then-existing Licensor Improvements applicable to such Licensed Indication and, if Licensee has notified Licensor that it wishes to include Licensed Know-How in the license, the Parties shall promptly amend Exhibit B to include a general description of such Licensed Know-How, and (v) Licensee will have exhausted one of its four Commercial Options.

2.1.3.5 For purposes of nominating a disease indication for the exercise of a Commercial Option, the indication must be a specific type of condition and not a general disease class, for instance "mucopolysaccharidosis (MPS) VI" and not "mucopolysaccharidosis (MPS)" and "hemophilia A" not "hemophilia." If Licensor determines that a disease indication nominated by Licensee pursuant to this Section 2.1.3 is not sufficiently specific, prior to providing the Availability Notice and within **** of Licensor's receipt of the Nomination Notice, Licensor will notify Licensee, and the Parties will negotiate in good faith as to the proposed scope and definition of the nominated disease indication.

2.1.3.6 Licensee will be entitled to continue to nominate **** **** of specific disease indications at least **** prior to the expiration of the Election Term, until Licensee has exercised its four Commercial Options.

2.1.3.7 Nothing in this Agreement will prevent Licensor from granting licenses to any Third Parties for any disease indications or from initiating Licensor's own programs for any disease indications, in either case, other than the specific Licensed Indications with respect to which Licensee has exercised a Commercial Option.

2.1.3.8 Notwithstanding anything herein to the contrary, nothing in this Agreement will prevent Licensor from (a) granting non-exclusive research licenses to Third Parties in any field; or (b) maintaining Licensor's commercial reagent and services business.

2.1.3.9 Provided that Licensee has not already exercised all four of its Commercial Options, if a nominated disease indication that was subject to a Conflicting License or Conflicting Program becomes available for licensing prior to the expiration of the Election Term, Licensor will promptly notify Licensee, in which event Licensee may

submit a new Nomination Notice for such disease indication at least **** prior to the expiration of the Election Term.

2.1.4 License Grant Upon Exercise. If Licensee exercises one of its Commercial Options for a particular disease indication (after confirmation that the nominated disease indication is available as described in Section 2.1.3), effective only upon Licensor's receipt of the amounts set forth in, within the period set forth in, Section 2.1.3.4 (the date on which the payments are received in full shall be deemed to be the "Grant Date" for such disease indication), subject to the terms and conditions of this Agreement, including the Retained Rights and including any Excluded Rights, Licensor will be deemed to have granted to Licensee an exclusive, sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Technology to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field for the Licensed Indication, including, for the avoidance of doubt, the right to conduct research and development, including conducting pre-clinical and clinical trials.

2.1.5 Disease Indications. For the avoidance of doubt, the foregoing license granted pursuant to Section 2.1.4 will be deemed granted on the Grant Date on a Commercial Option-by-Commercial Option and Licensed Indication-by-Licensed Indication basis, solely with respect to the Field associated with the Licensed Indication for which the specific Commercial Option was exercised under this Section 2.1. The Parties acknowledge that there may be different Grant Dates for each Licensed Indication, depending on when and if Licensee exercises a Commercial Option for a Licensed Indication.

2.1.6 Expiration of Commercial Options. If Licensee fails to exercise all four Commercial Options pursuant to this Section 2.1 by the expiration of the Election Period, any unexercised Commercial Options will terminate and be of no further force or effect upon the expiration of the Election Term.

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1.4 (if and when effective), no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise, whether any such intellectual property dominates or is dominated by the Licensed Technology. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Technology for any research, development, commercial, or other purposes, outside of the Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees to the following rights retained by Licensor and its direct and indirect licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Field:

2.2.1 The rights and licenses granted in Section 2.1.4 (if and when effective) shall not include any right (and Licensor and its direct and indirect licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector.

2.2.2 Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Technology:

(a) A non-exclusive, sublicensable right under the Licensed Technology to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector; and

(b) A non-exclusive right for Licensor's direct and indirect licensors (which right is sublicensable by such licensors) to use the Licensed Technology for non-commercial research purposes and to use the Licensed Technology for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 The rights and licenses granted in Section 2.1.4 (if and when effective) shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under (a) the Licensed Technology that cover the rAAV serotype 8, to make, have made, use, sell, offer for sale, and import products for the treatment of all forms of hemophilia B; or (b) the Licensed Technology that cover the rAAV serotype 9, to make, have made, use, sell, offer for sale, and import products for the treatment of (i) all forms of Muscular Dystrophy; (ii) congestive heart failure suffered by Muscular Dystrophy patients; and (iii) any and all cardiovascular diseases by delivery of any or all of genes encoding I-lc and Serca2a and creatine kinase.

2.2.4 Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Technology: a non-exclusive, sublicensable right to make, have made, use, sell, offer for sale, and import all of the various serotypes of any adeno-associated vector that is the subject of at least one claim in the Licensed Patents solely for non-commercial research in the areas of Muscular Dystrophy, hemophilia B, congestive heart failure suffered by Muscular Dystrophy patients, and other cardiovascular disease.

2.2.5 Licensor retains the following rights with respect to the Licensed Technology: to the extent Licensed Technology pertains to recombinant adeno-associated virus serotype 8, an exclusive, sublicensable right to make, have made, use, sell, offer for sale, and import products for the treatment of hemophilia A.

2.2.6 The rights and licenses granted in Section 2.1.4 (if and when effective) shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Technology:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field; or

(b) to use the Licensed Technology to provide services to any Third Parties; provided that, for clarity, Licensee's license under Section 2.1.4 (if and when effective) does include the right to administer Licensed Products to

patients. For clarity, activities conducted by Licensee for a Sublicensee as part of a Collaboration are not intended to be deemed services under this Section 2.2.6(b).

2.2.7 Licensor retains the fully sublicensable right under the Licensed Technology to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Field or any rights to sell products in the Field.

2.2.8 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Technology solely for educational, research, and other non-commercial purposes.

2.2.9 The Parties acknowledge that the Retained Rights included in Sections 2.2.3 and 2.2.4 are excluded from this Agreement because they were retained by the licensor under the GSK Agreement and that the Retained Rights included in Section 2.2.5 are excluded from this Agreement because of rights granted by Licensor to other licensees or Third Parties. If Licensor is granted the rights described in Section 2.2.3 or 2.2.4 or regains the rights described in Section 2.2.5, Licensor will notify Licensee of such event, together with a description of the rights granted or regained, in which case, the applicable Retained Rights granted or regained will no longer be considered Retained Rights, and the license granted to Licensee under Section 2.1.4 (if and when effective) will no longer be subject to such granted or regained rights.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States absent, with respect to such manufacturing requirement, a waiver of such requirement obtained by Licensee from the applicable governmental agency.

2.4 Sublicensing.

2.4.1 Upon the effectiveness of each Grant Date and the rights granted pursuant to Section 2.1.4, Licensee's rights to sublicense will be limited to the specific Licensed Indication covered by such license. The license granted pursuant to Section 2.1.4 (if and when effective) is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may grant sublicenses **** only pursuant to a written sublicense agreement with the Sublicensee. Licensor must receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement; provided that nothing shall prevent Licensee from granting sublicenses of more limited scope than Licensee's rights, e.g. in a more limited territory, field of use, or term.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with Licensor's licensors under the Existing Licenses. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or any of its licensors') ability to ensure compliance with this Agreement; provided that, if any of Licensor's licensors require a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.4.3 Any sublicense agreement granted by Licensee hereunder to a Third Party shall survive termination of this Agreement in accordance with and subject to the terms of Section 6.6.2.

2.5 Licensee's Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license, effective only as of the first Grant Date:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights; and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with any recombinant adeno-associated virus vectors, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that, during the term of this Agreement, Licensor shall have no right under the license in this Section 2.5.1(b) to practice the Licensed Back

Improvements in the Field with respect to the applicable Licensed Indication.

2.5.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees, after the first Grant Date and during the term of this Agreement, to any vector that is the subject of a claim within the Licensed Patents.

2.5.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement.

2.6 Licensor Improvements.

2.6.1 Licensor agrees to provide notice within **** to Licensee upon the filing of any patent application covering any Licensor Improvement, together with a reasonably detailed description of or access to such Licensor Improvement to permit the practice of any such improvement. Upon the filing of any patent application covering any Licensor Improvement, Exhibit A attached hereto will be modified to add such patent application, but such patent application covering the Licensor Improvement will only be deemed a Licensed Patent with respect to Licensed Products for use in the Field for the applicable Licensed Indication to which such Licensor Improvements relates.

2.6.2 If Licensor files any patent or patent application that would constitute a Licensor Improvement but for the temporal limitation in Section 1.23(c), Licensor will within **** so inform Licensee, and, upon Licensee's written request, Licensor will, on a non-exclusive basis, discuss in good faith licensing such patent or patent application to Licensee for use in connection with the Licensed Products in the Field.

2.6.3 To the extent that the scope of Licensor's rights to any Licensor Improvements Controlled by Licensor pursuant to a Commercial License, as described in Section 1.23(c)(ii), are less than or more restrictive than the license rights granted to Licensee pursuant to Section 2.1.4 (if and when effective), then Licensee's rights with respect to such Licensor Improvements will be limited to the lesser or more restrictive rights Licensor can sublicense pursuant to the terms of the Commercial License. Examples of more restrictive provisions include Licensor's rights being limited to the following: (a) non-exclusive rights, (b) use in connection with only specific recombinant adeno-associate virus vectors, (c) use only in specific territories or specific fields, and (d) use only for research but not commercial purposes.

2.7 Transfer of Licensed Know-How.

2.7.1 During the **** period following the Grant Date for a particular Licensed Indication, at Licensee's sole expense, to the extent not previously disclosed to Licensee, (a) Licensor will deliver to Licensee copies of Licensed Know-How generally described in Exhibit B in the form that such Licensed Know-How then exists; (b) Licensor will use commercially reasonable efforts to deliver, in the form that such Licensed Know-How then exists, such additional Licensed Know-How not listed on Exhibit B that relates to such Licensed Indication

that is reasonably requested in writing by Licensee; and (c) Licensor will otherwise disclose, through not more than two meetings with Licensee personnel, other Licensed Know-How with respect to such Licensed Indication, which meetings will be at such times and in such places as are agreed to by the Parties.

2.7.2 Notwithstanding the foregoing, with respect to any Licensed Know-How not in Licensor's possession, Licensor's obligation will be limited to using reasonable efforts to cause such copies to be delivered to Licensee. Licensee acknowledges and agrees that all Licensed Know-How disclosed pursuant to this Section 2.7 will be deemed "Confidential Information" of Licensor, regardless of whether such information is marked or identified as confidential and without an obligation to summarize oral information.

2.8 Covenants Related to Existing Licenses. During the term of this Agreement, without the prior written consent of Licensee, which consent shall not be unreasonably withheld, Licensor agrees not to exercise its right to terminate and will not amend either of the Existing Licenses if such termination or amendment would materially, adversely alter the rights of Licensee under this Agreement. During the term of this Agreement, if Licensor receives a notice of termination under Section 6.3 of the Penn License, Licensor will so notify Licensee no later than **** before expiration of the applicable cure period and provide the particulars of the alleged breach.

ARTICLE 3: CONSIDERATION

3.1 Commercial Option Fee. Licensee shall pay Licensor a fee of $1,000,000 upon exercise of each Commercial Option, in accordance with clause (a) of Section 2.1.3.4.

3.2 Annual Maintenance Fee. In consideration of the rights and licenses granted to Licensee under Section 2.1 with respect to the exercise of a given Commercial Option, Licensee shall pay Licensor on-going annual maintenance fees of **** for the Licensed Indication associated with such Commercial Option, which annual maintenance fee will be paid on the next-occurring anniversary of the Effective Date following the Grant Date for such Licensed Indication. For clarity, Licensee shall owe an annual maintenance fee for each Licensed Indication with respect to which a license was granted under Section 2.1 upon exercise of each Commercial Option.

3.3 Milestone Fees. In consideration of the rights and licenses granted to Licensee under Section 2.1 with respect to the exercise of a given Commercial Option, Licensee shall pay Licensor the following one-time milestone payments, on a per Licensed Indication basis, for the first Licensed Product for each Licensed Indication in the Field to achieve such milestone event:

Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (i.e., first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (i.e., first patient, first dose)	****
3. NDA submission in any country	****
4. NDA approval in the United States	****

Milestone	Milestone Payment
5. NDA approval in the European Union	****
6. DA approval in any country other than the United States or the European Union	****
Total:	$9,000,000

For clarity, the milestone payments set forth in this Section 3.3 are payable **** with respect to the first Licensed Product in a Licensed Indication in the Field that achieves the milestone event, ****. To the extent that either of the two development milestones in this Section 3.3 (i.e., first treatment of human subject in a clinical trial or first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of the NDA submission milestone, then, upon the achievement of such NDA submission milestone, the preceding unpaid development milestone payments shall be made in addition to the payment of the NDA submission milestone. For clarity, the total amount payable under this Section 3.3 with respect to any Licensed Indication for which a license was granted under Section 2.1 is $9,000,000, and the total amount payable to Licensor if all four Commercial Options are exercised is $36,000,000.

For clarity, if a Licensed Product for a Licensed Indication ceases to be a Licensed Product as defined in Section 1.21, and thereafter one or more of the above milestone events occurs with respect to such product (or service, as applicable), then no associated milestone payments shall be due as such product (or service, as applicable) is no longer deemed a Licensed Product at the time of such milestone achievement.

3.4 Royalties. In consideration of the rights and licenses granted to Licensee under Section 2.1 with respect to the exercise of a given Commercial Option, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products, on a Licensed Indication-by-Licensed Indication basis, subject to the reductions in royalty rates set forth in Section 3.4.1:

Cumulative Annual Net Sales of all Licensed Products Worldwide for the Licensed Indication	Royalty Percentage
Portion of Net Sales less than ****	****
Portion of Net Sales between (and including) **** through (and including) ****	****
Portion of Net Sales greater than ****	****

3.4.1 Adjustment of Royalties For Licenses. On a Licensed Product-by-Licensed Product, country-by-country basis, upon the date on which the manufacture, use, sale, offer for sale, or import of a Licensed Product does not infringe or is not covered by a Valid Claim in such country, then the ****.

3.4.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end on a country-by-country basis, as follows:

(a) with respect to any Licensed Product under Section 1.21(a)(i) or 1.21(b)(i) only (which Licensed Product is not also a Licensed Product under Section 1.21(a)(ii) or 1.21(b)(ii)), such royalty obligations for any such Licensed Product will end at such time as ****; and

(b) with respect to any Licensed Product under Section 1.21(a)(ii) or 1.21(b)(ii) (whether it is only a Licensed Product under such sections or also a Licensed Product under Section 1.21(a)(i) or 1.21(b)(i)), such royalty obligations for any such Licensed Product will end on ****.

3.5 Sublicense Fees.

3.5.1 In consideration of the rights and licenses granted to Licensee under Section 2.1 with respect to the exercise of a given Commercial Option, and subject to the remainder of this Section 3.5, Licensee will pay Licensor a percentage of any sublicense fees (including upfront payments and milestone payments and including any equity consideration received by Licensee or its Affiliates) received by Licensee or its Affiliates for the Licensed Technology from any Third Party Sublicensee or from any Third Party granted any option to obtain such a sublicense. The applicable percentage due to Licensor for each sublicense (or option), on a Licensed Indication-by-Licensed Indication basis, under each exclusive license granted under Section 2.1 upon exercise of a Commercial Option shall be as follows:

Event	Sublicense Fee Rate
If sublicensed (or optioned) on or before the **** anniversary of the Grant Date for the applicable Licensed Indication	****
If sublicensed (or optioned) on or before the **** anniversary of the Grant Date for the applicable Licensed Indication, but after the **** anniversary of such Grant Date	****

If sublicensed (or optioned) on or before the **** anniversary of the Grant Date for the applicable Licensed Indication, but after the **** anniversary of such Grant Date	****
If sublicensed (or optioned) on or before the **** anniversary of the Grant Date for the applicable Licensed Indication, but after the **** anniversary of such Grant Date	****
If sublicensed (or optioned) on or before the **** anniversary of the Grant Date for the applicable Licensed Indication, but after the **** anniversary of such Grant Date	****
If sublicensed (or optioned) after the **** anniversary of the Grant Date for the applicable Licensed Indication	****

For the avoidance of doubt, with respect to a transaction with a Third Party involving the grant of an option to obtain a sublicense, if the sublicense is later granted as a result of the exercise of such option, the sublicense fees applicable to such sublicense will be determined by reference to the date the original option was granted, not the date the actual sublicense was granted.

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the development of Licensed Products pursuant to a specific agreement;

(b) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value or in the form of loans at commercially reasonable rates of interest; and

(c) Any and all amounts paid to Licensee or its Affiliates by a Third Party Sublicensee as royalties on sales of Licensed Product sold by such Sublicensee under a sublicense agreement.

3.5.3 To the extent Licensee or its Affiliates receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.3, then the amount of the payment made to Licensor under such Section 3.3 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.5; instead, the amounts due under this Section 3.5 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.5.1 above to the sublicense fees received by Licensee or its Affiliates from such Third Party after deducting the amount of the payment under Section 3.3.

3.5.4 If Licensee or its Affiliates receive sublicense fees from Third Party Sublicensees or from any Third Party granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.5 ****.

3.5.5 If Licensee or its Affiliate enters into any sublicense that is not an arm's length transaction, fees due under this Section 3.5 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined **** based on transactions of a similar type and standard industry practice, if any.

3.6 Third Party Obligations. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee agrees to the following:

3.6.1 Assumption of Obligations. Licensee acknowledges that certain Licensed Technology is licensed to Licensor pursuant to the Existing Licenses and will be sublicensed to Licensee hereunder. In addition to the obligations set forth herein, Licensee expressly agrees to be bound by and comply with all applicable provisions of the Existing Licenses to the extent such provisions apply to Licensee's or any of its Affiliates' or any Sublicensees' exploitation of Licensed Technology under this Agreement. To the extent that (a) any Licensed Technology is Controlled by Licensor pursuant to the Existing Licenses and sublicensed to Licensee under this Agreement and (b) the scope of rights granted under such Existing Licenses are less than the rights granted hereunder (such as Licensor's rights under the Existing Licenses being limited to non-exclusive rights), Licensee acknowledges that Licensee's rights and licenses hereunder with respect to such Licensed Technology are limited to such lesser scope.

3.6.2 Third Party Reports and Obligations. Licensee agrees to submit and to require its Affiliates and Sublicensees to submit to Licensor (or as otherwise directed by Licensor) all reports, including development and diligence reports, that Licensor is required to submit pursuant to the Existing Licenses, in each case, to the extent such reports are triggered by or otherwise result from Licensee's and its Affiliates' and any Sublicensees' exploitation of Licensed Technology under this Agreement. Unless otherwise agreed, with respect to any reporting obligations under the Existing Licenses, Licensee (or its Affiliates or any Sublicensees) will provide the required reports to Licensor in sufficient time for Licensor to provide them to the applicable licensor within the time periods required by the applicable Existing License; provided that such reports will be provided to Licensor by not less than **** prior to the date on which such reports must be delivered by Licensor to its licensors under the applicable Existing License. All financial reports required to be delivered will be certified by the chief financial officer of Licensee.

3.7 Reports and Records.

3.7.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the First Commercial Sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.27, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) An accounting of any royalty reductions applied pursuant to Section 3.4.1;

(f) Royalties owed to Licensor; and

(g) The computations for any applicable currency conversions.

3.7.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.7.1.

3.7.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3. In addition, within **** after the receipt of sublicense fees from any Third Party as described in Section 3.5, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.5.

3.7.4 All financial reports under this Section 3.7 will be certified by the chief financial officer of Licensee.

3.7.5 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records that enable the royalties, fees, and payments payable under this Agreement (directly or through the Existing Licenses) to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor (and its accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or, audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and accountants in connection with the review or audit. Without limiting the foregoing, Licensee acknowledges that its books and records will also be subject to the separate audit right of Licensor's licensors in accordance with the terms of the Existing Licenses.

3.8 Currency, Interest.

3.8.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.8.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.7.

3.8.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.9 Taxes and Withholding.

3.9.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.9.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.9.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Following the exercise of a Commercial Option, Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products for each of the Licensed Indications within the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****

****.

4.2 Specific Milestones. Without limiting Section 4.1, Licensee will meet the following milestones for each Licensed Indication with respect to which a Commercial Option is exercised:

Event	**Date**
Filing of an investigational new drug application with the FDA for the proposed initial clinical trial of a Licensed Product targeting the Licensed Indication	**** from the Grant Date for the Licensed Indication

Licensee will provide Licensor written notice within **** of the accomplishment of the foregoing milestone. If Licensee fails to meet the milestone for a particular Licensed Indication within the Field, the date of the milestone may, at Licensee's option, be extended for a period of **** from the original deadline date upon a payment to Licensor of **** within **** of the original deadline date; provided that Licensee will be entitled only to **** for each Licensed Indication within the Field, and **** extension will require a separate payment of ****.

****.

4.3 Development Plans

4.3.1 For each Licensed Indication and corresponding Licensed Product in the Field, Licensee will prepare and deliver to Licensor a development plan and budget (each a "Development Plan"). The initial Development Plans for each Licensed Indication will be delivered within **** after the Grant Date for such Licensed Indication.

4.3.2 Each Development Plan will cover the next two years, and will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period under Section 4.4 relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy.

4.3.3 Following receipt by Licensor of each Development Plan, Licensor will promptly notify Licensee of any comments or requested revisions, and the Parties will thereupon negotiate any appropriate revisions in good faith.

4.4 Reporting. Within **** after the first Grant Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing,

and commercialization of each Licensed Product for each Licensed Indication pursuant to each Development Plan. Licensee will also notify Licensor within **** of the First Commercial Sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.4.1 Date of Development Progress Report and time covered by such report;

4.4.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.4.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.4.4 Updates to each Development Plan, including coverage of the next two years;

4.4.5 Projected total development remaining before product launch of each Licensed Product; and

4.4.6 Summary of significant development efforts using the Licensed Technology being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.5 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its licensors under the Existing Licenses.

4.6 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information of the other Party are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements. The Parties agree they will release a joint press release in the form attached hereto as Exhibit E. Except as provided in Section 5.3, any other press releases by either

Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with any of Licensor's direct and indirect licensors of the Licensed Technology. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement.

6.1.1 Where at least one Commercial Option is exercised, this Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration of the royalty obligations with respect to all Licensed Products for all Licensed Indications under all licenses granted under all exercised Commercial Options, as described in Section 3.4.2. Upon expiration of this Agreement pursuant to this Section 6.1.1 (but not expiration pursuant to Section 6.1.2 or any early termination), Licensee's license to Licensed Know-How under Section 2.1.4 (to the extent it became effective) will become non-exclusive, perpetual, irrevocable, royalty-free with respect to the Licensed Know-How owned by Licensor and will continue with respect to all other Licensed Know-How for so long as Licensor's rights continue under the Existing Licenses (subject to Licensee paying any ongoing amounts due under the Existing Licenses and

complying with any applicable ongoing obligations under the Existing Licenses); but, for the avoidance of doubt, such license will remain limited to the applicable Licensed Indication in the Field under each such license and subject to the Retained Rights (and, if applicable, the Excluded Rights).

6.1.2 Where none of the Commercial Options is exercised in accordance with Section 2.1, this Agreement, unless sooner terminated as provided in this Agreement, expires on the expiration of the Election Term. Upon such expiration, Licensee shall have no further rights under any Commercial Option.

6.2 Licensee's Right to Terminate. Licensee may, upon ****' prior written notice to Licensor, terminate this Agreement for any reason. In exercising such termination right, Licensee may terminate this Agreement in its entirety or, if desired, Licensee may specify in the written notice that this Agreement is terminating only with respect to one or more of the Licensed Indications within the Field.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period; provided that, if termination is by Licensor as a result of Licensee's materially breaching Article 4, and if such breach only relates to one Licensed Indication within the Field, but not all, then Licensor's termination right shall only be with respect to such Licensed Indication with respect to which the breach related and not the remaining Licensed Indications; provided further that, if termination is by Licensor as a result of Licensee materially breaching Section 3.7.1, such cure period will be **** (in place of ****).

6.3.3 Notwithstanding the foregoing, if Licensee disputes in good faith that a payment is due or that such material breach exists, and gives Licensor written notice of such dispute within ****, in the case of payment, or ****, in the case of a material breach, following Licensee's receipt of Licensor's notice of default, then, Licensor may not terminate this Agreement until the dispute is resolved in accordance with Section 10.6 (and a payment is found to be due or a breach found to have occurred); provided that Licensor will be entitled to terminate this Agreement at the end of the original **** or **** cure period, as applicable, without waiting for resolution of the dispute in accordance with Section 10.6, if the breach by Licensee of this Agreement would cause Licensor to be in breach of the GSK Agreement or the Penn Agreement.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee or any of its Controlling Affiliates experiences any Trigger Event.

"Controlling Affiliate" means an Affiliate that directly or indirectly controls Licensee within the meaning of Section 1.1.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives Licensor's licensor a right of termination under the Penn Agreement and such licensor threatens to terminate the Penn Agreement, then, upon receipt of notice to such effect, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if the Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Controlling Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives Licensor's licensor a right of termination under the Penn Agreement and such licensor threatens to terminate the Penn Agreement, then, upon receipt of notice to such effect, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if the Sublicensee commences a Patent Challenge.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor or the Trustees of the University of Pennsylvania or SmithKline Beecham Corporation (or their successors under the Existing Licenses), including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The Commercial Options and licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.6.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Technology; provided that Licensee, its Affiliates, and Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.6.2 If a Commercial Option has been exercised with respect to a Licensed Indication, Licensee shall assign to Licensor, and Licensor shall accept, any or all sublicenses with respect to such Licensed Indication granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned, and Licensor will not be required to accept such sublicense; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not assigned to Licensor shall terminate;

6.6.3 If termination is by Licensee pursuant to Section 6.2, or by Licensor pursuant to Section 6.3, 6.4, or 6.5:

(a) Licensee shall grant, and hereby grants (effective only upon any such termination of this Agreement), to Licensor a non-exclusive, perpetual, irrevocable, worldwide, ****, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees during the term of this Agreement, to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication; provided that, if this Agreement is terminated only with respect to a specific Licensed Indication, the foregoing license granted to Licensor will not apply to products for use in any Licensed Indication for which, and for so long as, the license granted under Section 2.1.4 continues or any indication for which, and for so long as, a license has

been granted (and continues to be in effect) under the 2013 License Agreement;

(b) Licensee shall grant, and hereby grants (effective only upon any such termination of this Agreement), to Licensor an exclusive (even as to Licensee), worldwide, ****, transferable, perpetual license, with the right to grant sublicenses, under the Licensee Technology to make, have made, use, import, sell, and offer for sale Licensed Products, solely in the Field (or, if this Agreement is terminated only with respect to a specific Licensed Indication, such Licensed Indication in the Field). For this purpose, the "Licensee Technology" means Licensee's patents, Know-How, and other intellectual property that are improvements or modifications to or that are based on or derived in whole or in part from or that otherwise relate to any Licensed Technology to the extent such patents, Know-How, or other intellectual property pertains to (i) a recombinant adeno-associated virus vector or (ii) any expression construct provided by Licensor to Licensee as part of the Licensed Technology. To effectuate such license, upon any such termination of this Agreement, Licensee will promptly disclose to Licensor all Licensee Technology not already known to Licensor with respect to the Field or, if applicable, the Licensed Indication; and

(c) Licensee will transfer to Licensor ownership of any regulatory approvals then in Licensee's, its Affiliate's, or any Sublicensee's (to the extent the sublicense is not assigned pursuant to Section 6.6.2) name related to Licensed Products containing any expression construct provided by Licensor to Licensee as part of the Licensed Technology and notify the appropriate regulatory authorities and take any other action reasonably necessary to effect such transfer of ownership. If ownership of any such regulatory approval cannot be transferred to Licensor in any country, Licensee hereby grants (effective only upon any such termination of this Agreement) to Licensor a permanent, exclusive (even as to Licensee), and irrevocable right of access and reference to such regulatory approvals for Licensed Products containing any expression construct provided by Licensor to Licensee as part of the Licensed Technology in such country in the Field.

6.6.4****;

6.6.5 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.6.6 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

If termination is only with respect to a particular Licensed Indication for which Licensee exercised its Commercial Option, but not all Licensed Indications, then the provisions of this Section 6.6 shall only apply with respect to the terminated Licensed Indication(s), and this Agreement shall continue with respect to the non-terminated Licensed Indication(s).

6.7 Effects of Expiration Pursuant to Section 6.1.2. In the event of expiration pursuant to Section 6.1.2, each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.8 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2 (Retained Rights), 2.3 (Government Rights), 2.5 (Licensee's Improvements), 3.6 (if this Agreement expires and there are any continuing obligations under the Existing Licenses applicable to Licensee's continuing activities following expiration), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.7 (Reports and Records), Section 3.9 (Taxes and Withholding), Article 5 (Confidentiality), Article 6 (Term and Termination) except for Section 6.5, Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion and at its own expense. Subject to Section 7.1.3, following the first Grant Date under this Agreement, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that the Trustees of the University of Pennsylvania controls Prosecution of the Licensed Patents, with Licensor having certain rights to review.

Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of Licensor's licensors under the Existing Licenses, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under the Existing Licenses.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights or, if applicable, Excluded Rights) that may come to Licensee's attention. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 As between Licensor and Licensee, **** shall have the first right, but not the obligation, to prosecute any such infringement ****. In any action to enforce any of the Licensed Patents, ****, at the request and expense of ****, shall cooperate to the fullest extent reasonably possible, including in the event that, if **** is unable to initiate or prosecute such action solely in its own name, **** shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action.

7.2.3 Following the first Grant Date under this Agreement, if **** elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by any such Licensed Patent and such Licensed Patent is being infringed by another product **** (such infringement, the "**** Infringement"), **** shall have the second right, but not the obligation, to prosecute such **** Infringement with respect to such other product ****, at **** own expense. In any such action to enforce any of the Licensed Patents, ****, at the request and expense of ****, shall cooperate to the fullest extent reasonably possible, including in the event that, if **** is unable to initiate or prosecute such action solely in its own name, **** shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such **** Infringement, **** (a) shall not take any actions that would be detrimental to the Licensed Patents and **** rights with respect thereto **** **** and (b) shall not settle any such Competitive Infringement without the prior consent of Licensor.

7.2.4 Any recovery of damages by Licensor for any infringement prior to any Grant Date shall be ****. After the first Grant Date under this Agreement, (a) any recovery of damages by **** for any infringement other than a **** Infringement shall be ****; and (b) any recovery of damages by the Party undertaking enforcement or defense of a suit for **** Infringement shall be applied, as between Licensor and Licensee but subject to the obligations to Licensor's licensors under the Existing Licenses, first to reimburse each such Party for costs and expenses (including reasonable attorneys' fees and costs) incurred by such Party in connection with such suit, and the balance remaining, if any, from any such recovery shall be ****.

7.2.5 Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.2 are subject to the rights of Licensor's licensors under the Existing Licenses

(including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Licensor's obligations under this Agreement only apply to the extent that Licensor has any rights with respect to enforcing the Licensed Patents under the Existing Licenses. Furthermore, Licensee acknowledges the following:

7.2.5.1 All monies recovered upon the final judgment or settlement of any action with respect to **** Infringement will also need to be allocated to Licensor's licensors under the Existing Licenses (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

7.2.5.2 Licensor's licensors under the Existing Licenses retain the continuing right to intervene at their own expense and join Licensor or Licensee in any claim or suit for infringement of the Licensed Patents.

7.2.5.3 In any infringement of the Licensed Patents prosecuted by Licensors' licensors under the Existing Licenses, all financial recoveries will be ****.

7.2.5.4 In any infringement of the Licensed Patents prosecuted by Licensor's licensors under the Existing Licenses, **** agrees, at the request and expense of such licensors, to cooperate to the fullest extent reasonably possible, to the same extent as though **** were prosecuting such suit (as provided in this Section 7.2, including Section 7.2.2).

7.2.5.5 The written consent of **** under the **** will be required (a) for any decision that would have a materially adverse effect on the validity, scope of patent claims, or enforceability of the Licensed Patents and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on any of ****, or grants any rights to the Licensed Patents other than rights that ****.

7.3 Defense of Infringement Claims.

7.3.1 In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another.

7.3.2 To the extent Licensor takes any action, Licensor (or its licensors under the Existing Licenses) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, ****. Without Licensor's prior written permission, Licensee must not settle or compromise any such

suit in a manner that imposes any material obligations or restrictions on Licensor or any of its licensors under the Existing Licenses or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Execution Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Technology that would impact activities under this Agreement;

8.1.4 Licensor has provided to Licensee true, correct, and complete copies of the Existing Licenses;

8.1.5 To Licensor's knowledge, the Licensed Patents are solely owned by the Trustees of the University of Pennsylvania;

8.1.6 Licensor has not received any written notice from any of its licensors under the Existing Licenses informing Licensor that there are any actions, suits, proceedings, or arbitrations pending against such licensors relating to the Licensed Patents that would impact activities under this Agreement; and

8.1.7 To Licensor's knowledge, the Existing Licenses are in full force and effect and Licensor is not in breach of any provisions thereof.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Execution Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTIONS 8.1 AND 8.2, THE LICENSED TECHNOLOGY, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED BY EITHER PARTY TO THE OTHER UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, EXCEPT AS SET FORTH IN SECTIONS 8.1 AND 8.2, NEITHER PARTY MAKES ANY REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF ANY RIGHTS LICENSED BY EITHER PARTY TO THE OTHER, AND PROFITABILITY; OR (ii) THAT THE USE OF ANY RIGHTS GRANTED BY EITHER PARTY TO THE OTHER, INCLUDING ANY PRODUCTS RESULTING THEREFROM, WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH IN THIS AGREEMENT, NEITHER PARTY OR ANY OF SUCH PARTY'S DIRECT OR INDIRECT LICENSORS SHALL BE LIABLE TO THE OTHER PARTY, ITS SUCCESSORS OR ASSIGNS, OR ANY SUBLICENSEES OF EITHER PARTY, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF PRODUCTS ARISING THEREFROM; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its Affiliates, sublicensees, the licensors under the Existing Licenses, and their respective shareholders, members, partners, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims based

on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Technology or Licensed Products, including any claim by or on behalf of a ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its Affiliates and Sublicensees and their respective shareholders, members, partners, officers, trustees, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensor shall not be liable for claims based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner (i) that imposes any restrictions or obligations on the indemnified party (an "Indemnified Party") or, if Licensee is the Indemnifying Party, on Licensor's licensors under the Existing Licenses, without the other Party's prior written consent, (ii) if Licensee is the Indemnifying Party, that grants any rights to the Licensed Technology or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent, or (iii) if Licensor is the Indemnifying Party, that grants any rights that are inconsistent with those granted to Licensee under this Agreement without Licensee's prior written consent. The Indemnifying Party shall be permitted to control any

litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Within **** of the Execution Date, Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from **** of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensee acknowledges that Licensor's licensors under the Existing Licenses may review periodically the adequacy of the minimum amounts of insurance for each coverage required by the Existing Licenses, and Licensor reserves the right to require Licensee to adjust the limits set forth in this Section 8.5 to conform to any adjustments made by Licensor's licensors under the Existing Licenses. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Execution Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee; provided that, with Licensor's prior written consent, a Sublicensee may self-insure all or parts of the limits described above.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the Trustees of the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor; provided, however, that Licensee may assign this Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets to which this Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with notice of such assignment at least five business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. In addition, Licensee will provide Licensor with notice of any change of control (i.e., the acquisition by a person or group of "control" of Licensee, as defined in Section 1.1) of Licensee at least five business days prior to the effectiveness of such change of control. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Dimension Therapeutics, Inc.
840 Memorial Drive, 4th Floor
Cambridge, MA 02139
USA
Attn: President and CEO
Telephone: 617-231-2403
Facsimile: 617-231-2425

Either Party may change its official address upon written notice to the other Party.

General communications required under this Agreement (including notices under Sections 2.1, 2.4.2, 2.5.3, 2.6, 2.7.1, 3.6.2, 3.7, 4.2, 4.3, 4.4, 4.6, 7.1, 7.2, 7.3, 8.5, and 10.2 and notices of changes of address under this Section 10.4) may be sent by any of the means outlined in the first sentence of this Section 10.4 or a copy of the notice letter may be sent by electronic mail (without the requirement of a copy being sent by another means; provided that the receiving Party has confirmed receipt of such electronic mail); however, communications related to termination of the Penn Agreement, requests for disclosures of Confidential Information, breaches or termination of this Agreement, indemnification, and dispute resolution (including notices under Sections 2.8, 5.3, 6.2, 6.3, 6.4, 6.5, 8.4, and 10.6) must be sent by one of the means outlined in the first sentence of this Section 10.4.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations between senior executives of each Party with authority to resolve the dispute for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the

American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party

from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including the provisions of Section 6.16 and Exhibit B of the Series A SPA; provided that this Agreement does not supersede any other confidentiality agreements or obligations between the Parties, and, for the avoidance of doubt, this Agreement does not supersede the 2013 License Agreement. For clarity, the rights and obligations of the Parties under this Agreement are separate from and in addition to those under the 2013 License Agreement, and nothing in this Agreement shall be construed as modifying or restricting the rights of either Party under the 2013 License Agreement. This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full

force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections and exhibits in this Agreement are to Sections and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this Option and License Agreement to be executed by their duly authorized representatives.

REGENXBIO INC.

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

DIMENSION THERAPEUTICS, INC.

By: /s/ A. Jenkins
Name: A. Jenkins
Title: CEO Dimension

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Patents

App #	Title	Inventors	Nos.	Penn Docket #
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****

****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Licensed Know-How

[*To be completed pursuant to Section 2.1.3.4*]

CONFIDENTIAL TREATMENT REQUESTED

Exhibit C
Muscular Dystrophies

****

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CONFIDENTIAL TREATMENT REQUESTED

Exhibit D
Licensed Indications

Licensed Indication	Grant Date	Excluded Rights

CONFIDENTIAL TREATMENT REQUESTED

Exhibit E
Press Release

CONFIDENTIAL TREATMENT REQUESTED

Exhibit F
Additional Licensor Representation and Warranty

Except as provided in the Availability Notice, Licensor represents and warrants as of the Grant Date for the applicable Licensed Indication that: to Licensor's knowledge, with respect to the license granted under Section 2.1.4 for such Licensed Indication, Licensor does not Control (through ownership or Control pursuant to the Existing Licenses) any patent or patent application (other than the Licensed Patents set forth on Exhibit A) as of the Effective Date that would necessarily be infringed by Licensee's practice of the Licensed Patents set forth on Exhibit A in connection with using, importing selling, and offering for sale of adeno-associated virus vectors claimed in such Licensed Patents for such Licensed Indication. If it is determined, in accordance with the procedure of this Exhibit F, that Licensor Controls (through ownership or Control pursuant to the Existing Licenses) a patent or patent application (other than the Licensed Patents) as of the Effective Date that would necessarily be infringed by Licensee's practice of the Licensed Patents set forth on Exhibit A in connection with using, importing selling, and offering for sale of adeno-associated virus vectors claimed in such Licensed Patents for such Licensed Indication, then Licensor shall include the applicable patent or patent application as a "Licensed Patent" hereunder with respect to such Licensed Indication but solely to the extent of the claim(s) that would necessarily be infringed by such practice of such Licensed Patents by Licensee, which inclusion shall be Licensee's sole remedy.

At any time after the Grant Date for such Licensed Indication and during the term of this Agreement for such Licensed Indication, Licensee may notify Licensor in writing of any such patent or patent application that Licensee believes should be included as a "Licensed Patent" pursuant to this Exhibit F. Such written notice shall identify the relevant patent or patent application and relevant claim(s) and shall explain briefly why Licensee, in good faith, believes it should be included as a "Licensed Patent." If Licensor does not agree with Licensee, Licensor shall have **** following Licensor's receipt of Licensee's written notice to notify Licensee that Licensor disputes the inclusion of such patent or patent application or the scope of the remedy; in which event, such dispute will be resolved in accordance with Section 10.6 of the Agreement. Upon the Parties' agreement (or a resolution, in favor of Licensee, of the dispute pursuant to Section 10.6), the applicable claim(s) of the applicable patent or patent application will be deemed a "Licensed Patent" hereunder with respect to the applicable Licensed Indication. For the avoidance of doubt, Licensor makes no representation or warranty under this Exhibit F as to any claim of (a) a patent or patent application covering Manufacturing Technology or (b) a patent or patent application that is not Controlled by Licensor pursuant to the Existing Licenses or pursuant to Licensor's ownership thereof, and Licensee acknowledges that (i) Manufacturing Technology claims of any patents or patent applications or (ii) claims of any patents or patent applications not Controlled by Licensor pursuant to the Existing Licenses or pursuant to Licensor's ownership thereof will not be added as "Licensed Patents" pursuant to the procedure set forth in this Exhibit F.

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CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of March, 5th, 2014 (the "Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006, USA ("Licensor"), and Laboratorios Del Dr. Esteve, S.A., a corporation organized under the laws of Spain, with offices at Av. Mare de Déu de Montserrat, 221, 08041 Barcelona, Spain ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to adeno-associated virus serotype 9; and

WHEREAS, Licensee desires to obtain a non-exclusive license under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAV9" means the recombinant adeno-associated virus serotype 9 vector with the specified sequence set forth in GenBank ****

1.2 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.3 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.4 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.6 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

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1.4.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.4.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.4.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.4.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.4.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.5 "Disclosing Party" has the meaning set forth in Section 5.1.

1.6 "Domain Antibody" ****.

1.7 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.8 "Field" means the treatment of the Sanfilippo A (MPSIII Type A) in human beings by in vivo administration.

1.9 "Licensed Patents" means, to the extent they cover AAV9, (a) all United States patents and patent applications listed in Exhibit A, (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications, and (c) any additional claims of patents and patent applications as required pursuant to Section 8.1.5.

1.10 "Licensed Product" means (a) any product comprising an expression construct encoding Licensee's Gene packaged using the AAV9 capsid protein that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import, including products manufactured by a process that would infringe at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service with respect to the administration of any product comprising an expression construct encoding Licensee's Gene packaged using the AAV9 capsid protein to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe at least one Valid Claim in the country of sale.

1.11 "Licensee's Gene" means Licensee's proprietary codon-optimized Sulfamidase gene described on Exhibit B and functional variants thereof.

1.12 "Licensor's Knowledge" means the actual knowledge of Kenneth Mills, Vit Vasista, and Sara Berl.

1.13 "Muscular Dystrophy" ****.

1.14 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.15 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.16 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.17 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

1.18 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents.

1.19 "Receiving Party" has the meaning set forth in Section 5.1.

1.20 "Research Field" means Licensee's internal research and pre-clinical development of AAV9 agents that deliver any DNA, RNA, or other sequence or reagent, other than those expressing Domain Antibodies, for the prevention or treatment of diseases in humans. "Research Field" specifically excludes (without limitation) (i) all human clinical trial use, diagnostic use, therapeutic use, and prophylactic use, (ii) any commercial uses, and (iii) any use in the fields described in Section 2.2.1, 2.2.3, or 2.2.4.

1.21 "Retained Rights" has the meaning set forth in Section 2.2.

1.22 "Sublicensee" means any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement.

1.23 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.24 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee a non-exclusive, sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Patents (a) to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field, including, for the avoidance of doubt, the right to conduct research and development, and (b) to practice the Licensed Patents in the Research Field (including the limited right of Licensee to make and use research reagents solely for use by Licensee in the Research Field).

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1 or as provided in Section 8.1.5, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise, whether any such intellectual property dominates or is dominated by the Licensed Patents. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes, inside or outside of the Field or the Research Field. Without limiting the foregoing, Licensee acknowledges and agrees to the following rights retained by Licensor and its direct and indirect licensors

(individually and collectively, the "Retained Rights"), whether inside or outside the Field or the Research Field:

2.2.1 Notwithstanding anything in this Agreement to the contrary, the rights and licenses granted in Section 2.1 shall not include any right (and Licensor and its direct and indirect licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including AAV9.

2.2.2 Notwithstanding anything in this Agreement to the contrary, Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable tight under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including AAV9; and

(b) A non-exclusive right for Licensor's direct and indirect licensors (which right is sublicensable by such licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 Notwithstanding anything in this Agreement to the contrary, the rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer for sale, and import products for the treatment of (a) all forms of Muscular Dystrophy; (b) congestive heart failure suffered by Muscular Dystrophy patients; and (c) any and all cardiovascular diseases by delivery of any or all of genes encoding I-1c and Serca2a and creatine kinase.

2.2.4 Notwithstanding anything in this Agreement to the contrary, the rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that (i) for clarity, such exclusive rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field, though Licensor retains the non-exclusive right to do so; and (ii) the license granted in Section 2.1(b) includes the limited right to make and use research reagents solely for use by Licensee in the Research Field.

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Section 2.1(a) does include the right to provide the service of the administration of Licensed Products to patients.

2.2.5 Notwithstanding anything in this Agreement to the contrary, Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties.

2.2.6 Notwithstanding anything to the contrary in this Agreement, the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational, research, and other non-commercial purposes.

For the avoidance of doubt, except as specifically provided in this Agreement (including in Section 2.5), the retention of rights by Licensor in the Research Field under this Section 2.2 shall not be deemed a grant by Licensee to Licensor of rights to use Licensee's internal research and pre-clinical development; provided that Licensee acknowledges that nothing in this Agreement prohibits Licensor or its other licensees from conducting research, pre-clinical development, or other activities in the same fields as Licensee.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.4 Sublicensing.

2.4.1 The research license granted pursuant to Section 2.1(b) is sublicensable by Licensee only to the Universidad Autonoma de Barcelona and to Licensee's Affiliates; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.42). The license granted pursuant to Section 2.1(a) is sublicensable by Licensee to any Affiliates or Third Parties (including the Universidad Autonoma de Barcelona); provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee; ****. Licensor must receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with Licensor's direct and indirect licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or any of its direct or indirect licensors') ability to ensure compliance with this Agreement; provided that, if any of Licensor's direct or indirect licensors require a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy. If Licensor's direct or indirect licensors object to the initially provided redacted version of a sublicense, Licensee may have one opportunity to provide a less redacted version; provided that, if a delay in providing a complete, unredacted copy of the sublicense would result in Licensor being in breach of its direct or indirect license agreements, such opportunity will not apply, and Licensee will immediately provide a complete, unredacted copy of the sublicense.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.5 Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with AAV9, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that, during the term of this Agreement, Licensor shall have no right, under the license in this Section 2.5.1(b), to practice the Licensed Back Improvements in the Field.

2.5.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.5.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement by Licensor or its direct or indirect licensors or licensees.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor $500,000 within **** of the Effective Date.

3.2 Annual Maintenance Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor on-going annual maintenance fees of **** on each anniversary of the Effective Date.

3.3 Milestone Fees. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor the following milestone payments on a per-Licensed Product basis:

Milestone	Milestone Payment
1. First treatment of human subject in a clinical trial (*i.e.*, first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. NDA submission in the United States	****
4. First NDA submission in the European Union	****
5. NDA approval in the United States	****
6. First NDA approval in the European Union	****
Total:	$8.5 million

For clarity, the milestone payments set forth in this Section 3.3 are payable **** with respect to each Licensed Product that achieves the milestone event, ****. To the extent that either of the two development milestones in this Section 3.3 (*i.e.*, first treatment of human subject in a clinical trial or first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of either NDA submission milestone, then, upon the achievement of either of such NDA submission milestones, the preceding unpaid development milestone payments shall be made in addition to the payment corresponding to the NDA submission milestone that has been achieved.

3.4 Royalties. In further consideration of the license granted to Licensee under Section 2.1, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products, subject to the reductions in royalty rates set forth in Section 3.4.1:

Cumulative Annual Net Sales of all Licensed Products Worldwide	Royalty Percentage

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Portion of Net Sales less than $300 million	****
Portion of Net Sales between (and including) $300 million through (and including) $600 million	****
Portion of Net Sales greater than $600 million	****

3.4.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C * (A / (A+B)))

Where
R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty ****

Example Calculation:

assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R = (**** - ****) * (**** / (****+****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** — **** = ****

If an Affiliate of Licensee or any Sublicensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product, such Affiliate or Sublicensee **** the royalty owed with respect to such Affiliate's or Sublicensee's Net Sales for the given Licensed Product in the same manner as set forth above with respect to **** by Licensee.

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee, its Affiliates, or any Sublicensees if there were no royalties due to Third Parties.

3.4.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end

on a country-by-country basis when ****.

3.5 Sublicense Fees.

3.5.1 In further consideration of the license granted to Licensee under Section 2.1, Licensee will pay Licensor **** of any sublicense fees (including upfront payments and milestone payments and including any equity consideration received by Licensee or any equity investment in Licensee) received by Licensee for the Licensed Patents from any Sublicensee or from any person or entity granted any option to obtain a sublicense.

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee corresponding directly to the development of Licensed Products pursuant to a specific agreement; and

(b) Any and all amounts paid to Licensee by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement.

3.5.3 If Licensee receives sublicense fees from Sublicensees or from any person or entity granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.5 (a) in the form of the non-cash consideration received by Licensee or (b) a cash payment determined based on the fair market value of such non-cash consideration.

3.6 Reports and Records.

3.6.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.15, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.4.1;

(f) Royalties owed to Licensor, listed by category, and

(g) The computations for any applicable currency conversions.

3.6.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.6.1.

3.6.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3. In addition, within **** after the receipt of sublicense fees from any Sublicensee as described in Section 3.5, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.5.

3.6.4 All financial reports under this Section 3.6 will be certified by the chief financial officer of Licensee.

3.6.5 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or its direct or indirect licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and or its direct or indirect licensors and accountants in connection with the review or audit.

3.7 Currency, Interest.

3.7.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.7.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.6.

3.7.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.8 Taxes and Withholding.

3.8.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.8.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.8.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****.

4.2 Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its direct and indirect licensors.

4.4 Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements.

5.2.1 Following the Effective Date, the Parties agree they will release a joint press release in the form attached hereto as Exhibit D. Except as provided in Section 5.2.1, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval

by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or AAV9 program that have been publicly disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with any of Licensor's direct and indirect licensors of the Licensed Patents. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder, provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, or become abandoned or unenforceable in all countries of the world.

6.2 Licensee's Right to Terminate. Licensee may, upon three months' prior written notice to Licensor, terminate this Agreement for any reason, with or without cause.

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** after receipt of a written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period; provided that the cure period shall be **** for the failure of Licensee to make any payment due pursuant to Section 3.1.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after receipt of written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee or any of its Affiliates experiences any Trigger Event.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives Licensor's licensor a right of termination under the Penn Agreement, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives Licensor's licensor a right of termination under the Penn Agreement, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee commences a Patent Challenge.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor, the University of Pennsylvania, or any direct or indirect licensor of Licensor, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.6.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Patents (including ceasing to make and use research reagents under the Licensed Patents); provided that Licensee shall have the right to continue to sell its existing inventories of Licensed Products and to use any previously made research reagents, in each case, for a period not to exceed **** after the effective date of such termination;

6.6.2 At Licensor's request, Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not requested to be assigned to Licensor shall terminate;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5, Licensee shall grant, and hereby grants to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the

subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.6.4 Licensee shall pay all monies then-owed to Licensor under this Agreement; and

6.6.5 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2, (Retained Rights), 2.3 (Government Rights), 2.5 (Improvements), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.6 (Reports and Records), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, but subject to any obligations of Licensor to its direct and indirect licensors of the Licensed Patents, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention.

7.2.2 As between Licensor and Licensee, but subject to any obligations of Licensor to its direct and indirect licensors of the Licensed Patents, Licensor shall have the sole right, but not the obligation, to prosecute any such infringement at its **** recovered in connection therewith. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. Nothing in this Agreement obligates Licensor to bring or prosecute lawsuits against Third Parties for infringement of any Licensed Patents.

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7.2.3 Licensee shall have no right to undertake prosecution of any such infringement without Licensor's prior written consent.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another. To the extent Licensor takes any action, Licensor (or its direct or indirect licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or any of its direct or indirect licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's Knowledge, threatened against Licensor relating to the Licensed Patents that would impact activities under this Agreement;

8.1.4 To Licensor's Knowledge, Licensor has not received any written notice from any of its direct or indirect licensors informing Licensor that there are any actions, suits, proceedings, or arbitrations pending against Licensor's direct or indirect licensors relating to the Licensed Patents that would impact activities under this Agreement; and

8.1.5 To Licensor's Knowledge, Licensor does not Control as of the Effective Date any patent or patent application (other than the Licensed Patents (as defined in Section 1.9(a)) that has a claim directed to the use of the AAV9 capsid protein for use in the Field. If it is determined, in accordance with the procedure of this Section 8.1.5, that Licensor has breached the representation and warranty in this Section 8.1.5, then Licensee's sole remedy for such breach shall be the inclusion of the applicable patent or patent application as a "Licensed Patent" hereunder but solely to the extent of the claim(s) that is directed to the use of the AAV9 capsid protein for use in the Field; provided that Licensee shall be required to satisfy any obligations (including confidentiality agreements, obligations of indemnification of Licensor's direct and indirect licensors, and reporting obligations; but excluding any financial obligations) owed to

any Third Parties in connection with such rights to the same extent as Licensor has agreed. At any time during the term of this Agreement, Licensee may notify Licensor in writing of Licensor's breach of this Section 8.1.5. Such written notice shall identify the relevant patent or patent application and relevant claim(s) and shall explain briefly why Licensee, in good faith, believes it should be included as a "Licensed Patent." The Parties shall discuss whether any claims should be included in the "Licensed Patents," and Licensor shall disclose to Licensee any obligations that Licensee would be required to satisfy if such claims were to be added; provided that (a) no claims shall be included in the "Licensed Patents" pursuant to this Section 8.1.5, if Licensee elects not to include them (but Licensee acknowledges that, in making such election, Licensee shall be electing not to seek its sole remedy for any breach of the representation and warranty in this Section 8.1.5); and (b) Licensor has **** following Licensor's receipt of Licensee's written notice to dispute such breach or the scope of the remedy to resolve such breach; in which event, such dispute will be resolved in accordance with Section 10.6. Upon the Parties' agreement (or a resolution, in favor of Licensee, of the dispute pursuant to Section 10.6), the applicable claim(s) of the applicable patent or patent application will be deemed a "Licensed Patent" hereunder. For the avoidance of doubt, Licensor makes no representation or warranty under this Section 8.1.5 as to any claim of a patent or patent application covering the manufacture of AAV9, and Licensee acknowledges that manufacturing claims of any patents or patent applications will not be added as "Licensed Patents" pursuant to the procedure set forth in this Section 8.1.5. For the purpose of this Section 8.1.5, "Control" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent or patent application on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any Third Party.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS

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AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR OR ANY OF LICENSOR'S DIRECT OR INDIRECT LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its direct and indirect licensors of the Licensed Patents, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that

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was **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents or Licensed Products, including any claim by or on ****.

8.4.2 Indemnification Procedure. Licensee, as an indemnifying party (a "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on Licensor, its direct or indirect licensors, or any indemnified party (a "Indemnified Party") without Licensor's prior written consent or that grants any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights which such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit

per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of "****" or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor, provided, however, that Licensee may assign this Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with notice of such assignment at least five business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

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10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Laboratorios Del Dr. Esteve, S.A.,
Av. Mare de Diu de Montserrat, 221
08041 Barcelona
Spain
Attn: Chief Executive Officer
Telephone: +34 93 446 6179
Facsimile: +34 93 433 0072

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of Delaware with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

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10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent

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irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral. All "Confidential Information" (a) disclosed by Licensor to Ysios Capital (and then disclosed by Ysios Capital to Licensee) pursuant to that certain Mutual Non-Disclosure Agreement between Licensor and Ysios Capital dated June 17, 2013, (b) disclosed by Licensor to Licensee pursuant to any agreements between Licensor and Licensee, or (c) disclosed by Licensor to YESgene, S.L. (and then disclosed by YESgene, S.L. to Licensee) pursuant to any agreements between Licensor and YESgene, S.L., in each case, shall be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.4). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marling. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law to enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential

importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections and exhibits in this Agreement are to Sections and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth Mills

Name: Kenneth Mills

Title: CEO

LABORATORIOS DEL DR. ESTEVE, S.A.

By: /s/ Albert Esteve

Name: Albert Esteve

Title: CEO

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A

Licensed Patents

Application #	Patent #	Filing Date	Country	Status
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****		****	****	****

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CONFIDENTIAL TREATMENT REQUESTED

Exhibit B

Licensee's Gene

Nucleotide sequence of codon optimized human sulfamidase:

****

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CONFIDENTIAL TREATMENT REQUESTED

Exhibit C

Muscular Dystrophies

****

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CONFIDENTIAL TREATMENT REQUESTED

Exhibit D

Press Release

CONFIDENTIAL TREATMENT REQUESTED

REGENX BIOSCIENCES AND ESTEVE ENTER INTO LICENSE AGREEMENT FOR DEVELOPMENT OF TREATMENTS FOR SERIOUS, RARE LYSOSOMAL STORAGE DISORDER USING NAV® rAAV9 VECTORS

WASHINGTON, DC and BARCELONA, SPAIN February 28, 2014 – REGENX Biosciences, LLC (REGENX) and Laboratorios Dr. Esteve, S.A. (ESTEVE) announce that they have entered into an agreement enabling the development and commercialization of products to treat mucopolysaccharidosis type IIIA (MPS MA or Sanfilippo syndrome Type A) using NAV rAAV9.

Under the terms of the Agreement, REGENX granted ESTEVE a non-exclusive worldwide license, with rights to sublicense, to REGENX's NAV rAAV9 vectors for treatment of MPS MA in humans. In return for these rights, REGENX receives payments in the form of an up-front payment, certain milestone fees and royalties on net sales of products incorporating NAV rAAV9.

"We believe this license agreement will further advance the development of NAV-based gene delivery treatments for patients with MPS MA," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to further our mission of enabling the development of successful new AAV therapeutics by collaborating with the ESTEVE team."

"We are happy to be working with REGENX and believe the signing of this agreement enables ESTEVE to advance the development of our gene therapeutic for Sanfilippo A towards clinical trials," said Albert Esteve, CEO of ESTEVE. "We share the same mission as ReGenX—the development of innovative products to meet patient needs—and that is why this is one of our highest priority projects today."

About MPS HI A (SanfilOpo syndrome Type A)

Sanfilippo syndrome is a devastating disease that leads to progressive and significant deterioration in mental status of children who rarely live beyond their twenties. The Sanfilippo syndrome Type HIA is a lysosomal storage disease caused by the loss of the activity of the enzyme sulfamidase. It affects approximately 1 in 100,000 births and is often diagnosed only once symptoms have begun to appear.

About REGENX Biosciences

REGENX Biosciences (www.regenxbio.com) is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary NAV vector technology platform, for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, Lysogene, and Audentes Therapeutics. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding REGENX, please visit www.regenxbio.com.

About ESTEVE

ESTEVE (www.esteve.com) is a leading pharmaceutical chemical group based in Barcelona, Spain. Since it was founded in 1929, ESTEVE has been firmly committed to excellence in healthcare, dedicating efforts to innovative R&D of new medicines for unmet medical needs and focusing on high science and evidence-based research. ESTEVE has a strong partnership approach to drug discovery, development and commercialization. The company works both independently and in collaboration to bring new, differentiated best-in-class treatments to patients who need them. The company currently employs 2,300 professionals and has subsidiaries and production facilities in several European countries, USA, China and Mexico.

Contacts:

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ESTEVE

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For media enquiries: Angels Valls, Director of ESTEVE Corporate Communications, Tel. +34 93 446 6286, avalls@esteve.es

CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of December 2, 2013 ("Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 USA ("Licensor"), and Lysogene Société par Actions Simplifiée, a simplified joint stock company organized under the laws of France, with offices at 52 rue de la Boetie, 75008 - Paris, France ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain Licensed Patents (as defined herein) pertaining to adeno-associated virus serotype rh10; and

WHEREAS, Licensee desires to obtain an exclusive license under the Licensed Patents under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAVrh10" means (a) the recombinant adeno-associated virus serotype rh10 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype rh10 vector that are covered by the claims of the Licensed Patents.

1.2 "Affiliate" means any legal entity directly or indirectly controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity.

1.3 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.4 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.6 will be deemed the Confidential Information of both Parties, regardless of whether, such information is marked or identified as confidential. In addition, information provided to Licensee pursuant to the provisions of Section 7.1 will be deemed the Confidential Information of Licensor, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing,

Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.4.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.4.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.4.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.4.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.4.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.5 "Disclosing Party" has the meaning set forth in Section 5.1.

1.6 "Domain Antibody" ****.

1.7 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.8 "Field" means the treatment of the Sanfilippo syndrome Type A (MPSIII Type A) in humans by *in vivo* gene therapy using AAVrh10.

1.9 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.10 "Licensed Patents" means (a) all United States patents and patent applications listed in Exhibit A, and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications.

1.11 "Licensed Product" means (a) any AAVrh10 product that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which -product, in the absence of the license grafted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or imp*, including products manufactured by a process that would infringe or is covered by at least one Valid Claim in the country of manufacture, use, sale, offer for sale, or import; or (b) any service with respect to the

administration of AAVrh10 product to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim in the country of sale.

1.12 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.13 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any 'Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.14 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.15 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.16 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, and interferences.

1.17 "Receiving Party" has the meaning set forth in Section 5.1.

1.18 "Retained Rights" has the meaning set forth in Section 2.2.

1.19 "Sublicensee" means any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement.

1.20 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.21 "Valid Claim" means a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or a claim of a pending patent application included within the Licensed Patents, which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANT

2.1 License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor hereby grants to Licensee an exclusive (even as to Licensor), sublicensable (as provided in Section 2.4 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license, under the Licensed Patents to make, have made, use, import, sell, and offer for sale Licensed Products solely in the Field, including, for the avoidance of doubt, the right to conduct research and development.

2.2 Retained Rights. Except for the rights and licenses specified in Section 2.1, no license or 1 other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise, whether any such intellectual property dominates or is dominated by the Licensed Patents. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes outside of the Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees to the following rights retained by Licensor and its direct and indirect licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Field:

2.2.1 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor and its direct and indirect licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including AAVrh10.

2.2.2 Licensor and its direct and indirect licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including AAVrh10; and

(b) A non-exclusive right for Licensor's direct and indirect licensors (which right is sublicensable by such licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such licensors' discovery research efforts with non-profit organizations and collaborators.

2.2.3 The rights and licenses granted in Section 2.1 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that, for clarity, such rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Field; or

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Section 2.1 does include the right to provide the service of the administration of Licensed Products to patients.

2.2.4 Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties; provided that such development rights granted by Licensor shall not include the right to conduct clinical trials in humans in the Field or any rights to sell products in the Field.

2.2.5 The University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational, research, and other non-commercial purposes.

2.3 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grant hereunder is expressly subject to all applicable United States government rights, including any applicable requirement that products resulting from such intellectual property sold in the United States must be substantially manufactured in the United States.

2.4 Sublicensing.

2.4.1 The license granted pursuant to Section 2.1 is sublicensable by Licensee to any Affiliates or Third Parties; provided that any such sublicense must comply with the provisions of this Section 2.4 (including Section 2.4.2).

2.4.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may grant sublicenses **** but only pursuant to a written sublicense agreement with the Sublicensee. Licensor must

receive written notice as soon as practicable following execution of any such sublicenses.

(b) In each sublicense agreement, the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed and must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(c) The official language of any sublicense agreement shall be English.

(d) Within **** after entering into a sublicense, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with Licensor's direct and indirect licensors. The copy of the sublicense may be redacted to exclude confidential information of the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or any of its direct or indirect licensors') ability to ensure compliance with this Agreement; provided that, if any of Licensor's direct or indirect licensors require a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(e) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in 'this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.5 Improvements.

2.5.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license:

(a) to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights, and

(b) to practice the Licensed Back Improvements (and any intellectual property rights with respect thereto) in connection with AAVrh10, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that, during the term of this Agreement, Licensor shall have no right, under the license in this Section 2.5.1(b), to practice the Licensed Back Improvements in the Field.

2.5.2 For purposes of this Agreement, "Licensed Back Improvements" means any patentable modifications or improvements developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents.

2.5.3 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed description of or access to such Licensed Back Improvement to permit the practice of any such invention or improvement by Licensor or its direct or indirect licensors or licensees as provided for in Section 2.5.1 above.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor an initial fee of $500,000, of which ****will be paid upon the Effective Date and ****will be paid upon the **** of the ****provided that such **** portion of the initial fee will be immediately payable upon any termination of this Agreement prior to the **** of the Effective Date.

3.2 Annual Maintenance Fee. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor on-going annual maintenance fees of **** on each anniversary of the Effective Date.

3.3 Milestone Fees. In consideration of the license granted to Licensee under Section 2.1, Licensee shall pay Licensor the following milestone payments:

	Milestone	Milestone Payment
1.	First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
2.	NDA submission in the United States	****
3.	NDA submission in the European Union	****
4.	NDA approval in the United States	****
5.	NDA approval in the European Union	****
6.	First rolling 12-month period during which aggregate Net Sales are greater than $50.0 million	****

For clarity, the milestone payments set forth in this Section 3.3 are payable **** with respect to the first Licensed Product that achieves the milestone event, ****. To the extent that the development milestone in this Section 3.3 (i.e., first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of either NDA submission milestone, then, upon the achievement of either of such NDA submission milestones, the preceding unpaid development milestone payment shall be made in addition to the payment corresponding to the NDA submission milestone that has been achieved.

3.4 Royalties. In further consideration of the license granted to Licensee under Section 2.1, Licensee shall pay to Licensor the following royalties based upon Net Sales of Licensed Products:

Cumulative Annual Net Sales of all Licensed Products Worldwide	**Royalty Percentage**
Portion of Net Sales less than $300 million	****
Portion of Net Sales between (and including) $300 million through (and including) $600 million	****
Portion of Net Sales greater than $600 million	****

By way of example, if the cumulative annual Net Sales of all Licensed Product worldwide equal $700,000,000 in a calendar year, the royalty rate on the first $299,999,999 of such Net Sales will be ****, the royalty rate on the next $300,000,002 of such Net Sales will be ****, and the royalty rate on the remaining $99,999,999 of such Net Sales will be ****.

3.4.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C * (A / (A+B)))

Where

R = reduction of Licensor royalty,
A = unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****

Example Calculation:

assume:

i) all Third Party royalties = ****

ii) unreduced Licensor royalty =****

iii) projected total royalty = ****

R = (****–****) * (**** / (**** + ****))
R = **** * ****
R = ****

Licensor Stacked Royalty = **** – **** = ****

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.4.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.4 on the Net Sales of Licensed Products in a given country will end on a country-by-country basis when ****.

3.5 Sublicense Fees.

3.5.1 In further consideration of the license granted to Licensee under Section 2.1, Licensee will pay Licensor a percentage of any sublicense fees (including upfront payments and milestone payments) received by Licensee for the Licensed Patents from any Sublicensee or from any person or entity granted any option to obtain a sublicense. The applicable percentage due to Licensor for each sublicense (or option) shall be as follows:

Event	Sublicense Fee Rate
If sublicensed (or optioned) on or before the third anniversary of the Effective Date	****
If sublicensed (or optioned) after the third anniversary of the Effective Date	****

For the avoidance of doubt, with respect to an option to obtain a sublicense, if a sublicense is later granted as a result of the exercise of such option, the sublicense fees applicable to such sublicense will be determined by reference to the date the original option was granted, not the date the actual sublicense was granted.

3.5.2 With respect to the obligations under this Section 3.5, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee corresponding directly to the development of Licensed Products pursuant to a specific agreement;

(b) Consideration received for the purchase of an equity interest in Licensee at fair market value or in the form of loans at commercially reasonable rates of interest; and

(c) Any and all amounts paid to Licensee by a Sublicensee as royalties on sales of Licensed Product sold by the Sublicensee under a sublicense agreement.

3.5.3 To the extent Licensee receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.3, then the amount of the payment

made to Licensor under such Section 3.3 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.5; instead, the amounts due under this Section 3.5 shall be calculated by applying the applicable sublicense fee rate set forth in Section 3.5.1 above to the sublicense fees received by Licensee from such Third Party after deducting the amount of the payment under Section 3.3. By way of example,

Assume: (a) Sublicensee was granted a sublicense prior to the first anniversary of the Effective Date,

(b) Sublicensee achieves an NDA submission in the United States, and

(c) Sublicensee pays Licensee **** upon the achievement of such event.

Then, Licensee would owe Licensor ****, calculated as follows:

(i) **** pursuant to Section 3.3 (based on milestone event #2), and

(ii) **** pursuant to this Section 3.5, which is calculated as follows:

(x) the sublicense fee rate of **** multiplied by

(y) **** (which is determined by subtracting the **** milestone payment under Section 3.3 from the **** payment received from Sublicensee).

Alternatively: (A) If Sublicensee paid Licensee nothing upon the achievement of an NDA submission in the United States, then Licensee would owe Licensor (1) **** pursuant to Section 3.3 (based on milestone event #2) and (2) no amounts under this Section 3.5; and

(B) If Sublicensee paid Licensee **** upon the achievement of an NDA submission in the United States, then Licensee would owe Licensor (1) **** pursuant to Section 3.3 (based on milestone event #2) and (2) no amounts under this Section 3.5.

3.5.4 If Licensee receives sublicense fees from Sublicensees or from any person or entity granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.5 (a) in the form of the non-cash consideration received by Licensee or (b) a cash payment determined based on the fair market value of such non-cash consideration.

3.6 Reports and Records.

3.6.1 Licensee must deliver to Licensor within **** after the end' of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.13, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.4.1;

(f) Royalties owed to Licensor, listed by category; and

(g) The computations for any applicable currency conversions.

3.6.2 Licensee shall pay the royalties due under Section 3.4 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.6.1.

3.6.3 Within **** after the occurrence of a milestone event described in Section 3.3, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.3. In addition, within **** after the receipt of sublicense fees from any Sublicensee as described in Section 3.5, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.5.

3.6.4 All financial reports under this Section 3.6 will be certified by the chief financial officer of Licensee.

3.6.5 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or its direct or indirect licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. Licensor will promptly reimburse to License the amount of any overpayment determined by the review or audit, but no accrued interest will apply. If the review or audit determines that Licensee has underpaid any payment

by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and or its direct or indirect licensors and accountants in connection with the review or audit.

3.7 Currency, Interest.

3.7.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.7.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the Wall Street Journal, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.6.

3.7.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.8 Taxes and Withholding.

3.8.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.8.2. At the request of Licensee, Licensor will give Licensee such reasonable assistance, which will include the provision of documentation as may be ‘required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.8.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor’s gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell Licensed Products in the Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****

****. Without limiting the foregoing, Licensee will achieve first treatment in a Phase 3 Clinical Trial (i.e., first patient, first dose) by no later than ****. Licensee will notify Licensor in writing as soon as Licensee believes in good faith that Licensee will not be able to achieve the foregoing milestone by the relevant deadline date, and, upon the payment to Licensor of **** within ****of the original deadline date, the deadline date for such milestone set will be extended for ****from the original deadline date; provided that Licensee will only be ****.

****.

4.2 Reporting. Within **** after the Effective Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with its direct and indirect licensors.

4.4 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.5.3.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions at least as stringent as those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations at least as stringent as those contained under this Agreement.

5.2 Public Announcements. The Parties agree they will release a joint press release in the form attached hereto as Exhibit B. Any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms no less stringent than those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with any of Licensor's direct and indirect licensors of the Licensed Patents. In the event l that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information 1 or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the

Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim to expire, lapse, or become abandoned or unenforceable in all countries of the world.

6.2 Licensee's Right to Terminate. Licensee may, upon 90 days' prior written notice to Licensor, terminate this Agreement for any reason, with or without cause; provided that, if such termination notice is sent prior to the **** of the Effective Date, such termination notice shall be accompanied by Licensee's payment of **** in satisfaction of the remainder of the initial fee under Section 3.1.

6.3 Termination for Breach.

6.3.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.3.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4 Termination for Insolvency.

6.4.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee or any of its Affiliates experiences any Trigger Event.

6.4.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee experiences any Trigger Event; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee experiences any Trigger Event. In addition, if the Sublicensee's experiencing of a Trigger Event gives Licensor's

licensor a right of termination under the Penn Agreement, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee experiences any Trigger Event.

6.4.3 For purposes of this Section 6.4, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.4.3(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.4.3(b) through (d) above.

6.5 Patent Challenge.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge.

6.5.2 Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives Licensor's licensor a right of termination under the Penn Agreement, then, upon receipt of such notice, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee commences a Patent Challenge.

6.5.3 For purposes of this Section 6.5, "Patent Challenge" means any action against Licensor, the University of Pennsylvania, or any direct or indirect licensor of Licensor, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof

6.6 Effects of Termination. The effect of termination by Licensee pursuant to Section 6.2, by either Party, as applicable, under Section 6.3, or by Licensor pursuant to Section 6.4 or 6.5 shall be as follows:

6.6.1 The licenses granted by Licensor hereunder shall terminate, and Licensee and its Affiliates shall cease to make, have made, use, import, sell, and offer for sale all Licensed Products and shall cease to otherwise practice the Licensed Patents; provided that Licensee, its Affiliates, and Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.6.2 All sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee shall be assigned to Licensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned; and (ii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement;

6.6.3 If termination is by Licensee pursuant to Section 6.2 or by Licensor pursuant to Section 6.3, 6.4, or 6.5, Licensee shall grant, and hereby grants to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor for the research, development, and commercialization of products in any therapeutic indication;

6.6.4 Licensee shall pay all monies then-owed to Licensor under this Agreement, and, if applicable, Licensor shall pay all monies then-owed to Licensee under this Agreement;

6.6.5 The Parties acknowledge and agree that, if the GSK Agreement is terminated as described in Section 6.5 of the GSK Agreement, then, as provided in Section 6.5.2 thereof, Licensor will assign this Agreement to the licensor of the GSK Agreement to the extent this Agreement is related solely to the rights and products licensed to Licensor under the GSK Agreement; and

6.6.6 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations.

6.7 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement, and Licensor's obligation to pay all monies due and owed to Licensee under this Agreement, in each case, which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Section 2.2, (Retained Rights), 2.3 (Government Rights), 2.5 (Improvements), 3.1 (Initial Fee), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.6 (Reports and Records), Article 5 (Confidentiality), Article 6 (Term and Termination), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional

Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion. Subject to Section 7.1.3, Licensor shall provide Licensee with a reasonable opportunity to review and provide comments in connection with the Prosecution of the Licensed Patents; and Licensor shall keep Licensee reasonably informed as to all material developments with respect to such Licensed Patents and shall supply to Licensee copies of material communications received and filed in connection with the Prosecution of such Licensed Patents.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.1.3 Licensee acknowledges that the University of Pennsylvania controls Prosecution of the Licensed Patents, with Licensor having certain rights to review. Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.1 are subject to the rights of Licensor's direct and indirect licensors with respect to the Licensed Patents, and (b) Licensor's obligations under this Agreement only apply to the extent of Licensor's rights with respect to participation in Prosecuting the Licensed Patents under its agreements with its direct and indirect licensors.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents (other than Retained Rights) that may come to Licensee's attention. Licensee and Licensor shall consult one another in a timely manner concerning any appropriate response to the infringement.

7.2.2 As between Licensor and Licensee, Licensor shall have the first right, but not the obligation, to prosecute any such infringement ****. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensor is unable to initiate or prosecute such action solely in its own name, Licensee shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute, maintain, and settle such action.

7.2.3 If Licensor elects not to pursue any infringement of a Licensed Patent, then, to the extent that a Licensed Product is covered by an)) such License Patent and such Licensed Patent is being infringed by another product in the Field (such infringement, the "Competitive Infringement"), Licensee shall have the second right, but not the obligation, to prosecute such

Competitive Infringement with respect to such other product in the Field, at Licensee's own expense. In any such action to enforce any of the Licensed Patents, Licensor, at the request and expense of Licensee, shall cooperate to the fullest extent reasonably possible, including in the event that, if Licensee is unable to initiate or prosecute such action solely in its own name, Licensor shall join such action voluntarily and shall execute all documents necessary to initiate litigation to prosecute and maintain such action. In prosecuting any such Competitive Infringement, Licensee (a) shall not take any actions that would be detrimental to the Licensed Patents and Licensor's rights with respect thereto outside the Field and (b) shall not settle any such Competitive Infringement without the prior consent of Licensor.

7.2.4 Any recovery of damages by Licensor for any infringement other than a Competitive Infringement shall be ****. Any recovery of damages by the Party undertaking enforcement or defense of a suit for Competitive Infringement shall be applied, as between Licensor and Licensee but subject to the obligations to Licensor's direct and indirect licensors, first to reimburse each such Party for costs and expenses (including reasonable attorneys' fees and costs) incurred by such Party in connection with such suit, and the balance remaining, if any, from any such recovery shall be ****.

7.2.5 Licensee acknowledges and agrees that (a) the rights and obligations under this Section 7.2 are subject to the rights of Licensor's direct and indirect licensors of the Licensed Patents (including any consent or approval rights or rights to control or participate in any enforcement actions); and (b) Licensor's obligations under this Agreement only apply to the extent that Licensor has any rights with respect to enforcing the Licensed Patents under its agreements with its direct and indirect licensors. Furthermore, Licensee acknowledges the following:

7.2.5.1 All monies recovered upon the final judgment or settlement of any action with respect to Competitive Infringement will also need to be allocated to Licensor's direct and indirect licensors (a) to reimburse the costs and expenses (including reasonable attorneys' fees and costs) of such licensors, (b) to take into account the royalties payable to such licensors; and (c) to take into account the relative extent of such licensors' financial participation in such action, if applicable.

7.2.5.2 Licensor's direct and indirect licensors retain the continuing right to intervene at their own expense and join Licensor or Licensee in any claim or suit for infringement of the Licensed Patents.

7.2.5.3 In any infringement prosecuted by Licensors' direct and indirect licensors, all financial recoveries will be ****.

7.2.5.4 In any infringement prosecuted by Licensor's direct and indirect licensors, Licensee agrees, at the request and expense of such licensors, to cooperate to the fullest extent reasonably possible, to the same extent as though Licensor were prosecuting such suit (as provided in this Section 7.2, including Section 7.2.2).

7.2.5.5 The written consent of Licensor's direct and indirect licensors will be required (a) for any decision that would have a materially adverse effect on the validity,

scope of patent claims, or enforceability of the Patent Rights and (b) for any settlement or compromise of any infringement suit that would impose any obligations or restrictions on any of its direct or indirect licensors, or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringements by another. To the extent Licensor takes any action, Licensor (or its direct or indirect licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or any of its direct or indirect licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: WARRANTIES; INDEMNIFICATION

8.1 Warranty by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor is duly organized, validly existing, and in good standing under the laws of the jurisdiction of its formation;

8.1.2 Licensor has taken all necessary action on its part to authorize the execution of this Agreement and the performance of all of its obligations under this Agreement and the persons executing this Agreement are authorized to execute it;

8.1.3 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the rights specified in this Agreement;

8.1.4 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.5 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Patents that would impact activities under this Agreement;

8.1.6 To Licensor's knowledge, (a) the Licensed Patents are solely owned by the University of Pennsylvania, and (b) no Third Party (other than Licensor's direct and indirect licensors) has any right, interest? or claim in or to such Licensed Patents in the Field that are inconsistent with those granted td Licensee in the Field under this Agreement;

8.1.7 To Licensor's knowledge, no Third Party is infringing any of the Licensed Patents in a manner that is inconsistent with the scope of rights granted to Licensee in the Field under this Agreement;

8.1.8 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Patents in the Field;

8.1.9 To Licensor's knowledge, the Penn Agreement and GSK Agreement are in full force and effect and Licensor is not in breach of any provisions thereof; and

8.1.10 To Licensor's knowledge, Licensor does not Control as of the Effective Date any patent or patent application (other than the Licensed Patents) that has a claim expressly reciting a composition of matter of AAVrh10 (as defined in Section 1.1(a)). For the purpose of this Section 8.1.10, "Control" means the possession by Licensor (whether by ownership or license, other than pursuant to this Agreement) of the ability to grant to Licensee access, a license, or a sublicense (as applicable) to the applicable patent or patent application on the terms and conditions set forth herein without violating the terms of any agreement or other arrangement with any Third Party.

8.2 Warranty by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee is duly organized, validly existing, and in good standing under the laws of the jurisdiction of its formation;

8.2.2 Licensee has taken all necessary action on its part to authorize the execution of this Agreement and the performance of all of its obligations under this Agreement and the persons executing this Agreement are authorized to execute it;

8.2.3 Licensee has the right, power, and authority to enter into this Agreement and to grant the rights granted by it hereunder;

8.2.4 This Agreement when executed shall become the legal, valid and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.5 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.6 There are no actions, suits, proceedings, or arbitrations pending or, to the Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages. EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO

REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES. EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR OR ANY OF LICENSOR'S DIRECT OR INDIRECT LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO: (a) ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF LICENSED PRODUCTS; OR (b) ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES. NOTHING IN THIS SECTION 8.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER Article 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, its direct and indirect licensors of the Licensed Patents, and their respective shareholders, members, officers, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents or Licensed Products, including any claim by or on behalf of a ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties that result from or arise out of: **** provided, however, that Licensor shall not be liable for claims based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner that imposes any restrictions or obligations on any indemnified party (an "Indemnified Party") without the Indemnified Party's prior written consent or, if Licensee is the Indemnifying Party, that imposes any restrictions Or obligations on Licensor's direct or indirect licensors or grants any rights to the Licensed Patents or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including

broad form and contractual liability, in a minimum amount of **** combined single limit per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employee's and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, land shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement without the prior written consent of Licensor; provided, however, that Licensee may assign this

Agreement, without Licensor's prior written consent, pursuant to a merger or sale of all or substantially all of the assets to which the Agreement relates; provided that, as part of any permitted assignment, (a) Licensee provides Licensor with notice of such assignment at least five business days prior to the effectiveness of such assignment, and (b) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (e.g., Federal Express), by Express Mail, receipt requested, or by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Lysogene Société par Actions Simplifiée
52 rue de la Boetie, 75008
Paris, France
Attn: Chief Executive Officer
Telephone: +33 1 56 88 52 87
Facsimile: + 33 1 56 88 52 81

with a copy to:

Colombus Audit & Expertise
52 rue de la Boetie, 75008
Paris, France
Attn: President
Telephone: + 33 1 56 88 52 90
Facsimile: + 33 1 56 88 52 81

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of New York, without giving effect to conflict pf law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of New York with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the 1 AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of New York, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, a d cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or

allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Non-Disclosure Agreement between the Parties dated January 1, 2012. All "Confidential Information" disclosed by the Parties pursuant to such Mutual Non-Disclosure Agreement shall be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.4). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them with any notice of patent rights necessary or desirable under applicable law enable the Licensed Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections and exhibits in this Agreement are to Sections and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

10.16 Recordation of License. During the term of this Agreement, if Licensee determines that it Is necessary to record a confirmation of the license granted under this Agreement in a country where a Licensed Patent is filed, which recording is necessary to comply with law or to make such license effective against Third Parties, Licensee may notify Licensor in writing of such

requirement, including an explanation thereof and a draft document to be recorded. Licensor will discuss such requirement in good faith with Licensee, together with the form of document to be filed, which the Parties intend will be a summary or acknowledgement of the license granted under this Agreement. If the Parties agree to a recording, Licensee may make the applicable recording in the form agreed to by Licensor, at Licensee's sole expense. Licensee agrees that it will not make any recordings or filings with respect to this Agreement without the prior written consent of Licensor both as to the requirement of the recording and the form of the recording to be made. Licensor agrees that Licensee may make a recording relating to this Agreement in France, subject to the Parties agreeing on the form of document to be recorded. Following expiration or termination of this Agreement for any reason, Licensor may record, at Licensee's expense, any documentation needed to reflect such expiration or termination, and Licensee agrees to provide Licensor reasonable assistance thereto, including by executing any necessary acknowledgements.

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IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By /s/ Kenneth Mills
Name: Kenneth Mills
Title: President and CEO

LYSOGENE SOCIÉTÉ PAR ACTIONS SIMPLIFIÉE

By: /s/ Karen Arach
Name: Karen Arach
Title: President and CEO

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Patents

Appin #	Title	Inventors	Nos	Docket
****	****	****	****	****

Docket	Country	Appin No	Filing Date	Patent Number	Issue Date	Pubn Number	Pub Date
****	****	****	****	****	****		
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****CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Press Release

DRAFT-NOT FOR DISTRIBUTION

REGENX BIOSCIENCES AND LYSOGENE ENTER INTO EXCLUSIVE LICENSE AGREEMENT FOR DEVELOPMENT OF TREATMENTS FOR SERIOUS, RARE LYSOSOMAL STORAGE DISORDER USING NAV® rAAVrh10 VECTORS

WASHINGTON, DC and PARIS, FRANCE [December , 2013] — REGENX Biosciences, LLC (REGENX) and LYSOGENE SAS (LYSOGENE) announce that they have entered into an agreement enabling the development and commercialization of products to treat mucopolysaccharidosis type IIIA (MPS IIIA or Sanfilippo syndrome Type A) using **NAV** rAAVrh10.

Under the terms of the Agreement, REGENX granted LYSOGENE an exclusive worldwide license, with rights to sublicense, to REGENX's **NAV** rAAVrh10 vectors for treatment of MPS IIIA in humans. In return for these rights, REGENX receives payments in the form of an up-front payment, certain milestone fees and royalties on net sales of products incorporating **NAV** rAAVrh10.

"We believe this exclusive license agreement will enable LYSOGENE to advance the development of its **NAV** based treatment for patients with MPS IIIA," said Ken Mills, President and CEO of REGENX. "As a leader in gene therapy, we are pleased to be formally collaborating with the Lysogene team that, by the successful completion of a recent Phase I/II trial, demonstrates outstanding expertise, resources, and commitment to patients. Providing partners with access to our **NAV** technology further advances REGENX's mission to enable the development of successful new AAV therapeutics."

"Lysogene is a leading clinical stage gene therapy company committed to the development of breakthrough therapies in rare diseases. The company successfully completed a phase VII study (NCT01474343/EudraCT 2010-019962-10) using the **NAV** rAAVrh10 technology in Sanfilippo syndrome. We are very

pleased to enter into this agreement with REGENX, which we believe offers us the best path to expeditiously advance the clinical development of our lead product towards the market and to patients in extraordinary high demand", said Karen Aiach, Founder, President and CEO of LYSOGENE.

"Sanfillipo disease is a complete unmet medical need and our clinical study using **NAV** rAAVrh10 indicates that gene therapy may become an outstanding treatment option", said Olivier Danos PhD, Co-founder and Senior Scientific Advisor to LYSOGENE.

About MPS III A (Sanfilippo syndrome Type A)

Sanfilippo syndrome is a lethal, rare, autosomal recessive condition characterized by rapid neurodegeneration, severe and invasive behavioural disorders, and mild peripheral symptoms. Patients generally do not live above their second decade. There is currently no treatment. Sanfilippo syndrome is caused by mutations in a gene that encodes N-sulfoglucosamine sulfohydrolase (*sulfamidase*) which is needed to break down glycoaminoglycans - used in a number of biological functions. It affects approximately 1 in 100,000 births, and is still largely underdiagnosed.

About REGENX Biosciences

REGENX Biosciences is the leading AAV gene therapy company that is developing a new class of personalized therapies, based on its proprietary **NAV** vector technology platform, for a range of severe diseases with serious unmet needs. **NAV** vector technology includes novel AAV vectors such as rAAV7, rAAV8, rAAV9, and rAAVrh10. Our treatments in development include programs for hypercholesterolemia, mucopolysaccharidoses, and retinitis pigmentosa. REGENX leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Chatham Therapeutics, Fondazione Telethon, and Audentes Therapeutics. In addition, together with Fidelity Biosciences, REGENX has formed Dimension Therapeutics a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding REGENX, please visit www.regenxbio.com.

About LYSOGENE

LYSOGENE is a clinical stage biotechnology company committed to the development of innovative therapies for patients affected with rare disorders and high unmet medical needs. LYSOGENE's team translated its rAAVrh10 lead product for Sanfilippo from bench to bedside in an unprecedented fashion over the last years. Its lead product is for Sanfilippo syndrome, a neurodegenerative lysosomal storage disorder considered to be a perfect model for gene therapy. LYSOGENE is currently expanding its pipeline to additional diseases with high unmet medical needs.

For more information about LYSOGENE, please visit www.lvsogene.com.

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Contacts:
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Vit Vasista, 202-785-7438
vvasista@regenxbio.com

LYSOGENE
Karen AIACH
karen.aiach@lysogene.com

CONFIDENTIAL TREATMENT REQUESTED

LICENSE AGREEMENT

This LICENSE AGREEMENT ("Agreement") is entered into as of May 28, 2014 ("Effective Date") by and between ReGenX Biosciences, LLC, a limited liability company organized under the laws of the State of Delaware, with offices at 750 17th Street, NW, Suite 1100, Washington, DC 20006 ("Licensor"), and Voyager Therapeutics, Inc., a corporation organized under the laws of the State of Delaware, with offices at 75 Sidney Street, Cambridge, MA 02139 ("Licensee"). Licensor and Licensee are hereinafter referred to individually as a "Party" and collectively as the "Parties."

WHEREAS, Licensor has rights under certain patents pertaining to various recombinant adeno-associated virus vectors;

WHEREAS, Licensee desires to obtain from Licensor, and Licensor is willing to grant to Licensee, (a) a non-exclusive research license to conduct certain research to identify and select Specified Vectors for specified indications and (b) an option to obtain a non-exclusive license to research, develop, and commercialize Licensed Products for specified indications under the terms set forth herein;

NOW, THEREFORE, in consideration of the promises and covenants contained in this Agreement, and intending to be legally bound, the Parties hereby agree as follows:

ARTICLE 1: DEFINITIONS

1.1 "AAVrh10" means (a) the recombinant adeno-associated virus serotype rh10 vector with the specified sequence set forth in GenBank **** and (b) any recombinant adeno-associated virus derivatives of such serotype rh10 vector that are covered by the claims of the Licensed Research Patents.

1.2 "AAV Materials" means recombinant adeno-associated virus serotype vectors, and any materials that are made or used for the sole purpose of making recombinant adeno-associated virus serotype vectors, in each case, which, in the absence of the license granted pursuant to Section 2.1, would infringe or is covered by at least one Valid Claim of the Licensed Research Patents in the country of manufacture or use.

1.3 "Affiliate" means any legal entity directly or indirectly, during the term of this Agreement, controlling, controlled by, or under common control with another entity. For purposes of this Agreement, "control" means the direct or indirect ownership of more than 50% of the outstanding voting securities of a legal entity, or the right to receive more than 50% of the profits or earnings of a legal entity, or the right to control the policy decisions of a legal entity. For clarity, an entity may be or become an Affiliate of an entity and may cease to be an Affiliate of an entity, in each case, during the term of this Agreement. Notwithstanding the foregoing, any person or entity that would otherwise qualify as an Affiliate of Licensee hereunder by this definition will not be deemed to be, and will not be treated as, an Affiliate of Licensee if (i) the primary business of such person or entity is investing in securities, debt, or other investment vehicles; provided that a person or entity that satisfies the criteria under this clause (i) who, directly or indirectly, during the term of this Agreement, controls Licensee will be deemed an Affiliate under Sections 6.6 and 8.4.1 during the period of time in which such person or entity

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

controls Licensee; or (ii) such person or entity is a portfolio company of a person or entity that satisfies the criteria under clause (i). Licensee represents and warrants that, as of the Effective Date, Third Rock Ventures and its related funds satisfy the criteria under clause (i) of the preceding sentence; and, as such, the Parties agree that, for so long as the foregoing representation and warranty remains true, Third Rock Ventures and its related funds will be excluded from classification as Affiliates of Licensee under this Agreement to the extent provided in the immediately prior sentence.

1.4 "Calendar Quarter" means each three-month period or any portion thereof, beginning on January 1, April 1, July 1, and October 1.

1.5 "Commercial Field" means the treatment or prevention of a Disease Indication (if and when a Commercial Option is exercised for such Disease Indication by Licensee under Section 2.3) in human beings by *in vivo* gene therapy with the applicable Specified Vector selected for the applicable Disease Indication.

1.6 "Commercial Option" has the meaning set forth in Section 2.3.

1.7 "Confidential Information" means and includes all technical information, inventions, developments, discoveries, software, know-how, methods, techniques, formulae, animate and inanimate materials, data, processes, finances, business operations or affairs, and other proprietary ideas, whether or not patentable or copyrightable, of either Party that are (a) marked or otherwise identified as confidential or proprietary at the time of disclosure in writing; or (b) if disclosed orally, visually, or in another non-written form, identified as confidential at the time of disclosure and summarized in reasonable detail in writing as to its general content within 30 days after original disclosure. The Parties acknowledge that (i) the terms and conditions of this Agreement and (ii) the records and reports referred to in Section 3.7 will be deemed the Confidential Information of both Parties, regardless of whether such information is marked or identified as confidential. Notwithstanding the foregoing, Confidential Information will not include the following, in each case, to the extent evidenced by competent written proof of the Receiving Party:

1.7.1 information that was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party;

1.7.2 information that was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party;

1.7.3 information that became generally available to the public or otherwise part of the public domain after its disclosure, other than through any act or omission of the Receiving Party in breach of this Agreement;

1.7.4 information that is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or

1.7.5 information that was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

1.8 "Disclosing Party" has the meaning set forth in Section 5.1.

1.9 "Disease Indication(s)" means one or more of the following indications: (a) Friedreich's Ataxia that is treated or prevented by administration of the applicable recombinant adeno-associated virus serotype vector directly to the central nervous system (brain and spinal cord) ("Friedreich's Ataxia (CNS)"), (b) Friedreich's Ataxia that is treated or prevented by administration of the applicable recombinant adeno-associated virus serotype vector by any route except administration directly to the central nervous system (brain and spinal cord) ("Friedreich's Ataxia (Systemic)"), (c) Huntington's Disease, and (d) Amyotrophic Lateral Sclerosis.

1.10 "Domain Antibody" ****.

1.11 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.12 "GSK Agreement" means that certain License Agreement entered into between Licensor and SmithKline Beecham Corporation, effective on March 6, 2009, as amended by that certain Amendment to License Agreement dated April 15, 2009, and as amended from time to time.

1.13 "Licensed Commercial Patents" means, on a Specified Vector-by-Specified Vector basis, to the extent they cover such Specified Vector, (a) all United States patents and patent applications listed in Exhibit D (or on Exhibit A, until such time as this Agreement is amended to add Exhibit D in accordance with Section 2.3.3), including patents arising or issuing from such patent applications; and (b) any re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Commercial Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.14 "Licensed Patents" means the Licensed Commercial Patents or Licensed Research Patents, as applicable.

1.15 "Licensed Product" means (a) any product using the applicable Specified Vector capsid protein that is made, made for, used, sold, offered for sale, or imported by Licensee, its Affiliates, and any of its or their Sublicensees, the manufacture, use, sale, offer for sale, or import of which product, in the absence of the license granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim of the Licensed Commercial Patents in the country of manufacture, use, sale, offer for sale, or import; or (b) any service sold by Licensee, its Affiliates, and any of its or their Sublicensees with respect to the administration of any product using the applicable Specified Vector capsid protein to patients that, in the absence of the licenses granted pursuant to this Agreement, would infringe or is covered by at least one Valid Claim of the Licensed Commercial Patents in the country of sale.

1.16 "Licensed Research Patents" means (a) all United States patents and patent applications listed in Exhibit A, including patents arising or issuing from such patent applications; and (b) any

re-examination certificates thereof, and their foreign counterparts and extensions, continuations, divisionals, and re-issue applications; provided that "Licensed Research Patents" will not include any claim of a patent or patent application covering any Manufacturing Technology.

1.17 "Manufacturing Technology" means any and all patents, patent applications, know-how, and all intellectual property rights associated therewith that are owned or controlled by Licensor, and including all tangible embodiments thereof, that are necessary or useful for the manufacture of adeno-associated viruses, adeno-associated virus vectors, research or commercial reagents related thereto, Licensed Products, or other products, including manufacturing processes, technical information relating to the methods of manufacture, protocols, standard operating procedures, batch records, assays, formulations, quality control data, specifications, scale up, any and all improvements, modifications, and changes thereto, and any and all activities associated with such manufacture. Any and all chemistry, manufacturing, and controls (CMC), drug master files (DMFs), or similar materials provided to regulatory authorities and the information contained therein are deemed Manufacturing Technology.

1.18 "NDA" means a New Drug Application filed with the FDA as described in 21 C.F.R. § 314, a Biological License Application (BLA) pursuant to 21 C.F.R. § 601.2, or any equivalent or any corresponding application for regulatory approval in any country or regulatory jurisdiction other than the United States.

1.19 "Net Sales" means the gross receipts from sales or other disposition of a Licensed Product (including fees for services within the definition of "Licensed Product") by Licensee and/or its Affiliates and/or any Sublicensees to Third Parties less the following deductions that are directly attributable to a sale, specifically and separately identified on an invoice or other documentation and actually borne by Licensee, its Affiliates, or any Sublicensees: ****. In the event consideration other than cash is paid to Licensee, its Affiliates, or any Sublicensees, for purposes of determining Net Sales, the Parties shall use the cash consideration that Licensee, its Affiliates, or any Sublicensees would realize from an unrelated buyer in an arm's length sale of an identical item sold in the same quantity and at the time and place of the transaction, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

1.20 "Penn Agreement" means that certain License Agreement entered into between Licensor and The Trustees of the University of Pennsylvania, effective on February 24, 2009, as amended by that letter agreement dated March 6, 2009, and as amended from time to time.

1.21 "Phase 3 Clinical Trial" means a pivotal clinical trial in humans performed to gain evidence with statistical significance of the efficacy of a product in a target population, and to obtain expanded evidence of safety for such product that is needed to evaluate the overall benefit-risk relationship of such product, to form the basis for approval of an NDA and to provide an adequate basis for physician labeling, as described in 21 C.F.R. § 312.21(c) or the corresponding regulation in jurisdictions other than the United States.

1.22 "Prosecute" means preparation, filing, and prosecuting patent applications and maintaining patents, including any reexaminations, reissues, oppositions, inter partes review, and interferences.

1.23 "Receiving Party" has the meaning set forth in Section 5.1.

1.24 "ReGenX Licensors" means SmithKline Beecham Corporation (or any successor thereto under the GSK Agreement) and The Trustees of the University of Pennsylvania (or any successor thereto under the Penn Agreement).

1.25 "Research Field" means Licensee's internal research and pre-clinical development for the treatment or prevention of any of the Disease Indications in humans by *in vivo* gene therapy using AAV Materials. Notwithstanding the foregoing, "Research Field" specifically excludes the use of AAVrh10 for the treatment or prevention of Friedreich's Ataxia (Systemic). Furthermore, "Research Field" specifically excludes (without limitation) (a) all human clinical trial use, diagnostic use, therapeutic use, and prophylactic use, and (b) any commercial uses.

1.26 "Research Term" means, on a Disease Indication-by-Disease Indication basis, a period beginning with the Effective Date and ending on the earlier of (a) the Grant Date, if any, with respect to the applicable Disease Indication and (b) the 18-month anniversary of the Effective Date, or if the Research Term is extended pursuant to Section 2.2, the 30-month anniversary of the Effective Date.

1.27 "Retained Rights" has the meaning set forth in Section 2.4.

1.28 "Secondary Disease Indications" collectively mean (a) Friedreich's Ataxia (Systemic), (b) Huntington's Disease, and (c) Amyotrophic Lateral Sclerosis.

1.29 "Specified Vector" means the recombinant adeno-associated virus serotype vector with a specified sequence set forth in GenBank that is selected by Licensee pursuant to Section 2.3 and which is specified on Exhibit C (to be attached hereto as of the applicable Grant Date as provided in Section 2.3).

1.30 "Sublicensee" means (i) any Third Party or Affiliate to whom Licensee grants a sublicense of some or all of the rights granted to Licensee under this Agreement as permitted by this Agreement; and (ii) any other Third Party or Affiliate to whom a sublicensee described in clause (i) has granted a further sublicense as permitted by this Agreement.

1.31 "Third Party" means any person or entity other than a Party to this Agreement or Affiliates of a Party to this Agreement.

1.32 "Third Party Collaborator" means a Third Party with whom Licensee has entered into a collaboration for a particular Disease Indication under which (a) research and development activities will be performed on a shared basis during the Research Term for the purpose of Licensee and such Third Party determining which Specified Vector would be selected if the Commercial Option for such Disease Indication were exercised, and (b) the Third Party will be granted commercial rights upon exercise of a Commercial Option for such Disease Indication. For the avoidance of doubt, a Third Party Collaborator will not include a Third Party who is granted the right to conduct research and development activities independent of Licensee or unrelated to the exercise of a Commercial Option.

1.33 "Valid Claim" means (a) a claim of an issued and unexpired patent (including any patent claim the term of which is extended by any extension, supplementary protection certificate, patent term restoration, or the like) included within the Licensed Patents or (b) a claim of a pending patent application included within the Licensed Patents that has not been pending for more than 15 years from the earliest filing date to which such claim or the applicable patent application is entitled to claim priority, in each case under clauses (a) and (b) which has not lapsed, been abandoned, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

ARTICLE 2: LICENSE GRANTS

2.1 Research License Grant. Subject to the terms and conditions of this Agreement, including the Retained Rights, during the Research Term, Licensor hereby grants to Licensee a non-exclusive, sublicensable (as provided in Section 2.6 only), non-transferable (except as provided in Section 10.2), worldwide license under the Licensed Research Patents to make, have made, and use any and all AAV Materials in the Research Field (including, for the avoidance of doubt, the right to conduct research and pre-clinical development) solely for purposes of identifying and selecting Specified Vector(s) for use in the Commercial Field upon exercise of a Commercial Option. For the avoidance of doubt, the foregoing license in this Section 2.1 does not include the right to sell, offer for sale, or import any AAV Materials.

2.2 Research License Extension Option. Licensee may extend the Research Term with respect to any or all of the Disease Indications with respect to which the Commercial Option has not been exercised pursuant to Section 2.3 prior to the **** of the Effective Date by providing written notice to Licensor of such extension and simultaneously paying Licensor a fee of ****, which notice and payment must be received by Licensor at least **** prior to the **** of the Effective Date. If Licensee does not extend the Research Term under this Section 2.2, the Research Term with respect to any or all of the Disease Indications with respect to which the Commercial Option has not been exercised pursuant to Section 2.3 or otherwise terminated by Licensee pursuant to Section 6.3 prior to the **** of the Effective Date will expire on the **** of the Effective Date.

2.3 Commercial License Option. Subject to the terms and conditions of this Agreement, Licensor hereby grants to Licensee the option, exercisable at Licensee's sole discretion, to obtain a non-exclusive worldwide license with respect to each of the Disease Indications and a single

Specified Vector for such Disease Indication (each such right with respect to a particular Disease Indication, a "Commercial Option") in accordance with the following provisions:

2.3.1 Method of Exercise. To exercise the Commercial Option for a particular Disease Indication, Licensee must provide written notice to Licensor prior to the end of the applicable Research Term, which written notice must specify the Disease Indication(s) and Specified Vector (as further described in Section 2.3.2) with respect to which Licensee desires to exercise its Commercial Option. For each of the Secondary Disease Indications, such written notice must be accompanied by a wire transfer of the commercial option fee set forth in Section 3.2 for such Secondary Disease Indication.

2.3.2 Specified Vector. For purposes of selecting a Specified Vector for use with a Disease Indication, the Specified Vector must be a recombinant adeno-associated virus serotype vector with a specified sequence. Licensee's notice of the specified sequence will provide Licensor with a published source that refers to the sequence (which may include a reference to the Licensed Research Patents), if there is a public source. The sequence of the Specified Vector will be provided to Licensor in a written format setting forth the entire DNA sequence and amino acid sequence in Vector NTI format (from Life Technologies) (or such other format, as the Parties agree) that will enable Licensor to analyze the sequence through the Vector NTI electronic sequence editing program. Licensee may not select AAVrh10 as the Specified Vector for the treatment or prevention of Friedreich's Ataxia (Systemic). Upon Licensor's receipt of the notice and, if applicable, fee described in Section 2.3.1, this Agreement will be amended to add a new Exhibit C (or amend a then-existing Exhibit C) prepared by Licensor setting forth the Specified Vector for each Disease Indication with respect to which a Commercial Option is exercised.

2.3.3 Licensed Commercial Patents. Within **** after Licensor's receipt of the notice and, if applicable, fee described in Section 2.3.1, Licensor will prepare a new Exhibit D setting forth the applicable Licensed Commercial Patents that apply to the Specified Vector and applicable Disease Indication, which Licensed Commercial Patents will be taken solely from the Licensed Research Patents. Upon Licensee's acceptance of the new Exhibit D (which acceptance will not be unreasonably withheld, conditioned, or delayed), this Agreement will be amended to add such new exhibit. If different Specified Vectors are specified for use in connection with different Disease Indications, then Licensor may create a separate exhibit (labeled Exhibit D-1 through D-4, as necessary) for each Specified Vector. Until this Agreement is amended to include the new Exhibit D, Exhibit A will continue to form the basis for determining the scope of the applicable Licensed Commercial Patents.

2.3.4 License Grant Upon Exercise. If Licensee exercises the Commercial Option for a particular Disease Indication, effective upon both (a) Licensor's receipt of the notice and (b) in the case of a Secondary Disease Indication, the fee described in Section 2.3.1 for such Secondary Disease Indication (the date on which the notice and the fee (if applicable) are received shall be deemed to be the "Grant Date" for such Disease Indication), subject to the terms and conditions of this Agreement, including the Retained Rights, Licensor shall grant, and hereby grants, to Licensee a non-exclusive, sublicensable (as provided in Section 2.6 only), non-transferable (except as provided in Section 10.2), royalty-bearing, worldwide license under the applicable Licensed Commercial Patents to make, have made, use, import, sell, and offer for sale Licensed

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Products using the Specified Vector solely in the Commercial Field for such Disease Indication, including, for the avoidance of doubt, the right to conduct research and development.

2.3.5 Disease Indications. For the avoidance of doubt, the foregoing license granted pursuant to Section 2.3.4 will be deemed granted on the Grant Date on a Disease Indication-by-Disease Indication basis, solely with respect to the Commercial Field associated with the Disease Indication for which the Commercial Option was exercised under this Section 2.3 and solely with respect to Licensed Products using the Specified Vector selected for the particular Disease Indication. The Parties acknowledge that there may be different Grant Dates for each Disease Indication, depending on when and if Licensee exercises the Commercial Option for a particular Disease Indication. As set forth above, Licensee, at its sole discretion, may exercise the Commercial Option with respect to any or all of the four Disease Indications. If Licensee exercises the Commercial Option with respect to only some of the Disease Indications but not all, the Commercial Option will terminate with respect to any unexercised Disease Indications and the license granted under Section 2.1 will also terminate, in each case, at the end of the Research Term, and Licensee will have no further rights under this Agreement with respect to such unexercised Disease Indications.

2.4 Retained Rights. Except for the rights and licenses specified in Sections 2.1 and, if applicable, 2.3.4, no license or other rights are granted to Licensee under any intellectual property of Licensor, whether by implication, estoppel, or otherwise and whether such intellectual property is subordinate, dominant, or otherwise useful for the practice of the Licensed Patents. Notwithstanding anything to the contrary in this Agreement, Licensor may use and permit others to use the Licensed Patents for any research, development, commercial, or other purposes inside or outside of the Commercial Field or the Research Field. Without limiting the foregoing, and notwithstanding anything in this Agreement to the contrary, Licensee acknowledges and agrees to the following rights retained by Licensor and the ReGenX Licensors (individually and collectively, the "Retained Rights"), whether inside or outside the Commercial Field or Research Field:

2.4.1 The rights and licenses granted in Sections 2.1 and, if applicable, 2.3.4 shall not include any right (and Licensor and the ReGenX Licensors retain the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents to make, have made, use, sell, offer to sell, and import Domain Antibodies that are expressed by an adeno-associated vector, including any Specified Vector.

2.4.2 Licensor and the ReGenX Licensors retain the following rights with respect to the Licensed Patents:

(a) A non-exclusive, sublicensable right under the Licensed Patents to make, have made, use, sell, offer to sell, and import products that deliver RNA interference and antisense drugs using an adeno-associated vector, including any Specified Vector; and

(b) A non-exclusive right for the ReGenX Licensors (which right is sublicensable by such licensors) to use the Licensed Patents for non-commercial research purposes and to use the Licensed Patents for such

licensors' discovery research efforts with non-profit organizations and the ReGenX Licensors' collaborators.

2.4.3 The rights and licenses granted in Sections 2.1 and, if applicable, 2.3.4 shall not include any right (and Licensor retains the exclusive (even as to Licensee), fully sublicensable right) under the Licensed Patents:

(a) to conduct commercial reagent and services businesses, which includes the right to make, have made, use, sell, offer to sell, and import research reagents, including any viral vector construct; provided that for clarity, such exclusive rights retained by Licensor shall not include the right to conduct clinical trials in humans in the Commercial Field, though Licensor retains the non-exclusive right to do so; or

(b) to use the Licensed Patents to provide services to any Third Parties; provided that Licensee's license under Section 2.3.4, if applicable, does include the right to provide the services of the administration of Licensed Products to patients.

2.4.4 Licensor retains the fully sublicensable right under the Licensed Patents to grant non-exclusive research and development licenses to Affiliates and Third Parties.

2.4.5 The Trustees of the University of Pennsylvania may use and permit other non-profit organizations or other non-commercial entities to use the Licensed Patents for educational and research purposes.

2.5 Government Rights. Licensee acknowledges that the United States government retains certain rights in intellectual property funded in whole or part under any contract, grant, or similar agreement with a federal agency. The license grants hereunder are expressly subject to all applicable United States government rights, including any applicable requirement that products that result from such intellectual property and are sold in the United States must be substantially manufactured in the United States.

2.6 Sublicensing.

2.6.1 The research license granted pursuant to Section 2.1 is sublicensable by Licensee (a) to Affiliates of Licensee and (b) to one Third Party Collaborator with respect to each Disease Indication; any other sublicenses to Third Party Collaborators or Third Parties of the research license granted pursuant to Section 2.1 requires Licensor's prior written consent, which consent may not be unreasonably withheld, conditioned, or delayed. The license granted, if applicable, pursuant to Section 2.3.4 is sublicensable by Licensee to any Affiliates or Third Parties. Any sublicense of the rights under this Section 2.6, whether to an Affiliate or Third Party and whether relating to a sublicense of rights under Section 2.1 or 2.3.4, must comply with the provisions of this Section 2.6 (including Section 2.6.2).

2.6.2 The right to sublicense granted to Licensee under this Agreement is subject to the following conditions:

(a) Licensee may grant a sublicense to an Affiliate of Licensee; provided that (i) such sublicense must comply with the terms of this Section 2.6.2 (except to the extent such terms are limited to Third Party Sublicensees), including being granted pursuant to a written agreement and requiring the Sublicensee to comply with the applicable terms and conditions of this Agreement; (ii) Licensee must provide Licensor with written notice of any such sublicense within **** after entering into a sublicense, which notice will identify the Affiliate, the applicable Disease Indication, and the scope of the rights sublicensed; (iii) such sublicense must only remain in effect for as long as such sublicensee remains an Affiliate of Licensee; and (iv) without limiting Section 2.6.2(f) below, Licensee will be responsible for any and all obligations of any such Affiliate as if such Affiliate were "Licensee" hereunder. If either of the ReGenX Licensors requires additional information, including a copy of the sublicense agreement, Licensee shall provide such information, including such copy, to Licensor.

(b) Licensee may only grant sublicenses pursuant to a written sublicense agreement with the Sublicensee. Licensee may grant a direct Sublicensee (as defined in Section 1.30(i) only) of the rights under Section 2.3.4 the right to grant further sublicenses ****. For the avoidance of doubt, any further sublicenses granted by any Sublicensees must comply with the provisions of this Section 2.6 (including Section 2.6.2) to the same extent that Licensee would have to comply if Licensee were granting a sublicense directly to a Third Party (including the obligation of requiring the Sublicensee to comply with the applicable terms and conditions of this Agreement and providing Licensor with a copy of the sublicense). For clarity, Licensee is entitled to grant to a Sublicensee a sublicense with respect to any or all of the Disease Indications.

(c) In each sublicense agreement, (i) the Sublicensee must be required to comply with the terms and conditions of this Agreement to the same extent as Licensee has agreed, except to the extent that such terms and conditions do not relate to the specific rights granted to the Sublicensee pursuant to this Agreement (e.g., obligations related to a Disease Indication that has not been sublicensed); and (ii) if such Sublicensee is a Third Party, such Sublicensee must acknowledge that Licensor is an express third party beneficiary of such terms and conditions under such sublicense agreement.

(d) The official language of any sublicense agreement shall be English.

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(e) Within **** after entering into a sublicense with a Third Party Sublicensee, Licensor must receive a copy of the sublicense written in the English language for Licensor's records and to share with the ReGenX Licensors. The copy of the sublicense may be redacted to exclude confidential information of Licensee or the applicable Sublicensee, but such copy shall not be redacted to the extent that it impairs Licensor's (or the ReGenX Licensors') ability to ensure compliance with this Agreement; provided that, if either of the ReGenX Licensors requires a complete, unredacted copy of the sublicense, Licensee shall provide such complete, unredacted copy.

(f) Licensee's execution of a sublicense agreement will not relieve Licensee of any of its obligations under this Agreement. Licensee is and shall remain **** to Licensor for all of Licensee's duties and obligations contained in this Agreement and for any act or omission of an Affiliate or Sublicensee that would be a breach of this Agreement if performed or omitted by Licensee, and Licensee will be deemed to be in breach of this Agreement as a result of such act or omission.

2.7 Improvements.

2.7.1 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license to use any Licensed Back Improvements (and any intellectual property rights with respect thereto) consummate in scope to the Retained Rights.

2.7.2 Licensee hereby grants to Licensor a non-exclusive, worldwide, royalty-free, transferable, sublicensable, irrevocable, perpetual license to use and practice any Licensed Back Improvements (and any intellectual property rights with respect thereto) for any and all purposes, including the right to research, develop, make, have made, use, offer for sale, and sell products and services; provided that Licensor shall have no right, under the license in this Section 2.7.2, to use or practice the Licensed Back Improvements, on a Disease Indication-by-Disease Indication basis, (i) inside the Research Field during the Research Term for such Disease Indication or (ii) if the Commercial Option for such Disease Indication is exercised, inside the Commercial Field during the term of this Agreement for such Disease Indication.

2.7.3 For purposes of this Agreement, but subject to Sections 2.7.5 and 2.7.6, "Licensed Back Improvements" means (a) with respect to Section 2.7.1, any patentable modifications or improvements developed, during the term of this Agreement, by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within the Licensed Patents, and (b) with respect to Section 2.7.2, any patentable modifications or improvements developed, during the term of this Agreement, by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim as of the Effective Date within the Licensed Patents.

2.7.4 Licensee agrees to provide prompt notice to Licensor upon the filing of any patent application covering any Licensed Back Improvement, together with a reasonably detailed

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description of or access to such Licensed Back Improvement to permit the practice of any such Licensed Back Improvement in accordance with the rights granted hereunder.

2.7.5 With respect to any patentable modifications or improvements developed by any Third Party Sublicensee, the definition of "Licensed Back Improvement" under Section 2.7.3 will only include patentable modifications or improvements that are (a) developed by such Third Party Sublicensee during the term of the applicable sublicense granted to such Third Party Sublicensee; and (b) developed by such Third Party Sublicensee (i) to any vector if developed during the Research Term for the particular Disease Indication(s) sublicensed to such Third Party Sublicensee or (ii) to the Specified Vector(s) for the particular Disease Indication(s) sublicensed to such Third Party Sublicensee if developed following the Grant Date for such Disease Indication(s).

2.7.6 Notwithstanding Section 2.7.3, if Licensee undergoes a Change of Control pursuant to which a Third Party acquirer becomes an Affiliate of Licensee hereunder, patentable modifications and improvements that were developed by such acquirer and such acquirer's Affiliates (excluding Licensee and Licensee's Affiliates prior to such Change of Control) prior to such Change of Control will not become "Licensed Back Improvements" hereunder solely because of such Change of Control transaction, but thereafter the provisions of Section 2.7.3 will apply to patentable modifications or improvements of such acquirer and its Affiliates (if also Affiliates of Licensee) developed after such Change of Control.

ARTICLE 3: CONSIDERATION

3.1 Initial Fee. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor an initial fee of $500,000 within **** after the Effective Date.

3.2 Commercial Option Fee. If Licensee elects to exercise the Commercial Option granted to Licensee under Section 2.3 with respect to any Secondary Disease Indication, Licensee shall pay Licensor a fee of **** for the first Secondary Disease Indication and **** for each of the second and third Secondary Disease Indications. For clarity, no such fee will be required with respect to Friedreich's Ataxia (CNS).

3.3 Annual Maintenance Fee. In consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor on-going annual maintenance fees on each anniversary of the Effective Date. Licensor will invoice Licensee for the amount of such maintenance fee, and the invoiced amount will be due and payable by Licensee on the later of (i) 30 days after receipt of the invoice and (ii) the applicable anniversary of the Effective Date. The annual maintenance fees will equal (a) on each anniversary prior to Licensee exercising the Commercial Option with respect to any Disease Indication, ****, and (b) on each anniversary after Licensee has exercised the Commercial Option with respect to any Disease Indication, **** for each Disease Indication with respect to which the Commercial Option has been exercised as of such anniversary, up to a maximum under this clause (b) of **** for all four Disease Indications. If the royalty obligation with respect to any Disease Indication has expired or such Disease Indication has otherwise been terminated, the amount due pursuant to this

Section 3.3 will be decreased by **** for each Disease Indication with respect to which the royalty obligation has expired or such Disease Indication has otherwise been terminated.

3.4 Milestone Fees. If Licensee exercises the Commercial Option granted to Licensee under Section 2.3 with respect to any Disease Indication, in consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay Licensor the following milestone payments on a per-Disease Indication basis for the first Licensed Product for such Disease Indication to achieve such milestone event:

Milestone	**Milestone Payment**
1. First treatment of human subject in a clinical trial (*i.e.*, first patient, first dose)	****
2. First treatment in Phase 3 Clinical Trial (*i.e.*, first patient, first dose)	****
3. NDA submission in the United States	****
4. NDA submission in the European Union or the rest of the world (excluding the United States)	****
5. NDA approval in the United States	****
6. NDA approval in the European Union or the rest of the world (excluding the United States)	****
Total (per Disease Indication):	$ 5,000,000

For clarity, the milestone payments set forth in this Section 3.4 are payable **** with respect to each Disease Indication within the Commercial Field with respect to the first Licensed Product for such Disease Indication that achieves the milestone event, ****. To the extent that either of the two development milestones in this Section 3.4 (*i.e.*, first treatment of human subject in a clinical trial or first treatment in Phase 3 Clinical Trial) has not been paid at the time of achievement of either NDA submission milestone, then, upon the achievement of either of such NDA submission milestones, the preceding unpaid development milestone payments shall be made in addition to the payment corresponding to the NDA submission milestone that has been achieved.

3.5 Royalties. If Licensee exercises the Commercial Option granted to Licensee under Section 2.3 with respect to any Disease Indication, in consideration of the rights and licenses granted to Licensee under this Agreement, Licensee shall pay to Licensor the following royalties based upon the annual Net Sales worldwide of all Licensed Products for all Disease Indications in the Commercial Field in a given calendar year, subject to the reductions in royalty rates set forth in Section 3.5.1:

Cumulative Annual Net Sales of all Licensed Products for all Disease Indications in the Commercial Field Worldwide	Royalty Percentage
Portion of Net Sales less than $300,000,000	****
Portion of Net Sales between (and including) $300,000,000 through (and including) $600,000,0000	****
Portion of Net Sales greater than $600,000,000	****

3.5.1 Third Party Royalties Stacking Provision. If Licensee must obtain a license from a Third Party to avoid infringement of such Third Party's rights in order to manufacture, use, or commercialize a given Licensed Product and if the royalties required to be paid to such Third Party for such license, together with those royalties payable to Licensor, in the aggregate, exceed **** of Net Sales for any Licensed Product, then the royalty owed to Licensor for that Licensed Product will be reduced by an amount calculated as follows:

STACKING ROYALTY CALCULATIONS

R = (C * (A / (A+B)))

Where
R = Reduction of Licensor royalty,
A = Unreduced Licensor royalty,
B = sum of all Third Party royalties,
C = increment of projected total royalty above ****.

Example Calculation:

assume: i) all Third Party royalties = ****
ii) unreduced Licensor royalty = ****
iii) projected total royalty = ****

R = (**** - ****) * (**** / (****+****))
R = (**** * ****)
R = ****
Licensor Stacked Royalty = **** — **** = ****%

Notwithstanding the foregoing, Licensee will pay to Licensor no less than **** of the royalties that Licensee would otherwise pay to Licensor with respect to Net Sales of Licensee if there were no royalties due to Third Parties.

3.5.2 Royalty Payment Period. Licensee's obligation hereunder for payment of a royalty under this Section 3.5 on the Net Sales of Licensed Products in a given country will expire on a Licensed Product-by-Licensed Product and country-by-country basis ****.

3.5.3 No Multiple Royalties. If the manufacture, use, sale, offer for sale, or import of any Licensed Product infringes or is covered by more than one of the Licensed Commercial Patents, multiple royalties shall not be due.

3.6 Sublicense Fees.

3.6.1 In further consideration of the rights and licenses granted to Licensee under this Agreement, Licensee will pay Licensor **** of any sublicense fees (including upfront payments and milestone payments) received by Licensee or its Affiliates for the Licensed Commercial Patents from any Third Party Sublicensee or from any Third Party granted any option to obtain a sublicense.

3.6.2 With respect to the obligations under this Section 3.6, Licensee shall not be required to submit any amounts received from a Third Party for the following:

(a) Reimbursement for research, development, and/or manufacturing activities performed by Licensee or its Affiliates corresponding directly to the development of Licensed Products pursuant to a specific agreement;

(b) Consideration received for the purchase of an equity interest in Licensee or its Affiliates at fair market value or in the form of loans at commercially reasonable rates of interest; and

(c) Any and all amounts paid to Licensee or its Affiliates by a Third Party Sublicensee as royalties on sales of Licensed Product sold by such Sublicensee under a sublicense agreement.

3.6.3 If Licensee or its Affiliate receives sublicense fees from Third Party Sublicensees or from any Third Party granted any option to obtain a sublicense under this Agreement in the form of non-cash consideration, then, at Licensor's option, Licensee shall pay Licensor payments as required by this Section 3.6 (a) in the form of the non-cash consideration received by Licensee or its Affiliates or (b) a cash payment determined based on the fair market value of such non-cash consideration. If Licensee or its Affiliate enters into any sublicense with a Third Party Sublicensee that is not an arm's length transaction, fees due under this Section 3.6 will be calculated based on the fair market value of such transaction, at the time of the transaction, assuming an arm's length transaction made in the ordinary course of business, as determined jointly by Licensor and Licensee based on transactions of a similar type and standard industry practice, if any.

3.6.4 To the extent Licensee receives payment from a Third Party relating to one or more of the milestone events set forth in the table in Section 3.4, then the amount of the payment made to Licensor under such Section 3.4 with respect to such milestone event shall not be deemed sublicense fees under this Section 3.6; instead, the amounts due under this Section 3.6 shall be calculated by applying the sublicense fee rate set forth in Section 3.6.1 above to the sublicense fees received by Licensee from such Third Party after deducting the amount of the payment under Section 3.4.

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3.6.5 If a sublicense or option is part of a transaction in which Licensee or its Affiliates also licenses, sublicenses, or grants rights to technology, patent rights, or other intellectual property rights other than Licensed Patents, that portion of the consideration received by Licensee or its Affiliates and subject to this Section 3.6 shall be equitably apportioned between the Licensed Patents and those other rights, and such apportionment shall be reasonable and in accordance with customary standards in the industry. Licensee shall promptly deliver to Licensor a written report setting forth such apportionment and shall describe in reasonable detail the rationale for such allocation, together with a copy of all underlying documents necessary to determinate the basis and accuracy of such allocation. If Licensor disagrees with the determination made by Licensee, Licensor shall so notify Licensee within **** of receipt of Licensee's report, and the Parties shall meet to discuss and resolve such disagreement in good faith. If the Parties are unable to agree as to such apportionment within ****, then the matter shall be submitted in accordance with the dispute resolution process set forth in Section 10.6.

3.7 Reports and Records.

3.7.1 Licensee must deliver to Licensor within **** after the end of each Calendar Quarter after the first commercial sale of a Licensed Product a report setting forth the calculation of the royalties due to Licensor for such Calendar Quarter, including:

(a) Number of Licensed Products included within Net Sales, listed by country;

(b) Gross consideration for Net Sales of Licensed Product, including all amounts invoiced, billed, or received;

(c) Qualifying costs to be excluded from the gross consideration, as described in Section 1.19, listed by category of cost;

(d) Net Sales of Licensed Products listed by country;

(e) A detailed accounting of any royalty reductions applied pursuant to Section 3.5.1;

(f) Royalties owed to Licensor, listed by category; and

(g) The computations for any applicable currency conversions.

3.7.2 Licensee shall pay the royalties due under Section 3.5 within **** following the last day of the Calendar Quarter in which the royalties accrue. Licensee shall send the royalty payments along with the report described in Section 3.7.1.

3.7.3 Within **** after the occurrence of a milestone event described in Section 3.4, Licensee must deliver to Licensor a report describing the milestone event that occurred, together with a payment of the applicable amount due to Licensor pursuant to Section 3.4.

3.7.4 Within **** after the receipt of any fees from any Third Party as described in Section 3.6, Licensee must deliver to Licensor a report describing the fees received, together with a payment of the applicable amount due to Licensor pursuant to Section 3.6.

3.7.5 All financial reports under this Section 3.7 will be certified by the chief financial officer of Licensee.

3.7.6 Licensee shall maintain and require its Affiliates and all Sublicensees to maintain, complete and accurate books and records which enable the royalties, fees, and payments payable under this Agreement to be verified. The records must be maintained for **** after the submission of each report under Article 3. Upon reasonable prior written notice to Licensee, Licensee and its Affiliates and all Sublicensees will provide Licensor and/or the ReGenX Licensors (and their respective accountants) with access to all of the relevant books, records, and related background information required by this Section 3.7.6 to conduct a review or audit of the royalties, fees, and payments payable to Licensor under this Agreement to be verified. Access will be made available: (a) during normal business hours; (b) in a manner reasonably designed to facilitate the auditing party's review or audit without unreasonable disruption to Licensee's business; and (c) no more than once each calendar year during the term of this Agreement and for a period of **** thereafter. Licensee will promptly pay to Licensor the amount of any underpayment determined by the review or audit, plus accrued interest. If the review or audit determines that Licensee has underpaid any payment by **** or more, then Licensee will also promptly pay the costs and expenses of Licensor and the ReGenX Licensors and their respective accountants in connection with the review or audit.

3.8 Currency, Interest.

3.8.1 All dollar amounts referred to in this Agreement are expressed in United States dollars. All payments to Licensor under this Agreement must be made in United States dollars.

3.8.2 If Licensee receives payment in a currency other than United States dollars for which a royalty or fee or other payment is owed under this Agreement, then (a) the payment will be converted into United States dollars at the conversion rate for the foreign currency as published in the eastern edition of the *Wall Street Journal*, N.Y. edition, as of the last business day of the Calendar Quarter in which the payment was received by Licensee; and (b) the conversion computation will be documented by Licensee in the applicable report delivered to Licensor under Section 3.7.

3.8.3 All amounts that are not paid by Licensee when due will accrue interest from the date due until paid at a rate equal to 1.5% per month (or the maximum allowed by law, if less).

3.9 Taxes and Withholding.

3.9.1 All payments hereunder will be made free and clear of, and without deduction or deferment in respect of, and Licensee shall pay and be responsible for, and shall hold Licensor harmless from and against, any taxes, duties, levies, fees, or charges, including sales, use, transfer, excise, import, and value added taxes (including any interest, penalties, or additional amounts imposed with respect thereto) but excluding withholding taxes to the extent provided in Section 3.9.2. At the request of Licensee, Licensor will give Licensee such reasonable

assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable Licensee to pay and report and, as applicable, claim exemption from or reduction of, such tax, duty, levy, fee, or charge.

3.9.2 If any payment made by Licensee hereunder becomes subject to withholding taxes with respect to Licensor's gross or net income under the laws of any jurisdiction, Licensee will deduct and withhold the amount of such taxes for the account of Licensor to the extent required by law and will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to Licensor appropriate proof of payment of such withholding taxes. At the request of Licensor, Licensee will give Licensor such reasonable assistance, which will include the provision of appropriate certificates of such deductions made together with other supporting documentation as may be required by the relevant tax authority, to enable Licensor to claim exemption from or reduction of, or otherwise obtain repayment of, such withholding taxes, and will upon request provide such additional documentation from time to time as is reasonably required to confirm the payment of withholding tax.

ARTICLE 4: DILIGENCE

4.1 Diligence Obligations. If Licensee elects to exercise the Commercial Option granted to Licensee under Section 2.3 with respect to any Disease Indication, Licensee will use commercially reasonable efforts to develop, commercialize, market, promote, and sell at least one Licensed Product for each Disease Indication in the Commercial Field. Commercially reasonable efforts means efforts equivalent to those utilized by ****.

4.2 Reporting. Within **** after the Grant Date and within **** of each December 1 thereafter, Licensee shall provide Licensor with written progress reports, setting forth in such detail as Licensor may reasonably request, the progress of the development, evaluation, testing, and commercialization of each Licensed Product. Licensee will also notify Licensor within **** of the first commercial sale by Licensee, its Affiliates, or any Sublicensees of each Licensed Product. Such a report ("Development Progress Report"), setting forth the current stage of development of Licensed Products, shall include:

4.2.1 Date of Development Progress Report and time covered by such report;

4.2.2 Major activities and accomplishments completed by Licensee, its Affiliates, and any Sublicensees relating directly to the Licensed Product since the last Development Progress Report;

4.2.3 Significant research and development projects relating directly to the Licensed Product currently being performed by Licensee, its Affiliates, and any Sublicensees and projected dates of completion;

4.2.4 A development plan covering the next two years at least, which will include future development activities to be undertaken by Licensee, its Affiliates, or any Sublicensees during the next reporting period relating directly to the Licensed Product, Licensee's strategy to bring the Licensed Product to commercialization, and projected timeline for completing the necessary tasks to accomplish the goals of the strategy;

4.2.5 Projected total development remaining before product launch of each Licensed Product; and

4.2.6 Summary of significant development efforts using the Licensed Patents being performed by Third Parties, including the nature of the relationship between Licensee and such Third Parties.

4.3 Confidential Information. The Parties agree that Development Progress Reports shall be deemed Licensee's Confidential Information; provided that Licensor may share a copy of such reports with the ReGenX Licensors.

4.4 Improvements. Simultaneously with the Development Progress Report, Licensee shall deliver a detailed description of any Licensed Back Improvements, if not previously provided pursuant to Section 2.7.4.

ARTICLE 5: CONFIDENTIALITY

5.1 Treatment of Confidential Information. Each Party, as a receiving party (a "Receiving Party"), agrees that it will (a) treat Confidential Information of the other Party (the "Disclosing Party") as strictly confidential; (b) not disclose such Confidential Information to Third Parties without the prior written consent of the Disclosing Party, except as may be permitted in this Agreement; provided that any disclosure permitted hereunder be under confidentiality agreements with provisions substantially similar to those contained in this Agreement; and (c) not use such Confidential Information for purposes other than those authorized expressly in this Agreement. The Receiving Party agrees to ensure that its employees who have access to Confidential Information are obligated in writing to abide by confidentiality obligations substantially similar to those contained under this Agreement.

5.2 Public Announcements.

5.2.1 The Parties agree they will release a joint press release in the form attached hereto as Exhibit B. Except as provided in Section 5.2.2, any other press releases by either Party with respect to the other Party or any other public disclosures concerning the existence of or terms of this Agreement shall be subject to review and approval by the other Party. Once the joint press release or any other written statement is approved for disclosure by both Parties, either Party may make subsequent public disclosure of the contents of such statement without the further approval of the other Party.

5.2.2 Notwithstanding Section 5.2.1, Licensor has the right to publish (through press releases, scientific journals, or otherwise) and refer to any clinical, regulatory, or research results related to Licensee's Licensed Product or Specified Vector program that have been publicly

disclosed by Licensee, including referring to Licensee by name as a licensee of Licensor, which publication or referral by Licensor shall not require the prior consent of Licensee.

5.3 Authorized Disclosure. Notwithstanding the provisions of Section 5.1 or 5.2, either Party may disclose Confidential Information or make such a disclosure of the existence of and/or terms of this Agreement to any ****; provided that, in each case, such recipient of Confidential Information is obligated to keep such information confidential on terms substantially similar to those set forth in this Agreement. Furthermore, Licensee agrees that Licensor may share a copy of this Agreement, reports and notices provided by Licensee to Licensor pursuant to the terms of this Agreement, and copies of sublicense agreements provided to Licensor hereunder with the ReGenX Licensors. In the event that the Receiving Party receives service of legal process that purports to compel disclosure of the Disclosing Party's Confidential Information or becomes obligated by law to disclose the Confidential Information of the Disclosing Party or the existence of or terms of this Agreement to any governmental authority, the Receiving Party shall promptly notify the Disclosing Party, so that the Disclosing Party may seek an appropriate protective order or other remedy with respect to narrowing the scope of such requirement and/or waive compliance by the Receiving Party with the provisions of this Agreement. The Receiving Party will provide the Disclosing Party, at the Disclosing Party's expense, with reasonable assistance in obtaining such protective order or other remedy. If, in the absence of such protective order or other remedy, the Receiving Party is nonetheless required by law to disclose the existence of or terms of this Agreement or other Confidential Information of the Disclosing Party, the Receiving Party may disclose such Confidential Information without liability hereunder; provided that the Receiving Party shall furnish only such portion of the Confidential Information that is legally required to be disclosed and only to the extent required by law.

5.4 Term of Confidentiality. The obligations of this Article 5 shall continue for a period of **** following the expiration or termination of this Agreement.

ARTICLE 6: TERM AND TERMINATION

6.1 Term of Agreement. This Agreement, unless sooner terminated as provided in this Agreement, expires upon the expiration, lapse, abandonment, or invalidation of the last Valid Claim of the Licensed Commercial Patents to expire, lapse, or become abandoned or unenforceable in all the countries of the world.

6.2 Termination for Failure to Exercise Option. This Agreement will terminate automatically at the end of the Research Term if Licensee does not exercise the Commercial Option with respect to any Disease Indication in accordance with Section 2.3. If Licensee does not exercise the Commercial Option with respect to all Disease Indications, this Agreement will terminate with respect to all unexercised Disease Indications at the end of the Research Term.

6.3 Licensee's Right to Terminate. Licensee may, upon **** prior written notice to Licensor, terminate this Agreement for any reason, with or without cause. In exercising such termination right, Licensee may terminate the Agreement in its entirety or, if desired, Licensee

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may specify in the written notice that this Agreement is terminating only with respect to one or more of the Disease Indications within the Research Field or Commercial Field, as applicable.

6.4 Termination for Breach.

6.4.1 Licensor may terminate this Agreement, if Licensee is late in paying to Licensor royalties, fees, or any other monies due under this Agreement, and Licensee does not pay Licensor in full within **** upon written demand from Licensor, which termination shall be effective immediately upon the expiration of such **** cure period.

6.4.2 Either Party may terminate this Agreement, if the other Party materially breaches this Agreement and does not cure such material breach within **** after written notice of the breach, which termination shall be effective immediately upon the expiration of such **** cure period.

6.5 Termination for Insolvency.

6.5.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if Licensee, any of its Affiliates, or any Sublicensees experiences any Trigger Event.

6.5.2 For purposes of this Section 6.5, "Trigger Event" means any of the following: (a) if Licensee, any Affiliate, or any Sublicensee, as applicable, (i) becomes insolvent, becomes bankrupt, or generally fails to pay its debts as such debts become due, (ii) is adjudicated insolvent or bankrupt, (iii) admits in writing its inability to pay its debts, (iv) suffers the appointment of a custodian, receiver, or trustee for it or its property and, if appointed without its consent, is not discharged within ****, (v) makes an assignment for the benefit of creditors, or (vi) suffers proceedings being instituted against it under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors and, if contested by it, not dismissed or stayed within ****; (b) the institution or commencement by Licensee, any Affiliate, or any Sublicensee, as applicable, of any proceeding under any law related to bankruptcy, insolvency, liquidation, or the reorganization, readjustment, or release of debtors; (c) the entering of any order for relief relating to any of the proceedings described in Section 6.5.2(a) or (b) above; (d) the calling by Licensee, any Affiliate, or any Sublicensee, as applicable, of a meeting of its creditors with a view to arranging a composition or adjustment of its debts; or (e) the act or failure to act by Licensee, any Affiliate, or any Sublicensee, as applicable, indicating its consent to, approval of, or acquiescence in any of the proceedings described in Section 6.5.2(b) through (d) above.

6.6 Patent Challenge.

6.6.1 Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, upon the commencement by Licensee or any of its Affiliates of a Patent Challenge. Licensee shall include in each sublicense agreement entered into with a Sublicensee a right of Licensee to terminate such sublicense agreement if such Sublicensee commences a Patent Challenge; and Licensee shall terminate the sublicense agreement, effective immediately upon written notice to the Sublicensee, if the Sublicensee commences a Patent Challenge. In addition, if the Sublicensee's commencement of a Patent Challenge gives The Trustees of the

University of Pennsylvania (or any successor thereto under the Penn Agreement) a right of termination under the Penn Agreement, then, upon receipt of notice from the Trustees of the University of Pennsylvania, Licensor may terminate this Agreement, effective immediately upon written notice to Licensee, if any Sublicensee commences a Patent Challenge. If Licensor obtains actual knowledge of a Patent Challenge commenced by a Sublicensee, Licensor shall use commercially reasonable efforts to provide Licensee with written notice of such Patent Challenge; provided that Licensor's failure to provide such notice will not affect Licensee's obligations hereunder.

6.6.2 For purposes of this Section 6.6, "Patent Challenge" means any action against Licensor, The Trustees of the University of Pennsylvania, or the ReGenX Licensors, including an action for declaratory judgment, to declare or render invalid or unenforceable the Licensed Patents, or any claim thereof.

6.7 Effects of Termination. The effect of termination pursuant to Section 6.2, by Licensee pursuant to Section 6.3, by either Party, as applicable, under Section 6.4, or by Licensor pursuant to Section 6.5 or 6.6 shall be as follows:

6.7.1 The licenses granted by Licensor hereunder shall terminate, and Licensee, its Affiliates, and (unless the sublicense agreement is assigned pursuant to Section 6.7.2) all Sublicensees shall cease to make, have made, use, import, sell, and offer for sale all AAV Materials or Licensed Products and shall cease to otherwise practice the Licensed Patents; provided that Licensee, its Affiliates, and Sublicensees shall have the right to continue to sell their existing inventories of Licensed Products for a period not to exceed **** after the effective date of such termination;

6.7.2 Licensee shall assign to Licensor any or all sublicenses granted to Third Parties to the extent of the rights licensed to Licensee hereunder and sublicensed to the Sublicensee; provided that (i) prior to such assignment, Licensee shall advise Licensor whether such Sublicensee is then in full compliance with all terms and conditions of its sublicense and continues to perform thereunder, and, if such Sublicensee is not in full compliance or is not continuing to perform, Licensor may elect not to have such sublicense assigned, in which event such sublicense shall terminate; (ii) such Sublicensee must agree in writing to assume Licensee's terms, conditions, and obligations to Licensor set forth in this Agreement, including all payment obligations; and (iii) following such assignment, Licensor shall not be liable to such Sublicensee with respect to any obligations of Licensee to the Sublicensee that are not consistent with, or not required by, Licensor's obligations to Licensee under this Agreement; and all sublicenses not assigned to Licensor as provided in this Section 6.7.2 shall terminate;

6.7.3 If termination is by Licensee pursuant to Section 6.3 or by Licensor pursuant to Section 6.4, 6.5, or 6.6, then, effective as of such termination of this Agreement, Licensee shall grant, and hereby grants, to Licensor a non-exclusive, perpetual, irrevocable, worldwide, royalty-free, transferable, sublicensable license under any patentable modifications or improvements (and any intellectual property rights with respect thereto) developed, during the term of this Agreement, by Licensee, any Affiliates, or any Sublicensees to any vector that is the subject of a claim within any of the Licensed Patents, for use by Licensor (and its sublicensees) for the research, development, and commercialization of products in any therapeutic indication;

provided that the categorization of patentable modifications or improvements that are subject to this Section 6.7.3 will be subject to the same exclusions applicable to "Licensed Back Improvements" under Sections 2.7.5 and 2.7.6.

6.7.4 Licensee shall pay all monies then-owed to Licensor under this Agreement;

6.7.5 Each Receiving Party shall, at the other Party's request, return all Confidential Information of the Disclosing Party. Notwithstanding the foregoing, one copy may be kept by either Party for a record of that Party's obligations; and

6.7.6 If termination is only with respect to a particular Disease Indication within the Research Field or the Commercial Field, but not all Disease Indications, then the provisions of this Section 6.7 shall only apply with respect to the terminated Disease Indications, and this Agreement shall continue as provided herein with respect to the non-terminated Disease Indications.

6.8 Survival. Licensee's obligation to pay all monies due and owed to Licensor under this Agreement which have matured as of the effective date of termination or expiration shall survive the termination or expiration of this Agreement. In addition, the provisions of Article 1 (Definitions), Section 2.4, (Retained Rights), Section 2.5 (Government Rights), Section 2.7 (Improvements), Section 3.1 (Initial Fee), Article 3 (Consideration) (with respect to any final reports or to the extent any amounts are due but unpaid), Section 3.7 (Reports and Records), Article 5 (Confidentiality), Section 6.7 (Effects of Termination), Section 6.8 (Survival), Section 8.3 (Disclaimer of Warranties, Damages), Section 8.4 (Indemnification), Section 8.5 (Insurance), Article 9 (Use of Name), and Article 10 (Additional Provisions) shall survive such termination or expiration of this Agreement in accordance with their respective terms.

ARTICLE 7: PATENT MAINTENANCE; PATENT INFRINGEMENT

7.1 Prosecution of Licensed Patents. As between Licensor and Licensee, but subject to any obligations of Licensor to the ReGenX Licensors, the Parties agree as follows:

7.1.1 Licensor shall have the sole right, but not the obligation, to Prosecute patent applications and issued patents within Licensed Patents, in Licensor's sole discretion.

7.1.2 Nothing in this Agreement obligates Licensor to continue to Prosecute any patent applications or issued patents, and Licensee acknowledges that Licensor shall have no obligation to undertake any inter-party proceedings, such as oppositions or interferences, or to undertake any re-examination or re-issue proceedings, in either case, with respect to the Licensed Patents.

7.2 Infringement Actions Against Third Parties.

7.2.1 Licensee is responsible for notifying Licensor promptly of any infringement of Licensed Patents within the Disease Indications (other than Retained Rights) that may come to Licensee's attention.

7.2.2 As between Licensor and Licensee, but subject to any obligations of Licensor to the ReGenX Licensors, Licensor shall have the sole right, but not the obligation, to prosecute any

such infringement **** recovered in connection therewith. In any action to enforce any of the Licensed Patents, Licensee, at the request and expense of Licensor, shall cooperate to the fullest extent reasonably possible. Nothing in this Agreement obligates Licensor to bring or prosecute lawsuits against Third Parties for infringement of any Licensed Patents.

7.2.3 Licensee shall have no right to undertake prosecution of any such infringement.

7.3 Defense of Infringement Claims. In the event Licensee or Licensor becomes aware that Licensee's or any of its Affiliates' or any Sublicensees' practice of the Licensed Patents is the subject of a claim for patent infringement by a Third Party, that Party shall promptly notify the other, and the Parties shall consider the claim and the most appropriate action to take. Licensee shall cause each of its Affiliates and each Sublicensee to notify Licensee promptly in the event such entity becomes aware that its practice of the Licensed Patents is the subject of a claim of patent infringement by another. To the extent Licensor takes any action, Licensor (or the ReGenX Licensors) shall have the right to require Licensee's reasonable cooperation in any such suit, upon written notice to Licensee; and Licensee shall have the obligation to participate upon Licensor's request, in which event, Licensor shall bear the cost of Licensee's participation. Without Licensor's prior written permission, Licensee must not settle or compromise any such suit in a manner that imposes any material obligations or restrictions on Licensor or the ReGenX Licensors or grants any rights to the Licensed Patents other than rights that Licensee has the right to grant under this Agreement.

ARTICLE 8: REPRESENTATIONS AND WARRANTIES; INDEMNIFICATION

8.1 Representations and Warranties by Licensor. Licensor represents and warrants to Licensee as of the Effective Date:

8.1.1 Licensor has the right, power, and authority to enter into this Agreement and to grant to Licensee the licenses specified in this Agreement;

8.1.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.1.3 There are no actions, suits, proceedings, or arbitrations pending or, to Licensor's knowledge, threatened against Licensor relating to the Licensed Research Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.1.4 To Licensor's Knowledge, Licensor has not received any written notice from the ReGenX Licensors informing Licensor that there are any actions, suits, proceedings, or arbitrations pending against the ReGenX Licensors relating to the Licensed Research Patents that would be inconsistent with the rights granted to Licensee under this Agreement;

8.1.5 To Licensor's knowledge, (a) the Licensed Research Patents are solely owned by the Trustees of the University of Pennsylvania, and (b) no Third Party (other than the ReGenX Licensors) has any right, interest, or claim in or to such Licensed Research Patents with respect to the Disease Indications that are inconsistent with those granted to Licensee with respect to the Disease Indications;

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8.1.6 To Licensor's knowledge, GSK Agreement and Penn Agreement are in full force and effect;

8.1.7 To Licensor's knowledge, no Third Party is infringing any of the Licensed Research Patents in a manner that is inconsistent with the scope of rights granted to Licensee with respect to the Disease Indications; and

8.1.8 Licensor has not received any written notice from any Third Party patentee alleging infringement of such Third Party's patents by the practice of the Licensed Research Patents with respect to the Disease Indications.

8.2 Representations and Warranties by Licensee. Licensee represents and warrants to Licensor as of the Effective Date that:

8.2.1 Licensee has the right, power, and authority to enter into this Agreement and to grant the licenses granted by it hereunder;

8.2.2 This Agreement when executed shall become the legal, valid, and binding obligation of it, enforceable against it, in accordance with its terms;

8.2.3 Licensee has the ability and the resources, including financial resources, necessary to carry out its obligations under this Agreement; and

8.2.4 There are no actions, suits, proceedings, or arbitrations pending or, to Licensee's knowledge, threatened against Licensee that would impact activities under this Agreement.

8.3 Disclaimer of Warranties, Damages.

8.3.1 EXCEPT AS SET FORTH IN SECTION 8.1, THE LICENSED PATENTS, AAV MATERIALS, LICENSED PRODUCTS, AND ALL RIGHTS LICENSED UNDER THIS AGREEMENT ARE PROVIDED ON AN "AS IS" BASIS, AND LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, EXPRESS OR IMPLIED, WITH RESPECT THERETO. BY WAY OF EXAMPLE BUT NOT OF LIMITATION, LICENSOR MAKES NO REPRESENTATIONS OR WARRANTIES, AND HEREBY DISCLAIMS ALL EXPRESS AND IMPLIED REPRESENTATIONS AND WARRANTIES, (i) OF COMMERCIAL UTILITY, ACCURACY, COMPLETENESS, PERFORMANCE, MERCHANTABILITY, FITNESS FOR A PARTICULAR PURPOSE, VALIDITY OR ENFORCEABILITY OF THE LICENSED PATENTS, AND PROFITABILITY; OR (ii) THAT THE USE OF THE LICENSED PATENTS, AAV MATERIALS, OR LICENSED PRODUCTS WILL NOT INFRINGE ANY PATENT, COPYRIGHT, TRADEMARK, OR OTHER PROPRIETARY RIGHTS OF THIRD PARTIES.

8.3.2 EXCEPT AS SET FORTH HEREIN, NONE OF LICENSOR OR THE REGENX LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO ANY CLAIM ARISING FROM USE OF THE LICENSED PATENTS, AAV MATERIALS, LICENSED PRODUCTS, AND ANY OR ALL RIGHTS LICENSED UNDER THIS

AGREEMENT OR FROM THE DEVELOPMENT, TESTING, MANUFACTURE, USE, OR SALE OF AAV MATERIALS OR LICENSED PRODUCTS

8.3.3 NEITHER PARTY SHALL BE LIABLE TO THE OTHER PARTY OR ITS SUCCESSORS OR ASSIGNS, ANY SUBLICENSEE, OR THIRD PARTY AND NEITHER OF THE REGENX LICENSORS SHALL BE LIABLE TO LICENSEE, LICENSEE'S SUCCESSORS OR ASSIGNS, ANY SUBLICENSEES, OR ANY THIRD PARTY WITH RESPECT TO ANY CLAIM FOR LOSS OF PROFITS, LOSS OR INTERRUPTION OF BUSINESS, OR FOR INDIRECT, SPECIAL, INCIDENTAL, PUNITIVE, OR CONSEQUENTIAL DAMAGES OF ANY KIND, INCLUDING ANY ARISING FROM OR RELATING TO ANY BREACH OF THIS AGREEMENT OR THE EXERCISE OF RIGHTS HEREUNDER, REGARDLESS OF ANY NOTICE OF SUCH DAMAGES; PROVIDED THAT NOTHING IN THIS SECTION 8.3.3 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 8.4 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER ARTICLE 5.

8.4 Indemnification.

8.4.1 By Licensee. Licensee shall defend, indemnify, and hold harmless Licensor, the ReGenX Licensors, and their respective shareholders, members, officers, directors, trustees, faculty, students, contractors, agents, and employees (individually, a "Licensor Indemnified Party" and, collectively, the "Licensor Indemnified Parties") from and against any and all Third Party liability, loss, damage, action, claim, fee, cost, or expense (including attorneys' fees) (individually, a "Third Party Liability" and, collectively, the "Third Party Liabilities") suffered or incurred by the Licensor Indemnified Parties from claims of such Third Parties that result from or arise out of: ****; provided, however, that Licensee shall not be liable for claims to the extent based on any breach by Licensor of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensor Indemnified Parties. Without limiting the foregoing, Licensee must defend, indemnify, and hold harmless the Licensor Indemnified Parties from and against any Third Party Liabilities resulting from:

(a) any **** or other claim of any kind related to the **** by a Third Party of a Licensed Product that was **** by Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors;

(b) any claim by a Third Party that the ****; and

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(c) **** conducted by or on behalf of Licensee, its Affiliates, any Sublicensees, their respective assignees, or vendors relating to the Licensed Patents, AAV Materials, or Licensed Products, including any claim by or on ****.

8.4.2 By Licensor. Licensor shall defend, indemnify, and hold harmless Licensee, its shareholders, members, officers, directors, contractors, agents, and employees (individually, a "Licensee Indemnified Party" and, collectively, the "Licensee Indemnified Parties") from and against any and all Third Party Liabilities suffered or incurred by the Licensee Indemnified Parties from claims of such Third Parties to the extent that such claims result from or arise out of the ****; provided, however, that Licensor shall not be liable for claims to the extent based on any breach by Licensee of the representations, warranties, or obligations of this Agreement or the gross negligence or intentional misconduct of any of the Licensee Indemnified Parties.

8.4.3 Indemnification Procedure. Each Party, as an indemnifying party (an "Indemnifying Party"), shall not be permitted to settle or compromise any claim or action giving rise to Third Party Liabilities in a manner (a) that imposes any restrictions or obligations on any indemnified party (an "Indemnified Party") without the Indemnified Party's prior written consent, (b) if Licensee is the Indemnifying Party, that imposes any restrictions or obligations on the ReGenX Licensors or grants any rights to the Licensed Patents, AAV Materials, or Licensed Products other than those Licensee has the right to grant under this Agreement without Licensor's prior written consent, or (c) if Licensor is the Indemnifying Party, that grants any rights to the Licensed Back Improvements other than those Licensor has the right to grant under this Agreement without Licensee's prior written consent. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving the defense of any claim subject to indemnification pursuant to this Section 8.4, including the selection of counsel, with the reasonable approval of the Indemnified Party. If an Indemnifying Party fails or declines to assume the defense of any such claim or action within **** after notice thereof, the Indemnified Party may assume the defense of such claim or action at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be conclusively deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnifying Party will pay directly all Third Party Liabilities incurred for defense or negotiation of any claim hereunder or will reimburse the Indemnified Party for all documented Third Party Liabilities incident to the defense or negotiation of any such claim within **** after the Indemnifying Party's receipt of invoices for such fees, expenses, and charges.

8.5 Insurance. Licensee will procure and maintain insurance policies for the following coverages with respect to product liability, personal injury, bodily injury, and property damage arising out of Licensee's (and its Affiliates' and any Sublicensees') performance under this Agreement: (a) during the term of this Agreement, comprehensive general liability, including broad form and contractual liability, in a minimum amount of **** combined single limit

per occurrence (or claim) and in the aggregate annually; (b) prior to the commencement of clinical trials involving Licensed Products and thereafter for a period of not less than **** (or such longer period as Licensee is required by applicable law to continue to monitor the participants in the clinical trial), clinical trials coverage in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually; and (c) from prior to the first commercial sale of a Licensed Product until **** after the last sale of a Licensed Product, product liability coverage, in amounts that are reasonable and customary in the U.S. pharmaceutical industry, subject always to a minimum limit of **** combined single limit per occurrence (or claim) and in the aggregate annually. Licensor may review periodically the adequacy of the minimum amounts of insurance for each coverage required by this Section 8.5, and Licensor reserves the right to require Licensee to adjust the limits accordingly. The required minimum amounts of insurance do not constitute a limitation on Licensee's liability or indemnification obligations to the Licensor Indemnified Parties under this Agreement. The policies of insurance required by this Section 8.5 will be issued by an insurance carrier with an A.M. best rating of **** or better and will name Licensor as an additional insured with respect to Licensee's performance (and its Affiliates' and any Sublicensees') under this Agreement. Licensee will provide Licensor with insurance certificates evidencing the required coverage within **** after the Effective Date and the commencement of each policy period and any renewal periods. Each certificate will provide that the insurance carrier will notify Licensor in writing at least **** prior to the cancellation or material change in coverage. Licensee will cause all Sublicensees to comply with the terms of this Section 8.5 to the same extent as Licensee.

ARTICLE 9: USE OF NAME

Licensee, its Affiliates, any Sublicensees, and all of its and their employees and agents must not use Licensor's, the University of Pennsylvania's, or SmithKline Beecham Corporation's name, seal, logo, trademark, or service mark (or any adaptation thereof) or the name, seal, logo, trademark, or service mark (or any adaptation thereof) of any of such entities' representative, school, organization, employee, or student in any way without the prior written consent of Licensor or such entity, as applicable; provided, however that Licensee may acknowledge the existence and general nature of this Agreement, subject to Section 5.3.

ARTICLE 10: ADDITIONAL PROVISIONS

10.1 Relationship. Nothing in this Agreement shall be deemed to establish a relationship of principal and agent between Licensee and Licensor, nor any of their agents or employees for any purpose whatsoever, nor shall this Agreement be construed as creating any other form of legal association or arrangement which would impose liability upon one Party for the act or failure to act of the other Party.

10.2 Assignment. The rights and obligations of Licensee and Licensor hereunder shall inure to the benefit of, and shall be binding upon, their respective permitted successors and assigns. Licensee may not assign this Agreement or any of its rights or obligations under this Agreement

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without the prior written consent of Licensor, which consent may not be unreasonably withheld, conditioned, or delayed. Notwithstanding the foregoing, Licensee may assign this Agreement without Licensor's consent, (a) to an Affiliate; provided that such Affiliate will continue to have to perform under Section 4.1 with at least the same level of efforts that Licensee would have been required to exercise; or (b) pursuant to a sale or merger of Licensee or the transfer of substantially all of the assets of Licensee's business to which this Agreement relates (whether by sale, merger, reorganization, consolidation, or otherwise); provided that, as part of any permitted assignment, (i) Licensee provides Licensor with written notice of such assignment at least five business days prior to the effectiveness of such assignment; (ii) Licensee requires any such assignee to agree in writing to be legally bound by this Agreement to the same extent as Licensee and provides Licensor with a copy of such assignee undertaking; and (iii) if such assignment is to an Affiliate, Licensee remains responsible for the performance of this Agreement by such Affiliate. An assignment to an Affiliate will terminate, and all rights assigned will revert to Licensee, if and when such Affiliate ceases to be an Affiliate of Licensee, and Licensee will provide Licensor written notice of such assignment within five business days of such event. In addition, Licensee will provide Licensor with written notice of any Change of Control (for purposes of this Agreement, the term "Change of Control" means the acquisition by a person or group of "control" of Licensee, as defined in Section 1.3, whether or not the person or group acquiring control would be deemed an "Affiliate" under such Section 1.3) of Licensee at least five business days prior to the effectiveness of such Change of Control. Licensor may assign this Agreement and its rights and obligations without the consent of Licensee. No assignment shall relieve the assigning Party of responsibility for the performance of any accrued obligations which it has prior to such assignment. Any attempted assignment by Licensee in violation of this Section 10.2 shall be null and void and of no legal effect.

10.3 Waiver. A waiver by either Party of a breach of any provision of this Agreement will not constitute a waiver of any subsequent breach of that provision or a waiver of any breach of any other provision of this Agreement.

10.4 Notices. Notices, payments, statements, reports, and other communications under this Agreement shall be in writing and shall be deemed to have been received as of the date received if sent by public courier (*e.g.*, Federal Express), sent by Express Mail, receipt requested, delivered in person, or sent by facsimile (with a copy of such facsimile also sent by one of the other methods of delivery) and addressed as follows:

If for Licensor:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: Chief Executive Officer
Telephone: 202-785-7438
Facsimile: 202-785-7439

with a copy to:

ReGenX Biosciences, LLC
750 17th Street, NW
Suite 1100
Washington, DC 20006
USA
Attn: General Counsel
Telephone: 202-785-7438
Facsimile: 202-785-7439

If for Licensee:

Voyager Therapeutics, Inc.
75 Sidney Street
Cambridge, MA 02139
Attn: Chief Executive Officer
Telephone: 857-259-5340
Facsimile: 617-621-2971

Either Party may change its official address upon written notice to the other Party.

10.5 Applicable Law. This Agreement shall be construed and governed in accordance with the laws of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. Subject to Section 10.6, the Parties hereby submit to the exclusive jurisdiction of and venue in the courts located in the State of Delaware with respect to any and all disputes concerning the subject of this Agreement.

10.6 Dispute Resolution. In the event of any controversy or claim arising out of or relating to this Agreement, the Parties shall first attempt to resolve such controversy or claim through good faith negotiations for a period of not less than **** following notification of such controversy or claim to the other Party. If such controversy or claim cannot be resolved by means of such negotiations during such period, then such controversy or claim shall be resolved by binding arbitration administered by the American Arbitration Association ("AAA") in accordance with the Commercial Arbitration Rules of the AAA in effect on the date of commencement of the arbitration, subject to the provisions of this Section 10.6. The arbitration shall be conducted as follows:

10.6.1 The arbitration shall be conducted by three arbitrators, each of whom by training, education, or experience has knowledge of the research, development, and commercialization of biological therapeutic products in the United States. The arbitration shall be conducted in English and held in New York, New York.

10.6.2 In its demand for arbitration, the Party initiating the arbitration shall provide a statement setting forth the nature of the dispute, the names and addresses of all other parties, an estimate of the amount involved (if any), the remedy sought, otherwise specifying the issue to be resolved, and appointing one neutral arbitrator. In an answering statement to be filed by the responding Party within **** after confirmation of the notice of filing of the demand is sent by the AAA, the responding Party shall appoint one neutral arbitrator. Within **** from the date on which the responding Party appoints its neutral arbitrator, the first two arbitrators shall appoint a chairperson.

10.6.3 If a Party fails to make the appointment of an arbitrator as provided in Section 10.6.2, the AAA shall make the appointment. If the appointed arbitrators fail to appoint a chairperson within the time specified in Section 10.6.2 and there is no agreed extension of time, the AAA shall appoint the chairperson.

10.6.4 The arbitrators will render their award in writing and, unless all Parties agree otherwise, will include an explanation in reasonable detail of the reasons for their award. Judgment upon the award rendered by the arbitrators may be entered in any court having

jurisdiction thereof, including in the courts described in Section 10.5. The arbitrators will have the authority to grant injunctive relief and other specific performance; provided that the arbitrators will have no authority to award damages in contravention of this Agreement, and each Party irrevocably waives any claim to such damages in contravention of this Agreement. The arbitrators will, in rendering their decision, apply the substantive law of the State of Delaware, without giving effect to conflict of law provisions that may require the application of the laws of another jurisdiction. The decision and award rendered by the arbitrators will be final and non-appealable (except for an alleged act of corruption or fraud on the part of the arbitrator).

10.6.5 The Parties shall use their reasonable efforts to conduct all dispute resolution procedures under this Agreement as expeditiously, efficiently, and cost-effectively as possible.

10.6.6 All expenses and fees of the arbitrators and expenses for hearing facilities and other expenses of the arbitration will be borne equally by the Parties unless the Parties agree otherwise or unless the arbitrators in the award assess such expenses against one of the Parties or allocate such expenses other than equally between the Parties. Each of the Parties will bear its own counsel fees and the expenses of its witnesses except to the extent otherwise provided in this Agreement or by applicable law.

10.6.7 Compliance with this Section 10.6 is a condition precedent to seeking relief in any court or tribunal in respect of a dispute, but nothing in this Section 10.6 will prevent a Party from seeking equitable or other interlocutory relief in the courts of appropriate jurisdiction, pending the arbitrators' determination of the merits of the controversy, if applicable to protect the confidential information, property, or other rights of that Party or to otherwise prevent irreparable harm that may be caused by the other Party's actual or threatened breach of this Agreement.

10.7 No Discrimination. Licensee, its Affiliates, and any Sublicensees, in their respective activities under this Agreement, shall not discriminate against any employee or applicant for employment because of race, color, sex, sexual, or affectional preference, age, religion, national, or ethnic origin, handicap, or because he or she is a disabled veteran or a veteran (including a veteran of the Vietnam Era).

10.8 Compliance with Law. Licensee (and its Affiliates' and any Sublicensees') must comply with all prevailing laws, rules, and regulations that apply to its activities or obligations under this Agreement. Without limiting the foregoing, it is understood that this Agreement may be subject to United States laws and regulations controlling the export of technical data, computer software, laboratory prototypes, and other commodities, articles, and information, including the Arms Export Control Act as amended in the Export Administration Act of 1979 and that Licensee's obligations are contingent upon compliance with applicable United States export laws and regulations. The transfer of certain technical data and commodities may require a license from the cognizant agency of the United States Government and/or written assurances by Licensee that Licensee shall not export data or commodities to certain foreign countries without prior approval of such agency. Licensor neither represents that a license is not required nor that, if required, it will issue.

10.9 Entire Agreement. This Agreement embodies the entire understanding between the Parties relating to the subject matter hereof and supersedes all prior understandings and agreements, whether written or oral, including that certain Mutual Confidentiality Agreement effective as of December 12, 2013 between the Parties. All "Confidential Information" disclosed by the Parties pursuant to such Mutual Confidentiality Agreement shall be deemed "Confidential Information" under this Agreement (unless and until it falls within one of the exclusions set forth in Section 1.7). This Agreement may not be varied except by a written document signed by duly authorized representatives of both Parties.

10.10 Marking. Licensee, its Affiliates, and any Sublicensees shall mark any Licensed Product (or their containers or labels) made, sold, or otherwise distributed by it or them under this Agreement with any notice of patent rights necessary or (to the extent commercially feasible and consistent with prevailing business practices) desirable under applicable law to enable the Licensed Commercial Patents to be enforced to their full extent in any country where Licensed Products are made, used, sold, offered for sale, or imported.

10.11 Severability and Reformation. If any provision of this Agreement is held to be invalid or unenforceable by a court of competent jurisdiction, then such invalid or unenforceable provision will be automatically revised to be a valid or enforceable provision that comes as close as permitted by law to the Parties' original intent; provided that, if the Parties cannot agree upon such valid or enforceable provision, the remaining provisions of this Agreement will remain in full force and effect, unless the invalid or unenforceable provisions are of such essential importance to this Agreement that it is to be reasonably assumed that the Parties would not have entered into this Agreement without the invalid or unenforceable provisions.

10.12 Further Assurances. Each Party hereto agrees to execute, acknowledge, and deliver such further instruments, and to do all other acts, as may be necessary or appropriate in order to carry out the purposes and intent of this Agreement.

10.13 Interpretation; Construction. The captions to the several Articles and Sections of this Agreement are included only for convenience of reference and shall not in any way affect the construction of, or be taken into consideration in interpreting, this Agreement. In this Agreement, unless the context requires otherwise, (a) the word "including" shall be deemed to be followed by the phrase "without limitation" or like expression; (b) references to the singular shall include the plural and vice versa; (c) references to masculine, feminine, and neuter pronouns and expressions shall be interchangeable; (d) the words "herein" or "hereunder" relate to this Agreement; (e) "or" is disjunctive but not necessarily exclusive; (f) the word "will" shall be construed to have the same meaning and effect as the word "shall"; (g) all references to "dollars" or "$" herein shall mean U.S. Dollars; (h) unless otherwise provided, all reference to Sections, Articles, and exhibits in this Agreement are to Sections, Articles, and exhibits of and in this Agreement; and (i) whenever this Agreement refers to a number of days, such number shall refer to calendar days unless business days are specified. Business days shall mean a day on which banking institutions in Washington, D.C. are open for business. Each Party represents that it has been represented by legal counsel in connection with this Agreement and acknowledges that it has participated in the drafting hereof. In interpreting and applying the terms and provisions of this Agreement, the Parties agree that no presumption will apply against the Party which drafted such terms and provisions.

10.14 Cumulative Rights and Remedies. The rights and remedies provided in this Agreement and all other rights and remedies available to either Party at law or in equity are, to the extent permitted by law, cumulative and not exclusive of any other right or remedy now or hereafter available at law or in equity. Neither asserting a right nor employing a remedy shall preclude the concurrent assertion of any other right or employment of any other remedy, nor shall the failure to assert any right or remedy constitute a waiver of that right or remedy.

10.15 Counterparts. This Agreement may be executed in one or more counterparts, each of which will be deemed an original, but all of which together will constitute one and the same instrument.

[REMAINDER OF PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the Parties, intending to be legally bound, have caused this License Agreement to be executed by their duly authorized representatives.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President & CEO

VOYAGER THERAPEUTICS, INC.

By: Mark Levin
Name: Mark Levin
Title: CEO

CONFIDENTIAL TREATMENT REQUESTED

Exhibit A
Licensed Research Patents

Application #	Title	Inventors	Nos.	Penn Docket #
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****
****	****	****	****	****

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Exhibit B
Press Release

REGENX BIOSCIENCES AND VOYAGER THERAPEUTICS ANNOUNCE LICENSE AGREEMENT

- Voyager acquires rights to REGENX's proprietary NAV® vectors in multiple CNS disorders
    - Eighth third-party commercial license of REGENX's NAV vectors since 2010
- REGENX to receive undisclosed upfront payment, milestones and royalties in exchange for non-exclusive worldwide license

WASHINGTON, DC and CAMBRIDGE, Mass. June 2, 2014 – REGENX Biosciences, LLC and Voyager Therapeutics today announced that they have entered into a license agreement for use of REGENX's proprietary NAV® vectors for the development and commercialization of gene therapies to treat Amyotrophic Lateral Sclerosis (ALS), Friedreich's ataxia (FA) and Huntington's disease (HD).

Under the terms of the agreement, REGENX has granted Voyager a non-exclusive worldwide license, as well as sublicensing rights, to REGENX's NAV vectors for the treatment of ALS, FA and HD. In exchange for these rights, REGENX will receive an undisclosed upfront payment, ongoing fees, milestone payments, and royalties on net sales of products incorporating NAV vectors. REGENX will also receive a share of certain sublicensing revenues.

"This license agreement serves as further validation of our proprietary NAV vector technology platform, and is an important step towards the successful development of NAV-based gene delivery treatments for patients afflicted with the serious and debilitating rare diseases to which Voyager is committed," said Ken Mills, President and CEO of REGENX. "As the leader in next-generation AAV gene therapy, REGENX is pleased to be collaborating with Voyager, which is well-positioned to develop innovative treatments through the application of our NAV technology."

Mark Levin, Interim CEO of Voyager, commented, "Voyager is the leading AAV gene therapy company focused on developing life-changing treatments for patients with devastating CNS disorders. We are committed to advancing the AAV gene therapy field via broad-based investment in a number of key technological areas. In addition to providing a valuable addition to Voyager's intellectual property portfolio, the rights to use REGENX's NAV vectors will position us to rapidly advance the development of breakthrough CNS gene therapies."

About Amyotrophic Lateral Sclerosis

Amyotrophic Lateral Sclerosis (ALS), also known as Lou Gehrig's disease, is a progressive, fatal neurodegenerative disease that leads to muscle weakness, loss of mobility, impaired speech, and difficulty breathing and swallowing. Most ALS patients only live three to five years after initial

symptoms appear, and it is estimated that as many as 30, 000 patients in the United States and 450,000 worldwide are living with the disease. Familial ALS accounts for 5 to 10 percent of ALS cases, including an estimated 20 percent of familial ALS cases caused by toxic gain of function mutations in the SOD1 gene.

About Friedreich's Ataxia

Friedreich's ataxia (FA) is the most common hereditary ataxia, with approximately 8,000 patients living with the disease in the United States and Europe. FA patients have a genetic mutation in the FXN gene, which limits the production of the protein frataxin, causing a variety of debilitating symptoms and complications, loss of coordination and balance, muscle weakness, impaired vision, hearing and speech, scollosis, diabetes and cardiomyopathy.

About Huntington's Disease

Huntington's disease (HD) is an inherited neurodegenerative disorder where symptoms typically become noticeable between 30 and 50 years of age. HD is caused by a genetic mutation in the huntingtin gene, which leads to the production of a mutated huntingtin protein, resulting in symptoms such as chorea, rigidity, abnormal posturing, cognitive impairment and psychiatric symptoms, and difficulty with speech and swallowing. It is estimated that 1 in every 10,000 Americans has HD and more than 250,000 others are at-risk of having inherited the HD genetic mutation.

About REGENX Biosciences

ReGenX Biosciences is the leading next-generation AAV gene therapy company, developing a new class of personalized therapies based on its proprietary NAV® vector technology platform for a range of severe diseases with serious unmet needs. NAV vector technology includes novel AAV vectors rAAV7, rAAV8, rAAV9 and rAAVrh10. The company's treatments in development include programs addressing lysosomal storage disorders and ocular diseases. ReGenX's leadership in AAV gene therapy and corresponding intellectual property has enabled it to establish collaborations with leading global partners including Baxter Healthcare, Fondazione Telethon, Audentes Therapeutics, Lysogene, Esteve, AveXis and AAVLife. In addition, together with Fidelity Biosciences, ReGenX formed Dimension Therapeutics, a company focused on the development and commercialization of AAV gene therapies for rare diseases.

For more information regarding ReGenX, please visit www.regenxbio.com.

About Voyager Therapeutics

Voyager Therapeutics is a gene therapy company developing life-changing treatments for fatal and debilitating diseases of the central nervous system (CNS). Voyager is committed to advancing the field of AAV (adeno-associated virus) gene therapy through innovation and investment in vector optimization and engineering, dosing techniques, as well as process development and production. The company's initial pipeline is focused on CNS diseases in dire need of effective new therapies,

including Parkinson's disease, a monogenic form of amyotrophic lateral sclerosis (ALS), and Friedreich's ataxia. Founded by scientific and clinical leaders in the fields of AAV gene therapy, expressed RNA interference and neuroscience, Voyager Therapeutics was launched in 2014 with funding from leading life sciences investor Third Rock Ventures and is headquartered in Cambridge, Mass. For more information, please visit www.voyagertherapeutics.com.

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Contact:
REGENX Biosciences
Vit Vasista, 202-785-7438
vvasista@regenxbio.com

REGENX Biosciences (Media)
Annie Starr, 973-415-8838
astarr@6degreespr.com

Voyager Therapeutics (Media)
Katie Wilson Engleman
Pure Communications, Inc.
910-509-3977

CONFIDENTIAL TREATMENT REQUESTED

UNIVERSITY OF MINNESOTA

EXCLUSIVE PATENT LICENSE AGREEMENT

THIS EXCLUSIVE PATENT LICENSE AGREEMENT (this "EPLA") is made by and between Regents of the University of Minnesota, a constitutional corporation under the laws of the state of Minnesota, having a place of business at 200 Oak Street, SE, Suite 280, Minneapolis, Minnesota 55455 (the "University"), and the Licensee identified below. The University and the Licensee agree that:

The Terms and Conditions of Exclusive Patent License attached hereto as Exhibit A (the "Terms and Conditions") are incorporated herein by reference in their entirety. In the event of a conflict between provisions of this EPLA and the Terms and Conditions, the provisions in this EPLA shall govern. Capitalized terms used in this EPLA without definition shall have the meanings given to them in the Terms and Conditions. The section numbers used in the parentheses below correspond to the section numbers in the Terms and Conditions.

1. **Licensee (§1.10):** REGENXBIO Inc., a corporation under the laws of the state of Delaware, having a place of business at 1701 Pennsylvania Avenue, NW, Suite 900, Washington, DC 20006.

2. **Field(s) of Use (§1.5):** All fields of use for a period of Five Years from the Effective Date. Beginning on the fifth anniversary of the Effective Date, the Field of Use will be limited to: (i) all fields of use using the Licensee's proprietary adeno-associated virus vectors, and/or (ii) any indications in which the Licensee has done **** (and can document **** per indication). The Licensee shall provide the University with written notice of its proposed fields of use under clause (ii) within **** prior to the **** anniversary of the Effective Date for the University's review and confirmation that the proposed fields are consistent with the field of use described in such clause (ii).

3. **Territory (§1.17):** Any country or territory in which a Licensed Patent has been issued and is unexpired or a Licensed Patent Application is pending.

4. **Effective Date (§2):** Date of the last signature of this Agreement.

5. **Licensed Technology:**

5.1 **Licensed Patent Applications (§1.7):**

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

CONFIDENTIAL TREATMENT REQUESTED

Application No.	Country	Filing Date	Title
****	****	****	****
****	****	****	****

6. **Patent-Related Expenses (§§1.12 & 6.3):** [Select one of the following]

☐ The Licensee has no obligation under this Agreement to reimburse the University for Patent-Related Expenses.

☒ The Licensee shall reimburse the University for Patent-Related Expenses incurred before and during the Term as provided in section 6.3 of the attached Terms and Conditions; provided that, with respect to Patent-Related Expenses incurred before the Term, the Licensee is only responsible for Patent-Related Expenses in the amount of **** (which reflects the Patent-Related Expenses incurred after November 1, 2013).

☐ The Licensee shall reimburse the University for Patent-Related Expenses incurred during the Term as provided in section 6.3 of the Terms and Conditions. The Licensee shall have no obligation to reimburse the University for Patent-Related Expenses incurred before the Effective Date.

☐ The Licensee shall reimburse the University for Patent-Related Expenses incurred before the Effective Date, payable as follows: . The Licensee shall have no obligation to reimburse the University for Patent-Related Expenses during the Term.

7. **Sublicense Rights (§3.1.2):** [Select one of the following]

☒ Yes

☐ No

8. **Federal Government Rights (§3.2):** [Select one of the following]

☒ Yes

☐ No

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

9. **Performance Milestones ("PM") (§5.1):** The Licensee shall achieve the following milestones. The Licensee has an automatic right to extend any Performance Milestone for **** periods of **** each for a payment of **** per extension.

PM 1	Deliver written development plan to University	**** after the Effective Date
PM 2	Complete in vivo studies for a single indication to demonstrate efficacy (restoring normal enzyme levels, reducing metabolite storage, and correcting behavioral defects).	****
PM 3	Submit IND for a single indication	****
PM 4	Begin patient enrollment for a single indication	****
PM 5	First in human for a single indication	****

10. **Commercialization Reports (§5.4):** On each anniversary of the Effective Date, the Licensee shall deliver written commercialization reports to the University as provided in section 5.4 of the Terms and Conditions.

11. **Payments (§6.1).** All amounts are non-refundable, and payable as defined below or as specified in the University's invoice.

11.1 **Upfront Payment:** Twenty Five Thousand dollars ($25,000.00), payable within **** after the Effective Date.

11.2 **Annual Maintenance Fee:** Payable during the Term on each anniversary of the Effective Date. Creditable against royalties.

Years 1 – 5	****
Years 5 – 10	****
Years 10 =>	****

11.3 **Running Royalties and Annual Minimums.**

11.3.1 The Licensee shall pay the University a royalty of **** of the Net Sales Amount of the Licensee's and any sublicensee's Commercial Sales of Licensed Products, determined and payable as provided in section 6.4 of the Terms and Conditions. By way of example, if a sublicensee has Net Sales of $1,000, then the Licensee pays the University **** and not **** of what the Licensee receives from the sublicensee,

11.3.2 The annual minimum amount of Royalties owed by the Licensee under subsection 11.3,1 shall be ****.

11.4 **Sublicense Revenues.** Within **** after the last day of each calendar quarter, during the Term, the Licensee shall pay to the University Sublicense Revenues as received by the Licensee during such quarter as follows:

The Licensee shall pay the University according to the following schedule with respect to Sublicense Revenues. All amounts paid by the Licensee under this section 11.4 are creditable against future running royalty payments due the University pursuant to section 11.3 above and section 6.4 of the Terms and Conditions.

Sublicense Revenues received from the Effective Date through the first anniversary of the Effective Date	****
Sublicense Revenues received after the first anniversary of the Effective Date through the second anniversary of the Effective Date	****
Sublicense Revenues received after the second anniversary of the Effective Date through the third anniversary of the	****

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Effective Date	
Sublicense Revenues received after the third anniversary of the Effective Date	****

11.5 **Other Payments:** The Licensee shall pay to the University (a) **** upon approval of IND for the first Licensed Product by FDA; and (b) **** upon FDA approval of the first Licensed Product for AAV gene therapy that includes intrathecal delivery. Such payments will be due **** after the last day of the calendar quarter during the Term in which the event took place. Such payments are payable ****, regardless of how ****.

11.6 **Equity:** N/A

11.7 **Transfer Payment:** ****, payable as provided in section 12.5 of the Terms and Conditions.

11.8 **Administrative Handling Fee:** ****, payable as provided in subsection 8.1.1 of the Terms and Conditions.

11.9 **Interest Rate:** **** per annum.

11.10 **Other:** Anti-Stacking. The parties are currently working together on additional research which may result in the creation of additional intellectual property. If such new intellectual property arises and is licensed to the Licensee, the Licensee shall pay the University **** on each Licensed Product at the **** rate available in the license agreements between the parties.

12. **Licensee's Address for Notice (§12.13).** Notices will be sent to the Licensee at:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900
Washington, DC 20006
Attention: Chief Executive Officer
Facsimile No.: (202) 785-7439
Email: kmills@regenxbio.com

13. **Licensee's Contact Person for Patent Prosecution Consultation (§4.2.1).** The University will, as set forth in this Agreement, communicate with the contact person named below with

respect to patent prosecution and maintenance: (Upon ten (10) days prior written notice to the University, the Licensee may change the person designated below.)

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900
Washington, DC 20006
Attention: General Counsel
Facsimile No.: (202) 785-7439
Email: sberl@regenxbio.com

IN WITNESS WHEREOF, the parties hereto have caused their duly authorized representatives to execute this Agreement.

Regents of the University of Minnesota

By: /s/ Richard Huebsch
Richard Huebsch
Associate Director
Office for Technology Commercialization

Date: 11-7-14

REGENXBIO Inc.

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President and CEO

Date: 11-10-2014

EXHIBIT A
Terms and Conditions
Exclusive Patent License Agreement

These terms and conditions to the Exclusive Patent License Agreement ("Terms and Conditions") govern the grant of license by Regents of the University of Minnesota ("University") to the Licensee identified in the Exclusive Patent License Agreement (the "EPLA"). These Terms and Conditions are incorporated by reference into the EPLA. All section references in these Terms and Conditions refer to provisions in these Terms and Conditions unless explicitly stated otherwise.

1. **Definitions.** For purposes of interpreting this Agreement, the following terms have the following meanings:

1.1 "Affiliate" means an entity that controls the Licensee or the sublicensee, as the case may be, is controlled by the Licensee or sublicensee, or along with the Licensee or sublicensee, is under the common control of a Third Party. An entity shall be deemed to have control of the controlled entity if it (i) owns, directly or indirectly, fifty percent (50%) or more of the outstanding voting securities of the controlled entity, or (ii) has the right, power or authority, directly or indirectly, to direct or cause the direction of the policy decisions of the controlled entity, whether by ownership of securities, by representation on the controlled entity's governing body, by contract, or otherwise.

1.2 "Agreement" means, collectively, the EPLA and the Terms and Conditions.

1.3 "Commercial Sale" means a bona fide sale, use, lease, transfer or other disposition for value of a Licensed Product by the Licensee or a sublicensee to a Third Party that is not a sublicensee or an Affiliate of the Licensee or a sublicensee. Dispositions between or among any of the Licensee, sublicensees, and their respective Affiliates shall not be deemed a "Commercial Sale," except where such person is an end user, but "Commercial Sale" will include the subsequent final sales to Third Parties by such persons.

1.4 "FDA" means the United States Food and Drug Administration, or a successor agency in the United States with responsibilities comparable to those of the United States Food and Drug Administration.

1.5 "Field of Use" means the field(s) of use described in section 2 of the EPLA.

1.6 “Licensed Patent” means (a) the patent(s) described in section 5.1 of the EPLA and any patent(s) issued during the Term that arose out of a Licensed Patent Application, (b) any reexaminations, renewals, re-issues, and extensions (including any patent term extensions) of any of such patents, and (c) any foreign counterparts (including supplemental patent certificates) of any of the foregoing.

1.7 “Licensed Patent Application” means (a) the pending patent application(s) described in section 5.2 of the EPLA, (b) any continuations, continuations-in-part, divisionals, and substitutes, or any other patent application claiming priority, or entitled to claim priority, directly or indirectly to any, of such patent application(s), and (c) any foreign counterparts of any of the foregoing.

1.8 “Licensed Product” means any product or good in the Field of Use that is made by, made for, sold, transferred, or otherwise disposed of by the Licensee or its sublicensees during the Term and, if applicable, the Post-termination Period and that, on a country-by-country basis, but for the granting of the rights set forth in this Agreement, (i) infringes (including under the doctrine of equivalents) one or more Valid Claims in a Licensed Patent; or (ii) is covered by one or more Valid Claims in a Licensed Patent Application, or any product or good that is made by the Licensee or its sublicensees during the Term and, if applicable, the Post-termination Period using a process or method that, on a country-by-country basis but for the granting of rights set forth in this Agreement, (i) infringes (including under the doctrine of equivalents) one or more Valid Claims in a Licensed Patent; or (ii) is covered by one or more Valid Claims in a Licensed Patent Application. For purposes of this Agreement, Valid Claims in a Licensed Patent Application are to be treated as if they were allowed as proposed. “Licensed Product” also means any service that is provided by or for the Licensee or its sublicensees during the Term and, if applicable, the Post-termination Period and that, on a country-by-country basis, but for the granting of the rights set forth in this Agreement, (i) infringes (including under the doctrine of equivalents) one or more Valid Claims in a Licensed Patent; or (ii) is covered by one or more Valid Claims in a Licensed Patent Application.

1.9 “Licensed Technology” means collectively the inventions claimed in each Licensed Patent and each Licensed Patent Application.

1.10 “Licensee” means the entity identified in section 1 of the EPLA.

1.11 “Net Sales Amount” means the gross amount received by the Licensee or a sublicensee for a Commercial Sale of a Licensed Product minus ****.

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Commercial Sales of Licensed Products without charge or at cost in connection with research and development, clinical trials, compassionate use, humanitarian and charitable donations, or indigent programs or for use as samples shall be excluded from the computation of Net Sales Amounts, and no payments will be payable on such Commercial Sales.

1.12 “Patent-Related Expenses” means reasonable costs and expenses (including out-of-pocket attorneys’ fees, patent agent fees and governmental filing fees) that the University incurs in prosecuting and maintaining the Licensed Patents and Licensed Patent Applications.

1.13 Performance Milestone” means an act or event specified in section 5.1 and described in section 9 of the EPLA.

1.14 “Post-termination Period” means the **** period commencing on the date of early termination of the Term.

1.15 “Sublicense Revenues” means all cash revenue, but excluding Sublicense Royalties, received by the Licensee in consideration of its granting a Third Party a sublicense to any of its rights under this Agreement, including, without limitation, receipt of annual milestone attainment, sublicense issuance, maintenance or up-front payments, or technology access fee but excluding any portion of any revenue received from any Third Party sublicensee (a) relating to the sale or purchase of securities, (b) the receipt of real, personal or tangible property, (c) for the performance of research, development, or other services, (d) relating to the license or sublicense of any intellectual property other than the Licensed Patents, Licensed Patent Applications, or Licensed Technology, (e) for the sale of products other than the Licensed Products, (f) as reimbursement for patent or other expenses, or (g) for payments received from the Third Party sublicensee (including annual milestone attainment, sublicense issuance, maintenance or up-front payments, or technology access fees) to the extent such payments do not exceed each such payments owed by the Licensee to the University under this Agreement.

With respect to (g) above, by way of example: If a sublicensee achieves the milestone for approval of IND under Section 11.5(a) of the EPLA, the Licensee would owe University **** under Section 11.5 of the EPLA. If a sublicensee pays the Licensee **** for the achievement of such milestone, no portion of that payment would be considered Sublicense Revenues; however, if the sublicensee pays the Licensee more than **** for the achievement of such milestone, the initial $25,000 of such payment would not be considered Sublicense Revenues, but any amounts over such **** would be considered Sublicense Revenues.

1.16 Sublicense Royalties" means a royalty paid to the Licensee that is earned on Commercial Sales of Licensed Products by sublicensees and that is determined as percentage of the Net Sales Amount of such Commercial Sale or as a per unit amount by the sublicensee.

1.17 "Territory" means the geographical area described in section 3 of the EPLA.

1.18 "Third Party" means any party other than the University, the Licensee, or their respective Affiliates.

1.19 "Transfer Payment" means the payment to be made by the Licensee to the University specified in section 12.5 and described in section 11 of the EPLA.

1.20 "Valid Claim" means (a) a claim of an issued and unexpired Licensed Patent or (b) a claim of a Licensed Patent Application that has not been pending for more than seven years from the earliest filing date to which such claim or the applicable patent application is entitled to claim priority, in each case under clauses (a) and (b) that has not expired, lapsed, been abandoned or cancelled, been held revoked, or been deemed unenforceable or invalid by a non-appealable decision or an appealable decision from which no appeal was taken within the time allowed for such appeal of a court or other governmental agency of competent jurisdiction.

2. **Term.** The term of this Agreement commences on the Effective Date as defined in section 4 of the EPLA and, unless terminated earlier as provided in section 8, expires on the date on which both no Licensed Patent is active in the Territory and no Licensed Patent Application is pending in the Territory (the "Term"). Upon expiration of this Agreement, the Licensee's license under section 3.1 will become a royalty-free, fully-paid up, perpetual, and irrevocable license.

3. **Grant of License.**

3.1 **The Licensee's Rights.**

3.1.1 Subject to the terms and conditions of this Agreement, the University hereby grants to the Licensee, and the Licensee hereby accepts, an exclusive license, under the Licensed Patents, Licensed Patent Applications, and Licensed Technology, to make (including to have made on its behalf), use, offer to sell or sell (including to have sold on its behalf), offer to lease or lease (including to have leased on its behalf), import, or otherwise offer to dispose or dispose of Licensed Products in the Field of Use in the Territory, including, for the avoidance of doubt, the right to conduct research and development. No provision of this Agreement is to be construed to grant the Licensee, by implication, estoppel or otherwise, any rights (other than the rights expressly granted it in this Agreement) to the Licensed Technology, a Licensed Patent or Licensed Patent

Application, or to any other University-owned technology, patent applications, or patents.

3.1.2 The Licensee shall not sublicense its rights under this Agreement, unless otherwise provided in section 7 of the EPLA. If so provided, the Licensee may sublicense it rights under this Agreement, in whole or in part, through multiple tiers and to Third Parties and Affiliates; provided that, with respect to any sublicense to a Third Party, the Licensee shall deliver to the University a true and correct copy of the sublicense agreement or other agreement under which the Licensee purports or intends to grant such sublicense rights within **** the execution of such agreement, which copy may be redacted to exclude confidential information of the Licensee or the applicable sublicensee, but such copy shall not be redacted to the extent that it impairs the University's ability to ensure compliance with this Agreement. The Licensee shall not enter into such agreement if the terms of the agreement are inconsistent in any respect with the terms of this Agreement, including without limitation, sections 5.2 - 5.6, 6.5, 8.3, 9.6, 10.3, and 11.2. Any sublicense made in violation of this subsection is void.

3.2 **The United States Government's Rights**. If the University indicated in section 8 of the EPLA that the United States federal government funded the development, in whole or in part, of the Licensed Technology, then, (i) the federal government may have certain rights in and to the Licensed Technology as those rights are described in Chapter 18, Title 35 of the United States Code and accompanying regulations, including Part 401, Chapter 37 of the Code of Federal Regulations; and (ii) the parties' rights and obligations with respect to the Licensed Technology, including the grant of license set forth in subsection 3.1.1, are subject to the applicable terms of these laws and regulations.

3.3 **The University's Rights.** The University retains an irrevocable, world-wide, royalty-free, non-exclusive right to use the Licensed Technology for non-commercial teaching, research, and educational purposes. The University shall have the right to sublicense its rights under this section to one or more non-profit academic or research institutions for noncommercial teaching, research, and educational purposes, with no right to further sublicense. Notwithstanding the foregoing, the University shall not grant another sponsor rights to use the Licensed Technology and the University's Office for Technology Commercialization will use reasonable efforts to inform the other sponsor that the Licensed Technology has been licensed on an exclusive basis to Licensee.

4. **Applications and Patents.**

4.1 **Pre-EPLA Patent Filings.** The Licensee acknowledges that it has reviewed the pending patent application(s) described in section 5.1 of the EPLA, as of the Effective Date, and that it will not dispute the inventorship, validity, or enforceability of any of the claims made in such patent application as of the effective date. The Licensee further represents that, as of the

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Effective Date, it has not and does not manufacture, have manufactured, offer to sell, sell, offer to lease, lease, or import (a) any product or good that infringes (including under the doctrine of equivalents) a Valid Claim in any pending patent application(s) described in section 5.1 of the EPLA, or (b) any product or good that is made using a process or machine that infringes (including under the doctrine of equivalents) a Valid Claim in any such patent(s) or patent application(s).

4.2 **Patent Application Filings during the Term.**

4.2.1 The University, in consultation with the Licensee, shall determine in which countries patent application(s) will be filed and prosecuted with respect to the Licensed Technology. The University shall retain counsel of its choice (and reasonably acceptable to the Licensee) to file and prosecute such patent applications. The University shall inform the Licensee of the status of the prosecution of the patent application, including delivering to the Licensee pertinent notices, written and oral communications with governmental officials, and documents (including drafts of documents to be provided to governmental officials), and shall consult with the Licensee on the prosecution of the patent application and consider the Licensee's comments in good faith. The Licensee shall reasonably cooperate with the University in the filing and prosecution of all patent applications with respect to the Licensed Technology. In furtherance of the foregoing, the Licensee shall notify the University, in writing, of the individual whom the Licensee has designated to consult and cooperate as provided in this subsection and is identified in section 13 of the EPLA. The Contact Person shall respond to the University's request for consultation and cooperation on a pending matter within **** or sooner as may be required under the circumstances. If the Contact Person fails to respond in such time period, the University, exercising its own judgment and discretion, may respond to the matter as it deems appropriate. Except as provided in subsection 4.2.2, the Licensee shall reimburse the University for all Patent-Related Expenses as provided in section 6.3 and in section 6 of the EPLA. The grant of license in section 3.1 and the definition of "Licensed Patent" or "Licensed Patent Application" in section 1.6 or 1.7, respectively, shall not extend to or include any patent or patent application, on a country-by-country basis, with respect to which the Licensee elects, in writing to the University, not to pay or reimburse the payment of the cost, in whole or in part, to seek or maintain such patent or patent application.

4.2.2 No provision of this Agreement limits, conditions, or otherwise affects the University's right to prosecute a patent application with respect to the Licensed Technology in any country. The University retains the sole and exclusive right to file or otherwise prosecute a patent application with respect to the Licensed Technology. The

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Licensee shall cooperate with the University in the filing and prosecution of all patent applications with respect to the Licensed Technology.

4.3 **Rights in the Licensed Patents and Licensed Patent Applications.** No provision of this Agreement grants the Licensee any rights, titles, or interests (except for the grant of license in subsection 3.1.1) in the Licensed Patents or Licensed Patent Applications, notwithstanding the Licensee's payment of all or any portion of the patent prosecution, maintenance, and related costs.

5. **Commercialization.**

5.1 **Commercialization and Performance Milestones.** The Licensee (itself or through its Affiliates and sublicensees) shall use its commercially reasonable efforts, consistent with sound and reasonable business practices and judgment, to commercialize the Licensed Technology and to manufacture and offer to sell and sell Licensed Products as soon as reasonably practicable and to maximize sales thereof. The Licensee (itself or through its Affiliates and sublicensees) shall perform, or shall cause to happen or be performed, as the case may be, all the performance milestones described in section 9 of the EPLA.

5.2 **Covenants Regarding the Manufacture of Licensed Products.** The Licensee acknowledges that it is responsible for ensuring that the manufacture, use, sale, or transfer of Licensed Products complies with all applicable federal and state laws, including all federal export laws and regulations. The Licensee hereby further covenants and agrees that, pursuant to 35 United States Code Section 204, it shall, and it shall cause each sublicensee, to substantially manufacture in the United States of America all products to be used or sold in the United States that embody or are produced through the use of an invention that is subject to the rights of the federal government of the United States of America. Upon the Licensee's request, the University will provide reasonable assistance (not to exceed two hours of administrative time) in obtaining a waiver from the United States government with respect to such manufacturing requirement. If additional administrative time is needed, the parties will negotiate a reasonable rate for the University assistance, which would not be contingent on the outcome with respect to obtaining the waiver.

5.3 **Export and Regulatory Compliance.** The Licensee understands that the Arms Export Control Act (AECA), including its implementing International Traffic In Arms Regulations (ITAR,) and the Export Administration Act (EAA), including its Export Administration Regulations (EAR), are some (but not all) of the laws and regulations that comprise the U.S. export laws and regulations. The Licensee further understands that the U.S. export laws and regulations include (but are not limited to): (1) ITAR and EAR product/service/data-specific requirements; (ii) ITAR and EAR ultimate destination-specific requirements; (iii) ITAR and EAR end user-specific requirements; (iv) Foreign Corrupt Practices Act; and (v) antiboycott laws and regulations. The Licensee shall comply with all then-current applicable export laws and regulations of the U.S.

Government (and other applicable U.S. laws and regulations) pertaining to the Licensed Products (including any associated products, items, articles, computer software, media, services, technical data, and other information). The Licensee certifies that it shall not, directly or indirectly, export (including any deemed export), nor re-export (including any deemed re-export) the Licensed Products (including any associated products, items, articles, computer software, media, services, technical data, and other information) in violation of U.S. export laws and regulations or other applicable U.S. laws and regulations. The Licensee shall include an appropriate provision in its agreements with its authorized sublicensees to assure that these parties comply with all then-current applicable U.S. export laws and regulations and other applicable U.S. laws and regulations.

5.4 **Commercialization Reports.** Throughout the Term, and within **** of the date specified in the schedule set forth in section 10 of the EPLA, the Licensee shall deliver to the University written reports of the Licensee's and the sublicensees' efforts and plans to commercialize the Licensed Technology and to manufacture, offer to sell, or sell Licensed Products.

5.5 **Use of the University's Name and Trademarks or the Names of University Faculty, Staff, or Students.** No provision of this Agreement grants the Licensee or sublicensee any right or license to use the name, logo, or any marks owned by or associated with the University or the names, or identities of any member of the faculty, staff, or student body of the University. The Licensee shall not use and shall not permit a sublicensee to use any such logos, marks, names, or identities without the University's and, as the case may be, such member's prior written approval. Notwithstanding the foregoing, the Licensee may acknowledge the existence and general nature of this Agreement and the Licensee's status as a licensee under the Licensed Patents, Licensed Patent Applications, and Licensed Technology.

5.6 **Governmental Markings.**

5.6.1 The Licensee shall mark all Licensed Products, where feasible, with patent notice appropriate under Title 35, United States Code.

5.6.2 The Licensee is responsible for obtaining all necessary governmental approvals for the development, production, distribution, sale, and use of any Licensed Product, at the Licensee's expense, including, without limitation, any safety studies. The Licensee is responsible for including with the Licensed Product any warning labels, packaging and instructions as to the use and the quality control for any Licensed Product.

5.6.3 Upon the University's reasonable request and the agreement of the parties, the Licensee agrees to register this Agreement with any foreign governmental agency that requires such registration, and the Licensee shall pay all costs and legal fees

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in connection with such registration. The Licensee shall be responsible for complying with all foreign laws affecting this Agreement or the sale of Licensed Products.

6. **Payments, Reimbursements, Reports, and Records.**

6.1 **Payments.** The Licensee shall pay all amounts due under this Agreement by check (payable to the "Regents of the University of Minnesota" and sent to the address specified in section 12.13), wire transfer, or any other mutually agreed-upon method of payment.

6.2 **Interest.** All amounts due under this Agreement shall bear interest as provided in section 11 of the EPLA on the entire unpaid balance computed from the due date until the amount is paid.

6.3 **Reimbursement of Patent-Related Expenses.** The Licensee shall pay invoices for Patent-Related Expenses under this Agreement within **** of its receipt of the University's invoice. With respect to each invoice, the University shall use reasonable efforts to specify the date on which the Patent-Related Expense was incurred and the purpose of the expense (including, as applicable, a summary of patent attorney services giving rise to the expense); provided, however, the University is not required to disclose to the Licensee any information that is protected by the University's attorney-client privilege. Patent-Related Expenses incurred as of the Effective Date are set forth in section 6 of the EPLA.

6.4 **Royalty Payments/Sales Reports.** Within **** after the last day of the second and fourth calendar quarters during the Term and, if applicable, within **** after the last day of the Post-termination Period, the Licensee shall deliver to the University a written sales report, in the form attached hereto as Schedule 1, recounting the number and Net Sales Amount (expressed in U. S. dollars) of all Commercial Sales of Licensed Products, whether made by the Licensee or a sublicensee, during such semi-annual period. The Licensee shall deliver such written report to the University even if the Licensee is not required hereunder to pay to the University a payment for Commercial Sales of Licensed Products during the semiannual period. The Licensee shall deliver along with such sales reports its payment for royalties owed on all Commercial Sales of Licensed Products by the Licensee and the sublicensees during such semi-annual period. Only one royalty shall be payable by the Licensee for each Commercial Sale of a Licensed Product.

6.5 **Records Retention and Audit Rights.**

6.5.1 Throughout the Term and, if applicable, the Post-termination Period and for **** thereafter, the Licensee, at its expense, shall keep and maintain and shall cause each sublicensee and each non-affiliated Third Party that manufactures, sells, leases, or otherwise disposes of Licensed Products on behalf of the Licensee to

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keep and maintain complete and accurate records of all sales, leases, and other dispositions of Licensed Products during the Term and, if applicable, the Post-termination Period and all other records related to this Agreement.

6.5.2 In connection with an audit described in section 6.5.3, the Licensee, upon written request, shall deliver to the University and its representatives true, correct and complete copies of all documents and materials (including electronic records) reasonably relevant to the Licensee's and sublicensees' performance of this Agreement, including, without limitation, all sublicenses granted.

6.5.3 To determine the Licensee's compliance with the terms of this Agreement, the University, at its expense (except as set forth in this subsection), may inspect and audit the Licensee's records referred to in subsection 6.5.1 at the Licensee's address as set forth in this Agreement or such other location(s) as the parties mutually agree during the Licensee's normal business hours and with reasonable advance notice. The Licensee shall cooperate in the audit, including providing at no cost, commodious space in the Licensee's place of business for the auditor. The University may perform an audit no more frequently than once each calendar year and any period may not be audited more than once. The Licensee shall reimburse the University for all its out-of-pocket expenses to inspect and audit such records if the University, in accordance with the results of such inspection and audit, determines that the Licensee has underpaid amounts owed to the University by at least **** or ****, whichever is smaller, in a reporting period. The Licensee shall cause each sublicensee and each non-affiliated Third Party that manufactures, sells, leases, or otherwise disposes of Licensed Products on behalf of the Licensee to grant the University a right to inspect and audit the sublicensee's or Third Party's records substantially similar to the rights granted the University in this subsection. In connection with, and before the commencement of, an audit, if the Licensee requests in writing to the University, then prior to conducting such audit, the Licensee (or sublicensee, if applicable), the University and the auditor must enter into an agreement prohibiting the auditor and the University from disclosing the Licensee's (or sublicensee's) nonpublic, proprietary information to any Third Party without the Licensee's (or sublicense's) prior written consent or from using such information other than for purposes of determining the Licensee's compliance with the terms of this Agreement; provided, however, that consistent with generally accepted auditing standards and the auditor's professional judgment, the auditor may disclose such information to the University and its agents, counsel, or consultants. The Licensee acknowledges that such an agreement is adequate to protect its legitimate interests, and the parties agree that there shall be no additional nondisclosure agreement demanded as a condition to the commencement of an audit and the University's exercising its rights under this subsection.

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6.6 **Currency and Checks.** All computations and payments made under this Agreement shall be in United States dollars. To determine the dollar value of transactions conducted in non-United States dollar currencies, the parties shall use the exchange rate for the currency into dollars as reported in the Wall Street Journal as the New York foreign exchange mid-range rate on the last business day of the month in which the transaction occurred.

6.7 **Withholding.** If any payment made by the Licensee hereunder is subject to withholding taxes under the laws of any jurisdiction, the Licensee will be entitled to deduct and withhold the amount of such taxes for the account of the University to the extent required by law and, in such event, will pay the amounts of such taxes to the proper governmental authority in a timely manner and promptly transmit to the University appropriate proof of payment of such withholding taxes. At the request of the Licensee, the University will give the Licensee such reasonable assistance, which will include the provision of documentation as may be required by the relevant tax authority, to enable the Licensee to pay and report and, as applicable, claim exemption from or reduction of, such withholding tax. Any taxes withheld or remitted pursuant to this section will be treated as paid by the Licensee to the University.

7. **Infringement.**

7.1 If a party learns of substantial, credible evidence that a Third Party is making, using, or selling a product in the Field of Use in the Territory that infringes a Licensed Patent or would infringe a Licensed Patent Application if such application were to issue, such party shall promptly notify the other party in writing of the possible infringement and in such notice describe in detail the information suggesting infringement of the Licensed Patent or Licensed Patent Application. The Licensee, under its own control and at its own expense, shall have the first right but not the obligation to prosecute any third party infringement of the Licensed Patents or Licensed Patent Applications (an “Infringement Action”) with respect to any infringement related to the Field of Use, to the extent permitted by law. With respect to any infringement not related to the Field of Use, the University shall have the first right to bring an Infringement Action. Prior to commencing any action to enforce a Licensed Patent or Licensed Patent Application, the parties shall enter into good faith negotiations on the desirability of bringing suit, the parties to the action, the selection of counsel, and such other matters as the parties may agree to discuss; provided that the party bringing the Infringement Action shall have ultimate discretion over such matters. If a party is unsuccessful in persuading the alleged infringer to desist or fails to have initiated an Infringement Action within a reasonable time after such party first becomes aware of the basis for such action, the other party shall have the right, at its sole discretion, to prosecute such infringement under its sole control and at its sole expense, on notice to the other party. In any Infringement Action, the parties agree to cooperate reasonably (without a duty to join suit) with each other, at the enforcing party’s request and expense, including by using reasonable efforts to permit access to relevant personnel, records, papers, information, samples and specimens during regular business hours.

Notwithstanding the foregoing, if a court determines that the Licensee cannot prosecute an infringement Action without including the University as a party, and the court does not involuntarily join the University, then the University shall have no obligation to join such action but may do so if it determines, in its sole discretion, that joining the action would not be adverse to the best interests of the University; provided that, if the University does not join such action, the parties will negotiate in good faith an adjustment to the payment terms of this License, which may include, depending on the economic impact of the alleged infringement on Licensee's commercialization of the Licensed Technology, a suspension of Licensee's obligations to make any payments under sections 6.1 and 6.4 (including any payments described in sections 11.1, 11.2, 11.3, 11.4, 11.5 and 11.7 of the EPLA) for so long as the infringement continues. In any such Infringement Action, the enforcing party shall keep the non-enforcing party reasonably informed of the status and progress of the action, including, among other things, delivering to the non-enforcing party no less than once a quarter a written report of the status of the action. The non-enforcing party shall have the right to be represented in any such action by counsel of its own choice and at its own expense. Without the non-enforcing party's prior written consent, the enforcing party may not settle or compromise any such action in a manner that imposes any obligations or restrictions on the other party or grants any rights to the Licensed Patents or Licensed Patent Applications other than rights that the enforcing Party has the right to grant under this Agreement. Any amounts recovered (less amounts actually paid for costs and expenses associated with the litigation, including reasonable attorney's fees and legal expenses) by the Licensee in any such action or settlement that constitute compensation for lost profits or sales will be ****. All other amounts recovered (less amounts actually paid for costs and expenses associated with the litigation, including reasonable attorney's fees and legal expenses) by the Licensee in such action or settlement ****.

7.2 If any suit, action or proceeding is brought or commenced against the Licensee alleging the infringement of a patent or other intellectual property right owned by a Third Party by reason of the manufacture, use or sale of Licensed Products, the Licensee shall give the University prompt notice thereof. If the validity of a Licensed Patent is questioned in such suit, action or proceeding, the Licensee shall have no right to make any settlement or compromise which affects the scope, validity, enforceability or otherwise the Licensed Patent without the University's prior written approval.

8. **Termination.**

8.1 **By the University.**

8.1.1 If the Licensee materially breaches or materially fails to perform one or more of its obligations under this Agreement, the University may deliver a written notice of default to the Licensee. Without further action by a party, this Agreement shall

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terminate if (a) the University has not been paid the full amount of the greater of (i) the Administrative Handling Fee set forth in section 11 of the EPLA and (ii) interest in the amount set forth in section 11.9 of the EPLA, except neither such payment will be due if the default is not a monetary default (other than a breach of the obligation to meet the performance milestones pursuant to the last sentence of Section 5.1), and (b) the default has not been cured in full within either **** after the delivery to the Licensee of the notice of default if the default relates to a payment or reimbursement obligation under this Agreement, or **** after the delivery to the Licensee of the notice of default if the default relates to any other matter. If any default by the Licensee is as a result of an act of any sublicensee, the Licensee may cure such default by terminating such sublicensee's sublicense agreement.

8.1.2 The University may terminate this Agreement by delivering to the Licensee a written notice of termination at least **** before the date of termination if the Licensee (i) becomes insolvent; (ii) voluntarily files or has filed against it a petition under applicable bankruptcy or insolvency laws that the Licensee fails to have released within **** after filing; (iii) proposes any dissolution, composition, or financial reorganization with creditors or if a receiver, trustee, custodian, or similar agent is appointed; or (iv) makes a general assignment for the benefit of creditors.

8.1.3 The University may terminate this Agreement immediately by delivering to the Licensee a written notice of termination if the Licensee or its agents or representatives commences or maintains an action in any court of competent jurisdiction or a proceeding before any governmental agency asserting or alleging, in any respect, the invalidity or unenforceability of any of the Licensed Patent or Licensed Patent Application. The Licensee shall notify the University, in writing, at least **** prior to the commencement of any such action or the institution of any such proceeding.

8.2 **By the Licensee.**

8.2.1 If the University materially breaches or materially fails to perform one or more of its duties under this Agreement, the Licensee may deliver to the University a written notice of default. The Licensee may terminate this Agreement by delivering to the University a written notice of termination if the default has not cured in full within **** of the delivery to the University of the notice of default.

8.2.2 The Licensee may, upon **** prior written notice to the University, terminate this Agreement for any reason, with or without cause; provided that (i) the Licensee is current in all payment obligations due as of the notice and termination date; and (ii) the Licensee pays the University ****.

8.3 **Post-termination Period.** Except as provided in this section 8.3, upon early termination of this Agreement, the Licensee's license under the Licensed Technology terminates. If the Licensee terminates this Agreement under section 8.2 or the University terminates this Agreement under section 8.1.2 or 8.1.3, the Licensee may continue to use, offer to sell and sell, offer to lease and lease, import, and otherwise offer to dispose of or dispose of Licensed Products in the Territory that were manufactured before such termination. The Commercial Sales of Licensed Products during the Post-termination Period shall be governed by the terms of this Agreement, including the obligation to pay royalties on such Commercial Sales as provided in this Agreement.

8.4 **Survival of Sublicenses.** Except as otherwise provided in the sublicense agreement, if this Agreement terminates early for any reason, any Third Party sublicensee will, from the effective date of such termination, automatically become a direct licensee of the University with respect to the rights originally sublicensed to the Third Party sublicensee by the Licensee; provided that (a) such sublicensee is not in breach of its sublicense agreement and continues to perform thereunder, (b) such sublicensee agrees in writing to pay to the University the amounts that would have become due under this Agreement in respect of such sublicense if this Agreement had not been terminated, and (c) such sublicensee agrees in writing to the terms and conditions of this Agreement related to the rights sublicensed to such sublicensee. Notwithstanding the foregoing, the University will not be liable to such sublicensee with respect to any obligations of the Licensee to the sublicensee that are inconsistent with the University's obligations under this Agreement.

8.5 **Bankruptcy.** All rights and licenses granted under or pursuant to this Agreement by the University to the Licensee are, and shall otherwise be deemed to be, for purposes of Section 365(n) of the United States Bankruptcy Code, as amended (the "Code"), licenses of rights to "'intellectual property' as defined under Section 101(35A) of the Code. The Licensee, as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the Code. In the event of the commencement of a bankruptcy proceeding by or against the University under the Code, the Licensee shall be entitled to retain all of its rights under this Agreement.

9. **Release, Indemnification, and Insurance.**

9.1 **The Licensee's Release.** The Licensee hereby releases the University and its regents, employees, and agents forever from any and all suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses) relating to or arising out of the manufacture, use, lease, sale, or other disposition of a Licensed Product by the Licensee or any of its sublicensees (or any person that would be a sublicensee but for the sublicense being void pursuant to Section 3.1.2) as permitted by this Agreement; provided that the foregoing release is limited to any actions directly related to this

Agreement and does not apply to any suits, actions, claims, liabilities, demands, damages, losses, or expenses that may arise in connection with any other agreement between the University and the Licensee; furthermore, the foregoing release is not intended and shall not limit enforcement of the University's obligations under section 9.3 or enforcement of any claims related to the breach by the University of section 3 or its warranties under section 10.1.

9.2 **The Licensee's Indemnification.** Throughout the Term and thereafter, the Licensee shall indemnify, defend, and hold the University and its regents, employees, and agents harmless from all Third Party suits, actions, claims, liabilities, demands, damages, losses, or expenses (including reasonable attorneys' and investigative expenses) (collectively, "Third Party Liabilities"), relating to or arising out of the Licensee's ****.

9.3 **The University's indemnification.** Subject to the limitation on liability set forth in section 11, throughout the Term and thereafter, the University shall indemnify, defend, and hold the Licensee and its directors, employees, and agents harmless from all Third Party Liabilities relating to or arising out of the ****.

9.4 **Indemnification Procedure.** The party claiming indemnity under section 9.2 or 9.3 (the "Indemnified Party") will give written notice to the party from whom indemnity is being sought (the "Indemnifying Party") promptly after learning of the claim for which indemnity is being sought; provided that a failure to provide such notice promptly shall not relieve the Indemnifying Party of any liability to the Indemnified Party except to the extent the Indemnifying Party is actually prejudiced thereby. The Indemnifying Party shall be permitted to control any litigation or potential litigation involving any defense of any claim subject to indemnification pursuant to this section 9, including the selection of counsel, with the reasonable approval of the Indemnified Party; provided, however, that the Indemnified Party may participate in and monitor such defense with counsel of its own choosing at its sole expense, which shall not be subject to indemnification. The Indemnified Party shall provide the Indemnifying Party with reasonable assistance, at the Indemnifying Party's expense, in connection with the defense of any claim for which indemnity is being sought. If an Indemnifying Party fails or declines to assume the defense of any such claim within **** after notice thereof, the Indemnified Party may assume the defense of such claim at the cost and risk of the Indemnifying Party, and any Third Party Liabilities related thereto shall be deemed a Third Party Liability of the Indemnifying Party. The indemnification rights of a Indemnified Party contained in this Agreement are in addition to all other rights that such Indemnified Party may have at law or in equity or otherwise. The Indemnified Party shall not be permitted to settle or compromise any claim giving rise to Third Party Liabilities in a manner

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that imposes any restrictions or obligations on any Indemnified Party without the Indemnified Party's prior written consent.

9.5 **The Licensee's Insurance.**

9.5.1 Throughout the Term, or during such other period as the parties agree in writing, the Licensee shall maintain, and shall cause each sublicensee to maintain, in full force and effect comprehensive general liability ("CGL") insurance, with single claim limits in amounts that are reasonable and customary in the U.S. pharmaceutical industry. Such insurance policy shall include coverage for claims that are subject to indemnification of the University by the Licensee under section 9.2 and for claims by a Third Party against the Licensee or the University arising out of the purchase or use of a Licensed Product. Upon receipt of the University's written request, the Licensee shall deliver to the University a copy of the certificate of insurance for such policy.

9.5.2 The provisions of subsection 9.5.1 do not apply if the University agrees in writing to accept the Licensee's or a sublicensee's, as the case may be, self-insurance plan as adequate insurance.

9.6 **Sublicensees - Indemnification.** The Licensee shall cause each sublicensee to grant the University a release under terms substantially similar to the release by the Licensee in section 9.1 and to indemnify the University under terms substantially similar to the indemnification by the Licensee in section 9.2.

10. **Warranties.**

10.1 **Authority.** Each party represents and warrants to the other party, as of the Effective Date, that it has full corporate power and authority to execute, deliver, and perform this Agreement, and that no other corporate proceedings by such party are necessary to authorize the party's execution or delivery of this Agreement. Furthermore, the University represents and warrants to the Licensee, as of the Effective Date that (a) the University owns the Licensed Patents, Licensed Patent Applications and Licensed Technology; (b) the University inventors listed on the pending patent application(s) described in section 5.2 of the EPLA have assigned to the University their ownership interests in such patent application and the inventions claimed therein; and (c) the Office for Technology Commercialization has not entered into any agreement, or granted any rights to any person, that conflicts with the rights granted to the Licensee under this Agreement.

10.2 **Disclaimers.**

10.2.1 **EXCEPT FOR THE EXPRESS WARRANTY SET FORTH ABOVE IN SECTION 10.1, EACH OF THE UNIVERSITY AND THE LICENSEE DISCLAIMS AND EXCLUDES ALL**

WARRANTIES, EXPRESS AND IMPLIED, CONCERNING THE LICENSED TECHNOLOGY, EACH LICENSED PATENT, EACH LICENSED PATENT APPLICATION, AND EACH LICENSED PRODUCT, INCLUDING, WITHOUT LIMITATION, WARRANTIES OF NON-INFRINGEMENT, OF MERCHANTABILITY AND OF FITNESS FOR A PARTICULAR PURPOSE.

10.2.2 The University expressly disclaims any warranties concerning and makes no representations:

(i) that the Licensed Patent Applications will be allowed or granted or that a patent will issue from any Licensed Patent Application;

(ii) concerning the validity, enforceability, interpretation of claims or scope of any Licensed Patent; or

(iii) that the exercise of the rights or licenses granted to the Licensee under this Agreement will not infringe a Third Party's patent or violate its intellectual property rights.

10.3 **Sublicensees - Warranties.** The Licensee shall cause each sublicensee to acknowledge the University's disclaimers and exclusions of warranties substantially similar to the University's disclaimers and exclusions of warranties in subsections 10.2,1 and 10.2,2.

11. **Damages.**

11.1 **Remedy Limitation. EVEN IF ADVISED OF THE POSSIBILITY OF SUCH DAMAGES, NEITHER THE UNIVERSITY NOR THE LICENSEE SHALL BE LIABLE FOR LOST PROFITS, LOST BUSINESS OPPORTUNITY, INVENTORY LOSS, WORK STOPPAGE, LOST DATA OR ANY OTHER RELIANCE OR EXPECTANCY, INDIRECT, SPECIAL, INCIDENTAL OR CONSEQUENTIAL DAMAGES, OF ANY KIND; PROVIDED THAT NOTHING IN THIS SECTION 11.1 IS INTENDED TO LIMIT OR RESTRICT THE INDEMNIFICATION RIGHTS OR OBLIGATIONS OF EITHER PARTY UNDER SECTION 9 OR TO LIMIT A PARTY'S LIABILITY FOR BREACHES OF ITS OBLIGATION REGARDING CONFIDENTIALITY UNDER SECTION 12.1.2. NOTWITHSTANDING THE FOREGOING, THE TOTAL LIABILITY OF THE UNIVERSITY FOR THE BREACH OR NONPERFORMANCE OF THIS AGREEMENT SHALL NOT EXCEED THE AMOUNT OF PAYMENTS PAID TO THE UNIVERSITY UNDER SECTIONS 6.1 AND 6.4 (INCLUDING ALL PAYMENTS DESCRIBED IN SECTIONS 11.1, 11.2, 11.3, 11.4, 11.5 AND 11.7 OF THE EPLA). THIS LIMITATION APPLIES TO CONTRACT, TORT, AND ANY OTHER CLAIM OF WHATEVER NATURE.**

11.2 **Sublicensees - Damages.** The Licensee shall cause each sublicensee to agree to limitations of remedies and damages substantially similar to the limitations of remedies and damages set forth in section 11.1.

12. **General Terms**

12.1 **Access to University Information.**

12.1.1 **Data Practices Act.** The parties acknowledge that the University is subject to the terms and provisions of the Minnesota Government Data Practices Act, Minnesota Statutes §13.01 et seq. (the "Act"), and that the Act requires, with certain exceptions, the University to permit the public to inspect and copy any information that the University collects, creates, receives, maintains, or disseminates.

12.1.2 **Confidentiality.** To the extent permitted by law, including as provided in the Act, the University shall hold in confidence, disclose only to **** who need to know, and only use for purpose of this Agreement the copies of sublicense agreements provided to the University pursuant to section 3.1.2, the reports described in sections 5.4 and 6.4, the records inspected in accordance with section 6.5, the notices and information shared pursuant to sections 7.1 and 7,2, the Licensee's insurance certificates pursuant to section 9.5, and notices provided pursuant to Section 12.5. No provision of this Agreement is to be construed to further prohibit, limit, or condition the University's right to use and disclose any information in connection with enforcing this Agreement, in court or elsewhere.

12.2 **Amendment and Waiver.** The Agreement may be amended from time to time only by a written instrument signed by the parties. No term or provision of this Agreement may be waived and no breach excused unless such waiver or consent is in writing and signed by the party claimed to have waived or consented. No waiver of a breach is to be deemed a waiver of a different or subsequent breach.

12.3 **Applicable Law and Forum Selection.** The internal laws of the state of Minnesota, without giving effect to its conflict of laws principles, govern the validity, construction, and enforceability of this Agreement. A suit, claim, or other action to enforce the terms of this Agreement may be brought only in the state courts of Hennepin County, Minnesota. The Licensee hereby submits to the jurisdiction of that court and waives any objections it may have to that court asserting jurisdiction over the Licensee or its assets and property.

12.4 **Assignment and Sublicense.** Except as permitted under subsection 3.1.2 and section 12.5, the Licensee shall not assign or sublicense its interest or delegate its duties under this Agreement, without the prior written consent of the University. Any assignment, sublicense, or delegation attempted to be made in violation of this section is void. Absent the consent of all the parties, an assignment or delegation will not release the assigning or delegating party from its obligations. The Agreement inures to the benefit of the Licensee and the University and their respective permitted sublicensees and trustees.

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

12.5 **Change of Control.** Notwithstanding section 12.4, the Licensee, without the prior written consent of the University, may assign this Agreement as follows:

12.5.1 The Licensee may assign any or all of its rights and delegate any or all its duties under this Agreement to any Affiliate if the Licensee delivers to the University written notice of the assignment (along with pertinent information about the terms of the assignment and assignee) no later than **** after the effective date of such assignment; and

12.5.2 The Licensee may assign all, but no less than all, its rights and delegate all its duties under this Agreement to a Third Party if (i) the Licensee delivers to the University written notice of the proposed assignment (along with pertinent information about the terms of the assignment and assignee) no later than **** after the effective date of the event described in part iii of this paragraph, (ii) pay to the University the Transfer Payment within such **** period, and (iii) the assignment is made as a part of and in connection with (a) the sale, in one or a series of related transactions, by the Licensee of all or substantially all of its assets related to this Agreement, (b) the sale, transfer, or exchange, in one or a series of related transactions, by the shareholders, partners, or equity owners of the Licensee of a majority interest in the Licensee to a purchaser(s), or (c) the merger, consolidation, or reorganization, in one or a series of related transactions, of the Licensee into or with another corporation or other business entity.

Any assignment attempted to be made or made in violation of this subsection is void.

12.6 **Collection Costs and Attorneys' Fees.** If a party materially fails to perform an obligation or otherwise materially breaches one or more of the terms of this Agreement, the other party may recover from the non-performing breaching party all its reasonable costs (including actual attorneys' and investigative fees) to enforce the terms of this Agreement.

12.7 **Consent and Approvals.** Except as otherwise expressly provided, in order to be effective, all consents or approvals required under this Agreement must be in writing.

12.8 **Construction.** The headings preceding and labeling the sections of this Agreement are for the purpose of identification only and are not to be employed or used for the purpose of construction or interpretation of any portion of this Agreement. As used herein and where necessary, the singular includes the plural and vice versa, and masculine, feminine, and neuter expressions are interchangeable.

12.9 **Enforceability.** If a court of competent jurisdiction adjudges a provision of this Agreement to be unenforceable, invalid, or void, such determination is not to be construed as impairing the enforceability of any of the remaining provisions hereof and such provisions will

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

remain in full force and effect, unless the unenforceable, invalid or void provision is of such essential importance to this Agreement that it is to be reasonably assumed that the parties would not have entered into this Agreement without the unenforceable, invalid or void provision.

12.10 **Entire Agreement.** The parties intend this Agreement (including both the EPLA and these Terms and Conditions and al! attachments, exhibits, and amendments hereto) to be the final and binding expression of their contract and agreement and the complete and exclusive statement of the terms thereof. The Agreement cancels, supersedes, and revokes all prior negotiations, representations and agreements among the parties, whether oral or written, relating to the subject matter of this Agreement.

12.11 **Language and Currency.** Unless otherwise expressly provided in this Agreement and in order to be effective, all notices, reports, and other documents and instruments that a party elects or is required to deliver to the other party must be in English, and all notices, reports, and other documents and instruments detailing revenues under this Agreement or expenses chargeable to a party must be United States dollar denominated,

12.12 **No Third-Party Beneficiaries.** No provision of this Agreement, express or implied, is intended to confer upon any person other than the parties to this Agreement any rights, remedies, obligations, or liabilities hereunder. No sublicensee may enforce or seek damages under this Agreement.

12.13 **Notices.** In order to be effective, all notices, requests, and other communications that a party is required or elects to deliver must be in writing and must be delivered personally, or by facsimile or electronic mail (provided such delivery is confirmed), or by a recognized overnight courier service or by United States mail, first-class, certified or registered, postage prepaid, return receipt requested, to the other party at its address set forth below or to such other address as such party may designate by notice given under this section:

If to the University: University of Minnesota
Office for Technology Commercialization
200 Oak Street, SE
Suite 280
Minneapolis, MN 55455
Phone: 612.624.0550
Fax: 612.624.6554
E-mail: otcagree@umn.edu

For notices sent under section 8, with a copy to:	University of Minnesota Office of the General Counsel Attn: Transactional Law Services 360 McNamara Alumni Center 200 Oak Street S.E. Minneapolis, MN 55455-2006 Facsimile No.: 612.626.9624 E-mail: contracts@mail.ogc.umn.edu
If to the Licensee:	As indicated in section 12 of the EPLA.

Notices will be deemed to have been given as of the date received.

12.14 **Relationship of Parties.** In entering into, and performing their duties under this Agreement, the parties are acting as independent contractors and independent employers. No provision of this Agreement creates or is to be construed as creating a partnership, joint venture, or agency relationship between the parties. No party has the authority to act for or bind the other party in any respect.

12.15 **Survival.** Immediately upon the termination or expiration of this Agreement, except for certain rights granted for the Post-termination Period or certain rights that survive expiration as provided in section 2, all the Licensee's rights under this Agreement terminate; provided, however, either party's obligations that have accrued before the effective date of termination or expiration (e.g., the Licensee's obligation to report and make payments on sales, leases, or dispositions of Licensed Products and to reimburse the University for costs) survive. The obligations and rights set forth in sections 6.4, 8.3, and 8.4 and sections 9, 10, 11, and 12 also survive the termination or expiration of this Agreement.

CONFIDENTIAL TREATMENT REQUESTED

Schedule 1
Form of Sales Report

License Number : *A20150121*

Enter Reporting Period: **Report Date:**

This report must be submitted regardless of whether royalties are owed. State all information requested below - do not leave either column blank, Please reference the UM Case # on all royalty payments.

UM Case #	Product Description	Royalty Rate	Quantity/ Net Sales	Royalty Due
****		****		

Total Royalties Due: US$

Report Completed by:

REGENXBIO Inc.
1701 Pennsylvania Avenue, NW
Suite 900
Washington, DC 20006

**** CERTAIN INFORMATION HAS BEEN OMITTED AND FILED SEPARATELY WITH THE COMMISSION. CONFIDENTIAL TREATMENT HAS BEEN REQUESTED WITH RESPECT TO THE OMITTED PORTIONS.

Exhibit 10.26

LEASE

by and between

BMR-MEDICAL CENTER DRIVE LLC,
a Delaware limited liability company

and

REGENXBIO INC.,
a Delaware corporation

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LEASE

THIS LEASE (this "Lease") is entered into as of this 6th day of March, 2015 (the "Execution Date"), by and between BMR-MEDICAL CENTER DRIVE LLC, a Delaware limited liability company ("Landlord"), and REGENXBIO INC., a Delaware corporation ("Tenant").

RECITALS

A. WHEREAS, Landlord owns certain real property (the "Property") and the improvements on the Property located at 9704, 9708, 9712 and 9714 Medical Center Drive, Rockville, Maryland, including the buildings located thereon; and

B. WHEREAS, Landlord wishes to lease to Tenant, and Tenant desires to lease from Landlord, certain premises (the "Premises") located on the first (1st) floor of the building at 9712 Medical Center Drive, Rockville, Maryland (the "Building"), pursuant to the terms and conditions of this Lease, as detailed below.

AGREEMENT

NOW, THEREFORE, Landlord and Tenant, in consideration of the mutual promises contained herein and for other good and valuable consideration, the receipt and sufficiency of which are hereby acknowledged, and intending to be legally bound, agree as follows:

1. Lease of Premises.

1.1. Effective on the Term Commencement Date (as defined below), Landlord hereby leases to Tenant, and Tenant hereby leases from Landlord, the Premises, as shown on Exhibit A attached hereto, for use by Tenant in accordance with the Permitted Use (as defined below) and no other uses. The Property and all landscaping, parking facilities, private drives and other improvements and appurtenances related thereto, including the Building and the buildings located at 9704, 9708 and 9714 Medical Center Drive, Rockville, Maryland, are hereinafter collectively referred to as the "Project." The Building is located on a portion of the Project commonly referred to as the "South Campus," which is that part of the Project comprised of the Building and two (2) other buildings located at 9708 and 9714 Medical Center Drive, Rockville, Maryland, together with all appurtenances thereto (collectively, the "South Campus"). All portions of the Building that are for the non-exclusive use of the tenants of the Building only, and not the tenants of the Project generally, such as service corridors, stairways, elevators, public restrooms and public lobbies (all to the extent located in the Building), are hereinafter referred to as "Building Common Area." All portions of the Project that are for the non-exclusive use of tenants of the South Campus or the Project (as applicable) generally, including driveways, sidewalks, parking areas, landscaped areas, and service corridors, stairways, elevators, public restrooms, public lobbies and the Amenities Area (as defined below) (but excluding Building Common Area), are hereinafter referred to as "Project Common Area." The Building Common Area and Project Common Area are collectively referred to herein as "Common Area."

1.2. There currently exists an amenities area located in the amenities level of the building at 9714 Medical Center Drive, Rockville, Maryland, which consists of a fitness center, kitchenette, bathrooms, locker room, board room, and assembly area (collectively, the "Amenities Area"). The Amenities Area is currently for the use of the tenants of the South Campus only; provided, however, that Landlord reserves the right to allow the tenants of the entire Project to use the Amenities Area.

2. Basic Lease Provisions. For convenience of the parties, certain basic provisions of this Lease are set forth herein. The provisions set forth herein are subject to the remaining terms and conditions of this Lease and are to be interpreted in light of such remaining terms and conditions.

2.1. This Lease shall take effect upon the Execution Date and, except as specifically otherwise provided within this Lease, each of the provisions hereof shall be binding upon and inure to the benefit of Landlord and Tenant from the date of execution and delivery hereof by all parties hereto.

2.2. In the definitions below, each current Rentable Area (as defined below) is expressed in square feet. Rentable Area and "Tenant's Pro Rata Shares" are all subject to adjustment as provided in this Lease.

Definition or Provision	Means the Following (As of the Term Commencement Date)
Approximate Rentable Area of Premises	10,833 square feet
Approximate Rentable Area of Building	22,907 square feet
Approximate Rentable Area of South Campus	92,125 square feet
Approximate Rentable Area of Project	214,725 square feet
Tenant's Pro Rata Share of Building	47.29%
Tenant's Pro Rata Share of South Campus	11.76%
Tenant's Pro Rata Share of Project	5.05%

2.3. Monthly installments of Base Rent for the Premises ("Base Rent") as of the Term Commencement Date:

Dates	Square Feet of Rentable Area	Base Rent per Square Foot of Rentable Area	Monthly Base Rent
Months 1 – 6	10,833	$ 0.00 annually	$ 0.00

Dates	Square Feet of Rentable Area	Base Rent per Square Foot of Rentable Area	Monthly Base Rent
Months 7 – 12	10,833	$ 26.50 annually	$ 23,922.88
Months 13 – 24	10,833	$ 26.50 annually	$ 23,922.88
Months 25 – 36	10,833	$ 27.30 annually	$ 24,645.08
Months 37 – 48	10,833	$ 28.11 annually	$ 25,376.30
Months 49 – 60	10,833	$ 28.96 annually	$ 26,143.64
Months 61 – 66	10,833	$ 29.83 annually	$ 26,929.03

2.4. Estimated Term Commencement Date: April 1, 2015

2.5. Estimated Term Expiration Date: September 30, 2020

2.6. Security Deposit: $23,922.88

2.7. Permitted Use: Office use in conformity with all federal, state, municipal and local laws, codes, ordinances, rules and regulations of Governmental Authorities (as defined below), committees, associations, or other regulatory committees, agencies or governing bodies having jurisdiction over the Premises, the Building, the Property, the Project, Landlord or Tenant, including both statutory and common law and hazardous waste rules and regulations ("Applicable Laws")

2.8. Address for Rent Payment:

BMR-Medical Center Drive LLC
Attention Entity 472
P.O. Box 511415
Los Angeles, California 90051-7970

2.9. Address for Notices to Landlord:

BMR-Medical Center Drive LLC
17190 Bernardo Center Drive
San Diego, California 92128
Attn: Vice President, Real Estate Legal

2.10. Address for Notices to Tenant:

REGENXBIO Inc.
1701 Pennsylvania Avenue, Suite 900
Washington, DC 20006
Attn: General Counsel

2.11. Address for Invoices to Tenant:

REGENXBIO Inc.
1701 Pennsylvania Avenue, Suite 900
Washington, DC 20006
Attn: Chief Financial Officer

2.12. The following Exhibits are attached hereto and incorporated herein by reference:

Exhibit A	Premises
Exhibit B	Tenant Improvement Plans
Exhibit B-l	Tenant Work Insurance Schedule
Exhibit C	Acknowledgement of Term Commencement Date and Term Expiration Date
Exhibit D	Bill of Sale
Exhibit E	Form of Letter of Credit
Exhibit F	Rules and Regulations
Exhibit G	[Intentionally omitted]
Exhibit H	Tenant's Personal Property
Exhibit I	Form of Estoppel Certificate
Exhibit J	[Intentionally omitted]
Exhibit K	Janitorial Specifications

3. Term. The actual term of this Lease (as the same may be extended pursuant to Article 42 hereof, and as the same may be earlier terminated in accordance with this Lease, the "Term") shall commence on the actual Term Commencement Date (as defined in Article 4) and end on the date that is sixty-six (66) months after the actual Term Commencement Date (such date, the "Term Expiration Date"), subject to extension or earlier termination of this Lease as provided herein, provided that if the actual Term Commencement Date is not the first day of a calendar month, the Term and the Term Expiration Date shall be extended to the last day of the calendar month in which the Term Expiration Date would otherwise occur (and Base Rent for such extension period shall be payable at the rate in effect during the month immediately preceding such extension period).

4. Possession and Commencement Date.

4.1. Landlord shall use commercially reasonable efforts to tender possession of the Premises to Tenant on the Estimated Term Commencement Date, with the work (the "Tenant Improvements") required of Landlord described on Exhibit B Substantially Complete (as defined below). Tenant agrees that in the event such work is not Substantially Complete on or before the Estimated Term Commencement Date for any reason, then (a) this Lease shall not be void or voidable, (b) Landlord shall not be liable to Tenant for any loss or damage resulting therefrom, (c) the Term Expiration Date shall be extended accordingly and (d) Tenant shall not be responsible for the payment of any Base Rent or Tenant's Adjusted Share of Operating Expenses (as defined below) until the actual Term Commencement Date as described in Section 4.2 occurs. The term "Substantially Complete" or "Substantial Completion" means that (y) the

Tenant Improvements are substantially complete in accordance with the plans attached hereto as Exhibit B (the "Tenant Improvement Plans"), except for minor punch list items and (z) a certificate of occupancy has been issued for the Premises. Notwithstanding anything in this Lease to the contrary, Landlord's obligation to timely achieve Substantial Completion shall be subject to extension on a day-for-day basis as a result of Force Majeure (as defined below).

4.2. The "Term Commencement Date" shall be the day Landlord tenders possession of the Premises to Tenant with the Tenant Improvements Substantially Complete. If possession is delayed by action of Tenant, then the Term Commencement Date shall be the date that the Term Commencement Date would have occurred but for such delay. Tenant shall execute and deliver to Landlord written acknowledgment of the actual Term Commencement Date and the Term Expiration Date within ten (10) business days after Tenant takes occupancy of the Premises, in the form attached as Exhibit C hereto. Failure to execute and deliver such acknowledgment, however, shall not affect the Term Commencement Date or Landlord's or Tenant's liability hereunder.

4.3. Landlord shall permit Tenant and its contractors to enter upon the Premises, commencing upon the date that Landlord reasonably estimates to be thirty (30) days prior to the Term Commencement Date, for the purpose of installing therein improvements, cabling, equipment, furniture and personal property, provided that Tenant shall furnish to Landlord evidence satisfactory to Landlord in advance that insurance coverages required of Tenant and its contractors under the provisions of Article 23 are in effect and such entry shall be subject to all the terms and conditions of this Lease other than the payment of Base Rent or Tenant's Adjusted Share of Operating Expenses (as defined below); and provided, further, that if the Term Commencement Date is delayed due to such early access, then the Term Commencement Date shall be the date that the Term Commencement Date would have occurred but for such delay.

4.4. Landlord shall cause the Tenant Improvements to be constructed in the Premises at Landlord's sole cost and expense. All costs incurred by Landlord in connection with the Tenant Improvements including, without limitation, costs of (a) construction, (b) commissioning of mechanical, electrical and plumbing systems by a licensed, qualified commissioning agent hired by Landlord, (c) space planning, architect, engineering and other related services, (d) building permits and other taxes, fees, charges and levies by Governmental Authorities (as defined below) for permits or for inspections of the Tenant Improvements, and (e) fees, costs and expenses for Landlord's architect, and engineer, labor, material, equipment and fixtures shall be referred to in this Lease as the "Tenant Improvement Costs." In the event that Tenant fails to comply with any of its obligations under this Lease and such failure causes Landlord to incur additional Tenant Improvement Costs, Tenant shall pay to Landlord as Additional Rent (as defined below) the amount of any such additional costs within thirty (30) days of receiving an invoice from Landlord. Landlord shall construct the Tenant Improvements in a good and workmanlike manner and in compliance with all Applicable Laws and the Tenant Improvement Plans.

4.5. Any changes to the Tenant Improvement Plans requested by Tenant (each, a "Tenant Change") shall be requested and instituted in accordance with the provisions of this Section 4.5 and shall be subject to the written approval of Landlord as provided herein.

4.5.1 Tenant may request Tenant Changes by notifying Landlord in writing in substantially the same form as the AIA standard change order form (a "Tenant Change Request"), which Tenant Change Request shall detail the nature and extent of any requested Tenant Changes.

4.5.2 All Tenant Change Requests shall be subject to Landlord's prior written approval, which approval shall not be unreasonably withheld, conditioned or delayed (provided, however, that, in the event any Tenant Change would, in Landlord's reasonable judgment, delay the Substantial Completion of the Tenant Improvements, Landlord may withhold its approval with respect thereto in its sole and absolute discretion). Landlord shall have five (5) days after receipt of a Tenant Change Request to notify Tenant in writing of Landlord's approval or rejection of the Tenant Change and any rejection shall state the reasons therefor in reasonable detail. Landlord's failure to respond within such five (5) day period shall be deemed approval by Landlord.

4.5.3 Notwithstanding anything to the contrary in this Lease, Tenant shall be solely responsible for all costs and expenses related to any Tenant Changes (including, without limitation, costs of project management by Landlord (which fee shall equal three percent (3%) of the cost of the Tenant Change)). Tenant shall, within thirty (30) days of receiving an invoice therefore, pay to Landlord the amount of any such costs.

4.5.4 Notwithstanding anything to the contrary in this Lease, in the event that any Tenant Change causes a delay in the Substantial Completion of the Tenant Improvements, the Term Commencement Date shall be the date that the Term Commencement Date would have occurred but for such delay.

4.5.5 The Tenant Improvement Plans shall be automatically updated to include any Tenant Changes approved by Landlord in accordance with this Section 4.5.

4.6. Landlord shall be permitted to make changes to the Tenant Improvement Plans (each, a "Landlord Change") subject to the terms, conditions and provisions of this Section 4.6. Landlord shall be solely responsible for all costs and expenses related to any Landlord Changes.

4.6.1 Landlord may request Landlord Changes by notifying Tenant in writing in substantially the same form as the AIA standard change order form (a "Landlord Change Request"), which Landlord Change Request shall detail the nature and extent of any requested Landlord Changes.

4.6.2 Subject to Subsection 4.6.3, all Landlord Change Requests other than Landlord Permitted Changes (as defined below), shall be subject to Tenant's prior written approval, which approval shall not be unreasonably withheld, conditioned or delayed. Tenant shall have five (5) days after receipt of a Landlord Change Request to notify Landlord in writing of Tenant's approval or rejection of the Landlord Change. Tenant's failure to respond within such five (5) day period shall be deemed approval by Tenant.

4.6.3 Notwithstanding anything to the contrary in this Lease, Landlord shall be permitted to make Landlord Permitted Changes (as defined below) without obtaining Tenant's consent but with prior written notice to Tenant in the event of a Landlord Permitted Change under Subsection (b) below. "Landlord Permitted Changes" shall mean (a) minor changes (such as slight relocation of switches and/or outlets) to accommodate unforeseen field conditions and (b) changes required by Applicable Laws or by a Governmental Authority.

4.6.4 The Tenant Improvement Plans shall be automatically updated to include any Landlord Permitted Changes or other Landlord Changes approved by Tenant in accordance with this Section 4.6.

4.7. Notwithstanding anything to the contrary in this Lease, if Substantial Completion has not occurred by the date that is ninety (90) days after the Estimated Term Commencement Date (such date, the "Abatement Date"'), then Tenant shall be entitled to receive one (1) day of Base Rent abatement for each day thereafter that Substantial Completion has not occurred with such Base Rent abatement commencing on the first day of the seventh (7) month of the Term; provided, however, that the Abatement Date shall be subject to extension on a day-for-day basis as a result of any delays in Substantial Completion to the extent actually caused by (a) Force Majeure, (b) Tenant Changes or (c) Tenant.

5. Condition of Premises. Tenant acknowledges that neither Landlord nor any agent of Landlord has made any representation or warranty with respect to the condition of the Premises, the Building or the Project, or with respect to the suitability of the Premises, the Building or the Project for the conduct of Tenant's business. Tenant acknowledges that, except as otherwise expressly set forth in this Lease, (a) it is fully familiar with the condition of the Premises and agrees to take the same in its condition "as is" as of the Term Commencement Date and (b) Landlord shall have no obligation to alter, repair or otherwise prepare the Premises for Tenant's occupancy or to pay for or construct any improvements to the Premises, except with respect to the Tenant Improvements. Notwithstanding the foregoing, Landlord agrees that, as of the Term Commencement Date, (x) the Premises shall be clean and free of debris and the prior tenant's personal property and shall not contain any Hazardous Materials in violation of Applicable Laws, (y) the Premises and the Tenant Improvements shall be in compliance with all Applicable Laws including, without limitation, the ADA (as defined below) (provided, however, that if Landlord fails to so deliver the Premises and the Tenant Improvements in compliance with the ADA and Applicable Laws, then Tenant's sole and exclusive remedy shall be to deliver written notice to Landlord detailing the nature of such failure and, upon receipt of any such notice, Landlord shall (at its sole cost and expense) promptly make any modifications reasonably necessary correct such failure), and (c) the heating, ventilating and air conditioning ("HVAC"); mechanical; electrical; life safety and plumbing systems serving the Premises (such systems, collectively, the "Building Systems") shall be in good working condition, operating as designed and suitable for office use (and Landlord shall, at Landlord's sole cost and expense, repair any latent defects in the Building Systems to the extent that Tenant notifies Landlord of such latent defects on or before the date that is six (6) months after the Term Commencement Date). Tenant's taking of possession of the Premises shall, except as otherwise agreed to in writing by Landlord and Tenant, conclusively establish that the Premises, the Building and the Project were at such time in good, sanitary and satisfactory condition and repair; provided, however, that

nothing in this grammatical sentence shall take away from Landlord's express obligations under this Lease, including, without limitation, Landlord's repair, maintenance, restoration and legal compliance obligations expressly set forth in this Lease.

6. Rentable Area.

6.1. The term "Rentable Area" shall reflect such areas as reasonably calculated by Landlord's architect, as the same may be reasonably adjusted from time to time by Landlord in consultation with Landlord's architect to reflect changes to the Premises, the Building or the Project, as applicable. Notwithstanding the foregoing to the contrary, in no event shall the Rentable Area of the Premises be deemed to have increased unless due to a physical change in the size of the Premises.

6.2. The Rentable Area of the Building is generally determined by making separate calculations of Rentable Area applicable to each floor within the Building and totaling the Rentable Area of all floors within the Building. The Rentable Area of a floor is computed by measuring to the outside finished surface of the permanent outer Building walls. The full area calculated as previously set forth is included as Rentable Area, without deduction for columns and projections or vertical penetrations, including stairs, elevator shafts, flues, pipe shafts, vertical ducts and the like, as well as such items' enclosing walls.

6.3. The term "Rentable Area," when applied to the Premises, is that area equal to the usable area of the Premises, plus an equitable allocation of Rentable Area within the Building that is not then utilized or expected to be utilized as usable area, including that portion of the Building devoted to corridors, equipment rooms, restrooms, elevator lobby, atrium and mailroom.

6.4. The Rentable Area of the South Campus is the total Rentable Area of all buildings within the South Campus. The Rentable Area of the Project is the total Rentable Area of all buildings within the Project.

6.5. Review of allocations of Rentable Areas as between tenants of the Building and the Project shall be made as frequently as Landlord deems appropriate (but no more often than annually), including in order to facilitate an equitable apportionment of Operating Expenses (as defined below). If such review is by a licensed architect and allocations are certified by such licensed architect as being correct, then Tenant shall be bound by such certifications.

7. Rent.

7.1. Tenant shall pay to Landlord as Base Rent for the Premises, commencing on the Term Commencement Date, the sums set forth in Section 2.3. Base Rent shall be paid in equal monthly installments as set forth in Section 2.3, each in advance on the first day of each and every calendar month during the Term.

7.2. In addition to Base Rent, Tenant shall pay to Landlord as additional rent ("Additional Rent") at times hereinafter specified in this Lease (a) Tenant's Pro Rata Share of Building, South Campus or Project (as applicable) of any increases in (i) Real Estate Taxes (as defined below) over Real Estate Taxes for the 2015 calendar year (the "Base Year") and (ii)

Operating Expenses (as defined below) over Operating Expenses for the Base Year, (b) the Property Management Fee (as defined below) and (c) any other amounts that Tenant assumes or agrees to pay under the provisions of this Lease that are owed to Landlord, including any and all other sums that may become due by reason of any default of Tenant or failure on Tenant's part to comply with the agreements, terms, covenants and conditions of this Lease to be performed by Tenant, after notice and the lapse of any applicable cure periods.

7.3. Base Rent and Additional Rent shall together be denominated "Rent." Rent shall be paid to Landlord, without abatement, deduction or offset (except as otherwise expressly provided in this Lease), in lawful money of the United States of America pursuant to the address as set forth in Section 2.8 or to such other person or at such other place as Landlord may from time designate in writing. In the event the Term commences on a day other than the first day of a calendar month or ends on a day other than the last day of a calendar month, then the Rent for such fraction of a calendar month shall be prorated for such period on the basis of the number of days in the calendar month and shall be paid at the then-current rate for such fractional calendar month.

7.4. Except as otherwise expressly provided in this Lease, Tenant's obligation to pay Rent shall not be discharged or otherwise affected by (a) any Applicable Laws now or hereafter applicable to the Premises, (b) any other restriction on Tenant's use, (c) any casualty or taking or (d) any other occurrence; and, except as otherwise expressly provided in this Lease, Tenant waives all rights now or hereafter existing to terminate or cancel this Lease or quit or surrender the Premises or any part thereof, or to assert any defense in the nature of constructive eviction to any action seeking to recover rent. Tenant's obligation to pay Rent with respect to any period or obligations arising, existing or pertaining to the period prior to the date of the expiration or earlier termination of the Term or this Lease shall survive any such expiration or earlier termination; provided, however, that nothing in this sentence shall in any way affect Tenant's obligations with respect to any other period.

8. [Intentionally omitted]

9. Operating Expenses.

9.1. As used herein, the term "Operating Expenses" shall include (subject to the exclusions set forth in Section 9.1(c)):

(a) Government impositions, including property tax costs consisting of real and personal property taxes (including amounts due under any improvement bond upon the Building or the Project (including the parcel or parcels of real property upon which the Building, the other buildings in the Project and areas serving the Building and the Project are located)) or assessments in lieu thereof imposed by any federal, state, regional, local or municipal governmental authority, agency or subdivision (each, a "Governmental Authority"); taxes on or measured by gross rentals received from the rental of space in the Project; taxes based on the square footage of the Premises, the Building or the Project, as well as any parking charges, utilities surcharges or any other costs levied, assessed or imposed by, or at the direction of, or resulting from Applicable Laws or interpretations thereof, promulgated by any Governmental Authority in connection with the use or occupancy of the Project or the parking facilities serving

the Project; taxes on any document to which Tenant is a party creating or transferring an interest in the Premises; any fee for a business license to operate an office building; and any expenses, including the reasonable cost of attorneys or experts, reasonably incurred by Landlord in seeking reduction by the taxing authority of the applicable taxes, less tax refunds obtained as a result of an application for review thereof (collectively, "Real Estate Taxes"); and

(b) All other costs of any kind paid or incurred by Landlord in connection with the operation or maintenance of the Building and the Project (including the Amenities Area), and costs of repairs and replacements to improvements within the Project as appropriate to maintain the Project as required hereunder; costs of utilities furnished to the Common Area; sewer fees; cable television; trash collection; cleaning, including windows; heating, ventilation and air-conditioning; maintenance of landscaping and grounds; snow removal; maintenance of drives and parking areas; maintenance of the roof; security services and devices; building supplies; maintenance or replacement of equipment utilized for operation and maintenance of the Project; license, permit and inspection fees; sales, use and excise taxes on goods and services purchased by Landlord in connection with the operation, maintenance or repair of the Building or Project systems and equipment; telephone, postage, stationery supplies and other expenses incurred in connection with the operation, maintenance or repair of the Project; accounting, legal and other professional fees and expenses incurred in connection with the Project; costs of furniture, draperies, carpeting, landscaping supplies, snow removal and other customary and ordinary items of personal property provided by Landlord for use in Common Area; Project office rent or rental value for a commercially reasonable amount of space, to the extent an office used for Project operations is maintained at the Project, plus customary expenses for such office; capital expenditures incurred (i) for the primary purpose of reducing Operating Expenses or (ii) required by any Governmental Authority to comply with changes in Applicable Laws that take effect after the Execution Date or to ensure continued compliance with Applicable Laws in effect as of the Execution Date, in each case amortized over the useful life thereof on a straight-line basis, as reasonably determined by Landlord, in accordance with generally accepted accounting principles, but in no event longer than ten (10) years; non-capital costs of complying with Applicable Laws (except to the extent such costs are incurred to remedy non-compliance as of the Execution Date with Applicable Laws); costs to keep the Project in compliance with, or fees otherwise required under, any CC&Rs (as defined below); insurance premiums, including premiums for commercial general liability, property casualty, earthquake, terrorism and environmental coverages subject to commercially reasonable deductibles; portions of insured losses paid by Landlord as part of the commercially reasonable deductible portion of a loss pursuant to the terms of insurance policies; service contracts; costs of services of independent contractors retained to do work of a nature referenced above; and costs of compensation (including employment taxes and fringe benefits) of all persons who perform regular and recurring duties connected with the day-to-day operation and maintenance of the Project, its equipment, the adjacent walks, landscaped areas, drives and parking areas, including janitors, floor waxers, window washers, watchmen, gardeners, sweepers, plow truck drivers, handymen, and engineering/maintenance personnel.

(c) Notwithstanding the foregoing, Operating Expenses shall not include any net income, franchise, capital stock, estate or inheritance taxes, or taxes that are the personal obligation of Tenant or of another tenant of the Project; interest and penalties incurred as a result

of Landlord's late payment of any Operating Expense; any leasing commissions, legal fees, and advertising costs incurred in connection with leasing of space in the Project; expenses that relate to preparation, renovating or otherwise demolishing, modifying, improving, decorating, fixturing, furnishing, painting or altering of rental space for a tenant; expenses of initial development and construction or expansion of the Project, including grading, paving, landscaping and decorating (as distinguished from maintenance, repair and replacement of the foregoing); legal expenses relating to disputes with tenants; costs of repairs to the extent reimbursed by payment of insurance proceeds received by Landlord or pursuant to a warranty held by Landlord; payments of principal or interest upon loans to Landlord or secured by a mortgage or deed of trust covering the Project or a portion thereof (provided that interest upon a government assessment or improvement bond payable in installments shall be included in Real Estate Taxes as provided in Subsection 9.1(a)); ground and/or underlying lease payments; salaries of executive officers of Landlord or any employee of Landlord above the level of the acting property manager of the Project; Landlord's general overhead and administrative expenses; depreciation claimed by Landlord for tax purposes (provided that this exclusion of depreciation is not intended to delete from Operating Expenses actual costs of repairs and replacements that are provided for in Subsection 9.1(b)); taxes that are excluded from Operating Expenses by the last sentence of Subsection 9.1(a); points, commissions, closing costs, legal fees, costs or expenses incurred in connection with the financing or sale of the Project or any portion thereof; costs expressly excluded from Operating Expenses elsewhere in this Lease or that are charged to or paid by Tenant under other provisions of this Lease; professional fees and disbursements and other costs and expenses related to the ownership (as opposed to the use, occupancy, operation, maintenance or repair) of the Project; charitable or political contributions and/or trade association dues; asset management fees and property management fees of any property management firm based on a rate to the extent in excess of the rate of such fees paid by Landlord with respect to the Base Year (provided, however, that nothing in this Section 9.1 shall take away from Tenant's obligation to pay the Property Management Fee as required under Section 9.2 of this Lease); moving expense costs of tenants or occupants of the Project; reserves for future Operating Expenses; and any item that, if included in Operating Expenses, would involve a double collection for such item by Landlord. To the extent that Tenant uses more than Tenant's Pro Rata Share of any item of Operating Expenses, Tenant shall pay Landlord for such excess in addition to Tenant's obligation to pay Tenant's Pro Rata Share of any increase in Operating Expenses over Operating Expenses for the Base Year (such excess, together with Tenant's Pro Rata Share of any increase in Operating Expenses over Operating Expenses for the Base Year, "Tenant's Adjusted Share").

9.2. Tenant shall pay to Landlord on the first day of each calendar month of the Term, as Additional Rent, (a) commencing on the Term Commencement Date, the Property Management Fee (as defined below), (b) commencing on January 1, 2016, Landlord's good faith estimate of Tenant's Adjusted Share of any increase of Operating Expenses over the Base Year with respect to the Building, the South Campus and the Project, as applicable, for such month and (c) commencing on January 1, 2016, Landlord's good faith estimate of Tenant's Pro Rata Share of any increase of Real Estate Taxes over the Base Year with respect to the Building, South Campus and the Project, as applicable, for such month.

(w) The "Property Management Fee" shall equal three percent (3%) of Base Rent due from Tenant. Tenant shall pay the Property Management Fee in accordance with Section 9.2 with respect to the entire Term, including any extensions thereof or any holdover periods, regardless of whether Tenant is obligated to pay Base Rent, Operating Expenses or any other Rent with respect to any such period or portion thereof. For the first six (6) months of the Term, the Property Management Fee shall be calculated as if Tenant were paying Twenty-Three Thousand Nine Hundred Twenty-Two and 88/100 Dollars ($23,922.88) per month for Base Rent.

(x) Within ninety (90) days (or such longer period as may be reasonably required by Landlord) after the conclusion of each calendar year (commencing with calendar year 2016), Landlord shall furnish to Tenant a statement showing in reasonable detail the actual Operating Expenses for the previous calendar year and the Base Year, and Tenant's Adjusted Share of any increase in Operating Expenses for the previous calendar year over the Base Year ("Landlord's Statement"). Any additional sum due from Tenant to Landlord shall be due and payable within thirty (30) days after Tenant's receipt of Landlord's Statement. If the amounts paid by Tenant pursuant to this Section exceed Tenant's Adjusted Share of any increase in Operating Expenses for the previous calendar year over the Base Year, then Landlord shall credit the difference against the Rent next due and owing from Tenant; provided that, if the Lease term has expired, Landlord shall accompany Landlord's Statement with payment for the amount of such difference.

(y) Landlord's good faith estimate of Tenant's Pro Rata Share of any increase in Real Estate Taxes for the upcoming calendar year over the Base Year shall be completed in good faith by Landlord at the end of each calendar year and thereafter be payable to Landlord in equal estimated monthly installments as provided in Section 9.2, subject to readjustment when the actual amount of Real Estate Taxes is determined. After readjustment, any shortage shall be due and payable by Tenant within thirty (30) days after Tenant's receipt of Landlord's readjustment statement, and any overpayment by Tenant shall be credited by Landlord against the Rent next due and owing from Tenant; provided that, if the Term has expired, Landlord shall accompany any such statement of readjustment with payment for the amount of such overpayment. If the taxing authority provides an estimated tax bill, then monthly installments of Real Estate Taxes shall be based thereon until the final bill is ascertained. Landlord shall furnish to Tenant, upon Tenant's request, but not more than once in any calendar year, a copy of the tax bill and/or any estimated tax bill for the Project.

(z) Any amount due under this Section for any period that is less than a full month shall be prorated for such fractional month on the basis of the number of days in the month.

9.3. Landlord may, from time to time, modify Landlord's calculation and allocation procedures for Operating Expenses, so long as such modifications produce Dollar results substantially consistent with (i) Landlord's then-current reasonable management practices at the Project, and (ii) the requirements of this Article 9. Since the Project consists of multiple buildings, certain Operating Expenses may pertain to a particular building, certain Operating Expenses may pertain to the South Campus, and other Operating Expenses to the Project as a

whole. Landlord reserves the right in its sole discretion to allocate any such costs applicable to any particular building within the Project to such building, any costs applicable to the South Campus to the buildings comprising the South Campus (including the Building), and other such costs applicable to the Project to each building in the Project (including the Building), with the tenants in each building being responsible for paying their respective proportionate shares of their buildings to the extent required under their leases. Landlord shall allocate such costs to the buildings (including the Building) in a reasonable, non-discriminatory manner, and such allocation shall be binding on Tenant.

9.4. Tenant shall not be responsible for Real Estate Taxes or Operating Expenses with respect to any time period prior to the Term Commencement Date. Tenant's responsibility for Tenant's Adjusted Share of Operating Expenses and Tenant's Pro Rata Share of Real Estate Taxes shall continue to the latest of (a) the date of termination of the Lease, (b) the date Tenant has fully vacated the Premises and (c) if termination of the Lease is due to a default by Tenant, the date of rental commencement of a replacement tenant.

9.5. Real Estate Taxes and Operating Expenses for the calendar year in which Tenant's obligation to share therein commences and for the calendar year in which such obligation ceases shall be prorated on a basis reasonably determined by Landlord. Expenses such as taxes, assessments and insurance premiums that are incurred for an extended time period shall be prorated based upon the time periods to which they apply so that the amounts attributed to the Premises relate in a reasonable manner to the time period wherein Tenant has an obligation to share in Real Estate Taxes and Operating Expenses.

9.6. In the event that the Building, South Campus or Project is less than fully occupied during a calendar year, Tenant acknowledges that Landlord may extrapolate Operating Expenses that vary depending on the occupancy of the Building, South Campus or Project, as applicable, to equal Landlord's reasonable estimate of what such Operating Expenses would have been had the Building, South Campus or Project, as applicable, been ninety-five percent (95%) occupied during such calendar year; provided, however, that Landlord shall not recover more than one hundred percent (100%) of Operating Expenses.

10. Taxes on Tenant's Property.

10.1. Tenant shall pay prior to delinquency any and all taxes levied against any personal property or trade fixtures placed by Tenant in or about the Premises or on any gross or net receipts of or sales by Tenant.

10.2. If any such taxes on Tenant's personal property or trade fixtures are levied against Landlord or Landlord's property or, if the assessed valuation of the Building, the Property or the Project is increased by inclusion therein of a value attributable to Tenant's personal property or trade fixtures, and if Landlord, after written notice to Tenant, pays the taxes based upon any such increase in the assessed value of the Building, the Property or the Project, then Tenant shall, within thirty (30) days after written demand (accompanied by reasonable supporting documentation thereof), repay to Landlord the taxes so paid by Landlord.

10.3. If any improvements in or alterations to the Premises (excluding the initial Tenant Improvements), whether owned by Landlord or Tenant and whether or not affixed to the real property so as to become a part thereof, are assessed for real property tax purposes at a valuation higher than the valuation at which improvements conforming to Landlord's building standards (the "Building Standard") in other spaces in the Building are assessed, then the real property taxes and assessments levied against Landlord or the Building, the Property or the Project by reason of such excess assessed valuation shall be deemed to be taxes levied against personal property of Tenant and shall be governed by the provisions of Section 10.2. Any such excess assessed valuation due to improvements in or alterations to space in the Project leased by other tenants at the Project shall not be included in Operating Expenses. If the records of the applicable governmental assessor's office are available and sufficiently detailed to serve as a basis for determining whether such Tenant improvements or alterations are assessed at a higher valuation than the Building Standard, then such records shall be binding on both Landlord and Tenant.

11. Security Deposit.

11.1. Tenant shall deposit with Landlord on or before the Execution Date the sum set forth in Section 2.6 (the "Security Deposit"), which sum shall be held by Landlord as security for the faithful performance by Tenant of all of the terms, covenants and conditions of this Lease to be kept and performed by Tenant during the period commencing on the Execution Date and ending upon the expiration or termination of Tenant's obligations under this Lease. If Tenant Defaults (as defined below) with respect to any provision of this Lease, including any provision relating to the payment of Rent, then Landlord may (but shall not be required to) use, apply or retain all or any part of the Security Deposit for the payment of any Rent or any other sum in default, or to compensate Landlord for any other loss or damage that Landlord may suffer by reason of Tenant's default. If any portion of the Security Deposit is so used or applied, then Tenant shall, within ten (10) days following demand therefor, deposit cash with Landlord in an amount sufficient to restore the Security Deposit to its original amount, and Tenant's failure to do so shall be a material breach of this Lease. The provisions of this Article shall survive the expiration or earlier termination of this Lease.

11.2. In the event of bankruptcy or other debtor-creditor proceedings against Tenant, the Security Deposit shall be deemed to be applied first to the payment of Rent and other charges due Landlord for all periods prior to the filing of such proceedings.

11.3. Landlord may deliver to any purchaser of Landlord's interest in the Premises the funds deposited hereunder by Tenant, and thereupon Landlord shall be discharged from any further liability with respect to such deposit. This provision shall also apply to any subsequent transfers.

11.4. If Tenant shall fully and faithfully perform every provision of this Lease to be performed by it, then the Security Deposit, or any balance thereof, shall be returned to Tenant (or, at Landlord's option, to the last assignee of Tenant's interest hereunder) within thirty (30) days after the expiration or earlier termination of this Lease.

11.5. [Intentionally omitted]

11.6. If the Security Deposit shall be in cash, Landlord shall hold the Security Deposit in an account at a banking organization selected by Landlord; provided, however, that Landlord shall not be required to maintain a separate account for the Security Deposit, but may intermingle it with other funds of Landlord. Landlord shall be entitled to all interest and/or dividends, if any, accruing on the Security Deposit. Landlord shall not be required to credit Tenant with any interest for any period during which Landlord does not receive interest on the Security Deposit.

11.7. The Security Deposit may be in the form of cash, a letter of credit or any other security instrument acceptable to Landlord in its sole discretion. Tenant may at any time, except when Tenant is in Default (as defined below), deliver a letter of credit (the "L/C Security") as the entire Security Deposit, as follows:

(a) If Tenant elects to deliver L/C Security, then Tenant shall provide Landlord, and maintain in full force and effect throughout the Term and until the date that is six (6) months after the then-current Term Expiration Date, a letter of credit in the form of Exhibit E issued by an issuer reasonably satisfactory to Landlord, in the amount of the Security Deposit, with an initial term of at least one year. Landlord may require the L/C Security to be re-issued by a different issuer at any time during the Term if Landlord reasonably believes that the issuing bank of the L/C Security is or may soon become insolvent; provided, however, Landlord shall return the existing L/C Security to the existing issuer immediately upon receipt of the substitute L/C Security. If any issuer of the L/C Security shall become insolvent or placed into FDIC receivership, then Tenant shall immediately deliver to Landlord (without the requirement of notice from Landlord) substitute L/C Security issued by an issuer reasonably satisfactory to Landlord, and otherwise conforming to the requirements set forth in this Article. As used herein with respect to the issuer of the L/C Security, "insolvent" shall mean the determination of insolvency as made by such issuer's primary bank regulator *(i.e.*, the state bank supervisor for state chartered banks; the OCC or OTS, respectively, for federally chartered banks or thrifts; or the Federal Reserve for its member banks). If, at the Term Expiration Date, any Rent remains uncalculated or unpaid, then (i) Landlord shall with reasonable diligence complete any necessary calculations, (ii) Tenant shall extend the expiry date of such L/C Security from time to time as Landlord reasonably requires and (iii) in such extended period, Landlord shall not unreasonably refuse to consent to an appropriate reduction of the L/C Security. Tenant shall reimburse Landlord's legal costs (as estimated by Landlord's counsel) in handling Landlord's acceptance of L/C Security or its replacement or extension.

(b) If Tenant delivers to Landlord satisfactory L/C Security in place of the entire Security Deposit, Landlord shall refund to Tenant any cash Security Deposit Landlord previously held.

(c) Landlord may draw upon the L/C Security, and hold and apply the proceeds in the same manner and for the same purposes as the Security Deposit, if (i) an uncured Default (as defined below) exists, (ii) as of the date forty-five (45) days before any L/C Security expires (even if such scheduled expiry date is after the Term Expiration Date) Tenant has not delivered to Landlord an amendment or replacement for such L/C Security, reasonably satisfactory to Landlord, extending the expiry date to the earlier of (1) six (6) months after the

then-current Term Expiration Date or (2) the date one year after the then-current expiry date of the L/C Security, (iii) the L/C Security provides for automatic renewals, Landlord asks the issuer to confirm the current L/C Security expiry date, and the issuer fails to do so within ten (10) business days, (iv) Tenant fails to pay (when and as Landlord reasonably requires) any bank charges for Landlord's transfer of the L/C Security or (v) the issuer of the L/C Security ceases, or announces that it will cease, to maintain an office in the city where Landlord may present drafts under the L/C Security (and fails to permit drawing upon the L/C Security by overnight courier or facsimile). This Section does not limit any other provisions of this Lease allowing Landlord to draw the L/C Security under specified circumstances.

(d) Tenant shall not seek to enjoin, prevent, or otherwise interfere with Landlord's draw under L/C Security, even if it violates this Lease. Tenant acknowledges that the only effect of a wrongful draw would be to substitute a cash Security Deposit for L/C Security, causing Tenant no legally recognizable damage. Landlord shall hold the proceeds of any draw in the same manner and for the same purposes as a cash Security Deposit. In the event of a wrongful draw, the parties shall cooperate to allow Tenant to post replacement L/C Security simultaneously with the return to Tenant of the wrongfully drawn sums, and Landlord shall upon request confirm in writing to the issuer of the L/C Security that Landlord's draw was erroneous.

(e) If Landlord transfers its interest in the Premises, then Tenant shall at Tenant's expense, within ten (10) business days after receiving a request from Landlord, deliver (and, if the issuer requires, Landlord shall consent to) an amendment to the L/C Security naming Landlord's grantee as substitute beneficiary. If the required Security Deposit changes while L/C Security is in force, then Tenant shall deliver (and, if the issuer requires, Landlord shall consent to) a corresponding amendment to the L/C Security.

12. Use.

12.1. Tenant shall use the Premises for the Permitted Use, and shall not use the Premises, or permit or suffer the Premises to be used, for any other purpose without Landlord's prior written consent, which consent Landlord may withhold in its sole and absolute discretion.

12.2. Tenant shall not use or occupy the Premises in violation of Applicable Laws; zoning ordinances; or the certificate of occupancy issued for the Building or the Project, and shall, upon five (5) business days' written notice from Landlord, discontinue any use of the Premises that is declared or claimed by any Governmental Authority having jurisdiction to be a violation of any of the above, or that in Landlord's reasonable opinion violates any of the above. Tenant shall comply with any direction of any Governmental Authority having jurisdiction that shall, by reason of the nature of Tenant's use or occupancy of the Premises, impose any duty upon Tenant or Landlord with respect to the Premises or with respect to the use or occupation thereof.

12.3. Tenant shall not do or permit to be done anything that will invalidate or increase the cost of any fire, environmental, extended coverage or any other insurance policy covering the Building or the Project, and shall comply with all rules, orders, regulations and requirements of the insurers of the Building and the Project, and Tenant shall promptly, within thirty (30) days after written demand (accompanied by reasonable supporting documentation thereof), reimburse

Landlord for any additional premium charged for such policy by reason of Tenant's failure to comply with the provisions of this Article.

12.4. Tenant shall keep all doors opening onto public corridors closed, except when in use for ingress and egress.

12.5. No additional locks or bolts of any kind shall be placed upon any of the doors or windows by Tenant, nor shall any changes be made to existing locks or the mechanisms thereof without Landlord's prior written consent, not to be unreasonably withheld, conditioned or delayed. Tenant shall, upon termination of this Lease, return to Landlord all keys to offices and restrooms either furnished to or otherwise procured by Tenant. In the event any key so furnished to Tenant is lost, Tenant shall pay to Landlord the cost of replacing the same or of changing the lock or locks opened by such lost key if Landlord shall reasonably deem it necessary to make such change. Landlord shall provide Tenant with forty (40) access cards to the Building at no additional cost.

12.6. No awnings or other projections shall be attached to any outside wall of the Building. No curtains, blinds, shades or screens shall be attached to or hung in, or used in connection with, any window or door of the Premises other than Landlord's standard window coverings. Neither the interior nor exterior of any windows shall be coated or otherwise sunscreened without Landlord's prior written consent, nor shall any bottles, parcels or other articles be placed on the windowsills or items attached to windows that are visible from outside the Premises. No equipment, furniture or other items of personal property shall be placed on any exterior balcony without Landlord's prior written consent.

12.7. Subject to compliance with all Applicable Laws, Tenant shall have the right to install and maintain, at Tenant's sole cost and expense, one (1) exterior sign on the exterior of the Building identifying Tenant ("Exterior Signage"). The size, color, type and location of Exterior Signage shall be subject to Landlord's reasonable approval. No sign, advertisement or notice (including the Exterior Signage, "Signage") shall be exhibited, painted or affixed by Tenant on any part of the Premises, which Signage is visible from outside the Premises, or the Building without Landlord's prior written consent, which consent shall not be unreasonably withheld. Signage (other than within the Premises which is not visible from outside the Premises) shall conform to Landlord's standard design criteria. For any Signage, Tenant shall, at Tenant's own cost and expense, (a) acquire all permits for such Signage in compliance with Applicable Laws and (b) design, fabricate, install and maintain such Signage in a first-class condition. Prior to the expiration or earlier termination of this Lease, Tenant shall remove Exterior Signage at Tenant's sole cost and expense, and Tenant shall reimburse Landlord for costs incurred by Landlord in removing any of Tenant's Signage upon the expiration or earlier termination of the Lease. Initial interior signs on entry doors to the Premises and the directory tablet shall be inscribed, painted or affixed for Tenant by Landlord at Landlord's sole cost and expense, and shall be of a Building-standard size, color and type and be located in a place reasonably acceptable to Landlord. The directory tablet shall be provided exclusively for the display of the name and location of tenants only. Tenant shall not place anything on the exterior of the corridor walls or corridor doors other than Landlord's standard lettering. At Landlord's option, Landlord may install any Tenant Signage, and Tenant shall pay all reasonable out-of-

pocket costs (without mark-up, overhead or profit) associated with such installation within thirty (30) days after demand therefor (accompanied by reasonable supporting documentation thereof). The right to install Exterior Signage is personal to REGENXBIO Inc., and may not be exercised by any assignee, sublessee or transferee of this Lease, except to Tenant's Affiliate subsequent to an Exempt Transfer (as defined below). If Landlord elects, in Landlord's sole and absolute discretion, to make monument Signage available to all tenants of the Project, then Landlord shall make a portion of such monument Signage (with the size and location of such portion to be determined by Landlord in Landlord's sole and absolute discretion) available to Tenant for purposes of installing and maintaining Signage identifying Tenant (which Signage shall be installed, maintained and removed at Tenant's sole cost and expense and otherwise in accordance with all of the terms, conditions and provisions of this Section).

12.8. Tenant may only place equipment within the Premises with floor loading consistent with the Building's structural design unless Tenant obtains Landlord's prior written approval. Tenant may place such equipment only in a location designed to carry the weight of such equipment.

12.9. Tenant shall cause any equipment or machinery to be installed in the Premises so as to reasonably prevent sounds or vibrations therefrom from extending into the Common Area or other leased areas in the Project.

12.10. Tenant shall not (a) do or permit anything to be done in or about the Premises that shall in any way obstruct or interfere with the rights of other tenants or occupants of the Project, or injure or annoy them, (b) use or allow the Premises to be used for immoral, unlawful or objectionable purposes, (c) cause, maintain or permit any nuisance or waste in, on or about the Project or (d) take any other action that would in Landlord's reasonable determination in any manner adversely affect other tenants' quiet use and enjoyment of their space or adversely impact their ability to conduct business in a professional and suitable work environment. Notwithstanding anything in this Lease to the contrary, Tenant may not install any security systems (including cameras) outside the Premises or that record sounds or images outside the Premises without Landlord's prior written consent, which Landlord may withhold in its sole and absolute discretion.

12.11. Notwithstanding any other provision herein to the contrary (but subject to Landlord's obligations in Section 5(y) above), Tenant shall be responsible for all liabilities, costs and expenses arising out of or in connection with the compliance of the Premises with the Americans with Disabilities Act, 42 U.S.C. § 12101, et seq., and any state and local accessibility laws, codes, ordinances and rules (collectively, and together with regulations promulgated pursuant thereto, the "ADA"), and Tenant shall indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold Landlord and its affiliates, employees, agents and contractors; and any lender, mortgagee, ground lessor or beneficiary (each, a "Lender" and, collectively with Landlord and its affiliates, employees, agents and contractors, the "Landlord Indemnitees") harmless from and against any demands, claims, liabilities, losses, costs, expenses, actions, causes of action, damages, suits or judgments, and all reasonable expenses (including reasonable attorneys' fees, charges and disbursements, regardless of whether the applicable demand, claim, action, cause of action or suit is voluntarily withdrawn

or dismissed) incurred in investigating or resisting the same (collectively, "Claims") arising out of any such failure of the Premises to comply with the ADA. For the avoidance of doubt, "Lenders" shall also include historic tax credit investors and new market tax credit investors. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

13. Rules and Regulations, CC&Rs, Parking Facilities and Common Area.

13.1. Tenant shall have the non-exclusive right, in common with others, to use the Common Area in conjunction with Tenant's use of the Premises for the Permitted Use, and such use of the Common Area and Tenant's use of the Premises shall be subject to the rules and regulations adopted by Landlord and attached hereto as Exhibit F, together with such other reasonable and nondiscriminatory rules and regulations as are hereafter promulgated by Landlord in its sole and absolute discretion (the "Rules and Regulations"); provided, however, that such other rules and regulations shall not be binding upon Tenant until Tenant has received a written copy thereof and provided further that any such other rules and regulations shall not materially modify Tenant's rights or obligations hereunder. Tenant shall and shall ensure that its contractors, subcontractors, employees, subtenants and invitees faithfully observe and comply with the Rules and Regulations. Landlord shall not be responsible to Tenant for the violation or non-performance by any other tenant or any agent, employee or invitee thereof of any of the Rules and Regulations. In the event of a conflict or any inconsistency between the Rules and Regulations and the Lease, the Lease shall prevail.

13.2. This Lease is subject to any recorded covenants, conditions or restrictions on the Project or Property, as the same may be amended, amended and restated, supplemented or otherwise modified from time to time (the "CC&Rs"); provided that any such amendments, restatements, supplements or modifications do not materially modify Tenant's rights or obligations hereunder. Tenant shall comply with the CC&Rs.

13.3. Tenant shall have a non-exclusive, irrevocable license to use Tenant's Pro Rata Share of parking facilities serving the Project in common on an unreserved basis with other tenants of the Project during the Term at no additional cost. As of the Execution Date, Tenant's Pro Rata Share of the parking facilities described above is equal to two point six (2.6) parking spaces per one thousand (1,000) square feet of Rentable Area of the Premises.

13.4. Tenant agrees not to unreasonably overburden the parking facilities serving the Project and agrees to reasonably cooperate with Landlord and other tenants in the use of the parking facilities. Landlord reserves the right to determine that parking facilities are becoming overcrowded and to limit Tenant's use thereof. Upon such determination, Landlord may reasonably allocate parking spaces among Tenant and other tenants of the Building or the Project. Nothing in this Section, however, is intended to create an affirmative duty on Landlord's part to monitor parking.

14. Project Control by Landlord.

14.1. Landlord reserves full control over the Building and the Project to the extent not inconsistent with (a) Tenant's enjoyment of the Premises as provided by this Lease, (b) Tenant's rights under this Lease or (c) Landlord's obligations under this Lease. This reservation includes Landlord's right to subdivide the Project; convert the Building and other buildings within the

Project to condominium units; change the size of the Project by selling all or a portion of the Project or adding real property and any improvements thereon to the Project; grant easements and licenses to third parties; maintain or establish ownership of the Building separate from fee title to the Property; make additions to or reconstruct portions of the Building and the Project; install, use, maintain, repair, replace and relocate for service to the Premises and other parts of the Building or the Project pipes, ducts, conduits, wires and appurtenant fixtures, wherever located in the Premises (provided that such installation, use, maintenance, repair, replacement or relocation does not materially reduce the useable area of the Premises), the Building or elsewhere at the Project; access areas of the Premises necessary for utilities, services, safety and operation of the Building; and alter or relocate any other Common Area or facility, including private drives, lobbies, entrances and landscaping; provided, however, that any entry into the Premises shall be subject to the provisions of Section 14.4 of this Lease, and such rights shall be exercised in a way that does not materially adversely affect Tenant's beneficial use and occupancy of the Premises, including the Permitted Use and Tenant's access to the Premises. Tenant acknowledges that Landlord specifically reserves the right to allow the exclusive use of corridors and restroom facilities located on specific floors to one or more tenants occupying such floors; provided, however, that Tenant shall not be deprived of the use of the corridors reasonably required to serve the Premises or of restroom facilities serving the floor upon which the Premises are located.

14.2. Possession of areas of the Premises necessary for utilities, services, safety and operation of the Building is reserved to Landlord.

14.3. Tenant shall, at Landlord's request, promptly execute such further documents as may be reasonably appropriate to assist Landlord in the performance of its obligations hereunder; provided that Tenant need not execute any document that creates additional liability or obligations for Tenant or that deprives Tenant of any rights (including, without limitation, the quiet enjoyment and use of, and access to, the Premises) as provided for in this Lease.

14.4. Landlord may, at any and all reasonable times during non-business hours (or during business hours, if (a) with respect to Subsections 14.4(u) through 14.4(y), Tenant so requests, and (b) with respect to Subsection 14.4(z), if Landlord so requests), and upon twenty-four (24) hours' prior notice (which may be oral or by email to the office manager or other Tenant-designated individual at the Premises; but provided that no time restrictions shall apply or advance notice be required if an emergency necessitates immediate entry), enter the Premises to (t) exercise its rights under Sections 14.1 and 21.4, (u) inspect the same and to determine whether Tenant is in compliance with its obligations hereunder, (v) supply any service Landlord is required to provide hereunder, (w) alter, improve or repair any portion of the Building other than the Premises for which access to the Premises is reasonably necessary, (x) post notices of nonresponsibility, (y) access the telephone equipment, electrical substation and fire risers and (z) show the Premises to prospective tenants during the final nine (9) months of the Term and current and prospective purchasers and lenders at any time. In connection with any such alteration, improvement or repair as described in Subsection 14.4(w), Landlord may erect in the Premises or elsewhere in the Project scaffolding and other structures to the extent and for the time reasonably required for the alteration, improvement or repair work to be performed. In no event shall Tenant's Rent abate as a result of Landlord's activities pursuant to this Section;

provided, however, that all such activities shall be conducted in such a manner so as to cause as little interference to Tenant as is reasonably possible. Landlord shall at all times retain a key with which to unlock all of the doors in the Premises. If an emergency necessitates immediate access to the Premises, Landlord may use whatever force is necessary to enter the Premises, and any such entry to the Premises shall not constitute a forcible or unlawful entry to the Premises, a detainer of the Premises, or an eviction of Tenant from the Premises or any portion thereof.

15. Quiet Enjoyment. Landlord covenants that Tenant, upon paying the Rent and performing its obligations contained in this Lease, may peacefully and quietly have, hold and enjoy the Premises, free from any claim or interference by Landlord or persons claiming by, through or under Landlord, but subject to all of the terms and provisions hereof, provisions of Applicable Laws and rights of record to which this Lease is or may become subordinate. This covenant is in lieu of any other quiet enjoyment covenant, either express or implied.

16. Utilities and Services.

16.1. Landlord shall pay for all water (from a local municipal or similar source), gas and electricity supplied to the Premises, and Landlord shall be entitled to include these sums in Operating Expenses (provided, however, that utility costs attributable to the Supplemental HVAC Unit (as defined below) shall be paid by Tenant in accordance with Section 16.11 below). Landlord may base its bills for utilities on reasonable estimates; provided that Landlord adjusts such billings promptly thereafter or as part of the next Landlord's Statement to reflect the actual cost of providing utilities to the Premises. To the extent that Tenant uses more than Tenant's Pro Rata Share of any utilities, then Tenant shall pay Landlord for Tenant's Adjusted Share of such utilities to reflect such excess. In the event that the Building, South Campus or Project is less than fully occupied during a calendar year, Tenant acknowledges that Landlord may extrapolate utility usage that varies depending on the occupancy of the Building, South Campus or Project (as applicable) to equal Landlord's reasonable estimate of what such utility usage would have been had the Building, South Campus or Project, as applicable, been ninety-five percent (95%) occupied during such calendar year; provided, however, that Landlord shall not recover more than one hundred percent (100%) of the cost of such utilities. Tenant shall not be liable for the cost of utilities supplied to the Premises attributable to the time period prior to the Term Commencement Date; provided, however, that, if Landlord shall permit Tenant possession of the Premises prior to the Term Commencement Date and Tenant uses the Premises for any purpose other than placement of cabling, equipment, furniture and personal property as set forth in Section 4.3, then Tenant shall be responsible for the cost of utilities supplied to the Premises from such earlier date of possession.

16.2. Landlord shall not be liable for, nor shall any eviction of Tenant result from, the failure to furnish any utility or service, whether or not such failure is caused by accidents; breakage; casualties (to the extent not caused by the party claiming Force Majeure); Severe Weather Conditions (as defined below); physical natural disasters (but excluding weather conditions that are not Severe Weather Conditions); strikes, lockouts or other labor disturbances or labor disputes (other than labor disturbances and labor disputes resulting solely from the acts or omissions of the party claiming Force Majeure); acts of terrorism; riots or civil disturbances; wars or insurrections; shortages of materials (which shortages are not unique to the party claiming Force Majeure); government regulations, moratoria or other governmental actions,

inactions or delays; failures by third parties to deliver gas, oil or another suitable fuel supply, or inability of the party claiming Force Majeure, by exercise of reasonable diligence, to obtain gas, oil or another suitable fuel; or other causes beyond the reasonable control of the party claiming that Force Majeure has occurred (collectively, "Force Majeure"); or, to the extent permitted by Applicable Laws, Landlord's negligence. In the event of such failure, Tenant shall not be entitled to termination of this Lease or any abatement or reduction of Rent, nor shall Tenant be relieved from the operation of any covenant or agreement of this Lease. "Severe Weather Conditions" means weather conditions that are materially worse than those that reasonably would be anticipated for the Property at the applicable time based on historic meteorological records. Notwithstanding anything to the contrary in this Lease, if, for more than ten (10) consecutive business days following written notice to Landlord and as a direct result of Landlord's gross negligence or willful misconduct (and except to the extent that such failure is caused in whole or in part by the action or inaction of a Tenant Party (as defined below)), the provision of Base HVAC (as defined below) or other utilities to all or a material portion of the Premises that Landlord must provide pursuant to this Lease is interrupted (a "Material Services Failure"), then Tenant's Base Rent and Tenant's obligations under this Lease to pay Real Estate Taxes and Operating Expenses (or, to the extent that less than all of the Premises are affected, a proportionate amount (based on the Rentable Area of the Premises that is rendered unusable) of Base Rent and Tenant's obligations under this Lease to pay Real Estate Taxes and Operating Expenses) shall thereafter be abated until the Premises are again usable by Tenant for the Permitted Use; provided, however, that, if Landlord is diligently pursuing the restoration of such Base HVAC and other utilities and Landlord provides substitute Base HVAC and other utilities reasonably suitable for Tenant's continued use and occupancy of the Premises for the Permitted Use (e.g., supplying portable air conditioning equipment), then neither Base Rent nor Tenant's obligations under this Lease to pay Real Estate Taxes and Operating Expenses shall be abated. During any Material Services Failure, Tenant will cooperate with Landlord to arrange for the provision of any interrupted utility services on an interim basis via temporary measures until final corrective measures can be accomplished, and Tenant will permit Landlord the necessary access to the Premises to remedy such Material Service Failure. In the event of any interruption of Base HVAC or other utilities that Landlord must provide pursuant to this Lease, regardless of the cause, Landlord shall diligently pursue the restoration of such Base HVAC and other utilities. Notwithstanding anything in this Lease to the contrary, but subject to Article 24 (which shall govern in the event of a casualty), the provisions of this Section shall be Tenant's sole recourse and remedy in the event of an interruption of Base HVAC or other utilities to the Premises.

16.3. Tenant shall pay for, prior to delinquency of payment therefor, any utilities and services that may be furnished to the Premises during or, if Tenant occupies the Premises after the expiration or earlier termination of the Term, after the Term, beyond those utilities provided by Landlord, including telephone, internet service, cable television other telecommunications and other utilities together with any fees, surcharges and taxes thereon. Upon Landlord's demand, utilities and services provided to the Premises that are separately metered shall be paid by Tenant directly to the supplier of such utilities or services. If any such utility for which Tenant is responsible is not separately metered to Tenant, Tenant shall pay, no more often than monthly, Tenant's Adjusted Share of all charges of such utility (at the rate charged to the Project by the utility provider, without markup, overhead or profit) jointly metered with other premises as Additional Rent (outside of Operating Expenses within thirty (30) days of receiving an

invoice from Landlord) or, in the alternative, Landlord may, at its option, and sole cost (outside of Operating Expenses), meter the usage of such utilities by Tenant, and Tenant shall pay, no more often than monthly, all charges of such utility (at the rate charged to the Project by the utility provider, without mark-up, overhead or profit) as Additional Rent (outside of Operating Expenses within thirty (30) days of receiving an invoice from Landlord).

16.4. Tenant shall not, without Landlord's prior written consent, use any device in the Premises (including data processing machines) that will in any way (a) increase the amount of ventilation, air exchange, gas, steam, electricity or water required or consumed in the Premises based upon Tenant's Pro Rata Share of the Building or Project (as applicable) beyond the existing capacity of the Building or the Project usually furnished or supplied for the Permitted Use or (b) exceed Tenant's Pro Rata Share of the Building's or Project's (as applicable) capacity to provide such utilities or services.

16.5. If Tenant shall require utilities or services materially in excess of those usually furnished or supplied for tenants in similar spaces in the Building or the Project by reason of Tenant's equipment or extended hours of business operations, then Tenant shall first procure Landlord's consent for the use thereof, which consent Landlord may condition upon the availability of such excess utilities or services, and Tenant shall pay as Additional Rent an amount equal to the cost of providing such excess utilities and services.

16.6. Landlord shall provide water in Common Area for lavatory and landscaping purposes only, which water shall be from the local municipal or similar source; provided, however, that if Landlord reasonably determines that Tenant requires, uses or consumes water provided to the Common Area for any purpose other than ordinary lavatory purposes, and such additional requirement, use or consumption is not promptly discontinued after written demand from Landlord, Landlord may install a water meter ("Tenant Water Meter") and thereby measure Tenant's water consumption for all purposes. Tenant shall pay Landlord for the costs of any Tenant Water Meter and the installation and maintenance thereof during the Term. If Landlord installs a Tenant Water Meter, Tenant shall pay for water consumed, as shown on such meter, as and when bills are rendered. If Tenant fails to timely make such payments, Landlord may pay such charges and collect the same from Tenant. Any such costs or expenses incurred or payments made by Landlord for any of the reasons or purposes stated in this Section shall be deemed to be Additional Rent payable by Tenant and collectible by Landlord as such.

16.7. Landlord reserves the right to stop service of the elevator, plumbing, ventilation, air conditioning and utility systems, when Landlord reasonably deems necessary, due to accident, emergency or the need to make repairs, alterations or improvements, until such repairs, alterations or improvements shall have been completed, and except as provided in Section 16.2, Landlord shall further have no responsibility or liability for failure to supply elevator facilities, plumbing, ventilation, air conditioning or utility service when prevented from doing so by Force Majeure or, to the extent permitted by Applicable Laws, Landlord's negligence. Without limiting the foregoing, it is expressly understood and agreed that any covenants on Landlord's part to furnish any service pursuant to any of the terms, covenants, conditions, provisions or agreements of this Lease, or to perform any act or thing for the benefit of Tenant, shall not be

deemed breached if Landlord is unable to furnish or perform the same by virtue of Force Majeure or, to the extent permitted by Applicable Laws, Landlord's negligence.

16.8. Landlord shall provide janitorial and cleaning services to the Premises meeting the specifications attached hereto as Exhibit K, Monday through Friday excluding holidays recognized as such by the federal government and/or the State of Maryland.

16.9. For the Premises, Landlord shall (a) maintain and operate the HVAC systems serving the Premises (excluding the Supplemental HVAC Unit) used for the Permitted Use only ("Base HVAC") and (b) subject to Subsection 16.9(a), furnish Base HVAC as reasonably required (except as this Lease otherwise provides or as to any special requirements that arise from Tenant's particular use of the Premises) for reasonably comfortable occupancy of the Premises twenty-four (24) hours a day, every day during the Term, subject to casualty, eminent domain or as otherwise specified in this Article; provided that Tenant complies with the next sentence. If Tenant will require Base HVAC outside normal business hours of business days (defined as Monday through Friday from 8:00 a.m. until 6:00 p.m. and Saturday from 8:00 a.m. until 12:00 p.m.) in the Premises ("Overtime HVAC"), then Landlord shall be obligated to provide Overtime HVAC only if Tenant (y) requests it by 4 p.m. on the immediately preceding business day and (z) requires a minimum of at least two (2) hours of Overtime HVAC. Tenant shall pay Landlord, as Additional Rent, one hundred percent (100%) of Landlord's actual total cost of delivering steam and chilled water for Overtime HVAC for the Premises, the rate of which is currently Fifty Dollars ($50.00) per hour. Notwithstanding anything to the contrary in this Section (but subject to Section 16.2 above), Landlord shall have no liability, and Tenant shall have no right or remedy, on account of any interruption or impairment in Base HVAC services; provided that Landlord diligently endeavors to cure any such interruption or impairment.

16.10. For any utilities serving the Premises for which Tenant is billed directly by such utility provider, Tenant agrees to furnish to Landlord, within thirty (30) days after Landlord's request, (a) any invoices or statements for such utilities, (b) any other utility usage information reasonably requested by Landlord, and (c) authorization to allow Landlord to access Tenant's usage information reasonably available and reasonably necessary for Landlord to complete an ENERGY STAR® Statement of Performance (or similar comprehensive utility usage report, if requested by Landlord) and any other reasonably available information as reasonably requested by Landlord for the immediately preceding year; and Tenant shall comply with any other energy usage or consumption requirements required by Applicable Laws. Tenant shall retain records of utility usage at the Premises, including invoices and statements from the utility provider, for at least sixty (60) months, or such other period of time as may be requested by Landlord. Tenant acknowledges that any utility information for the Premises, the Building and the Project may be shared with third parties, including Landlord's consultants and Governmental Authorities. In addition to the foregoing, Tenant shall comply with all Applicable Laws related to the disclosure and tracking of energy consumption at the Premises. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

16.11. Notwithstanding anything to the contrary in this Lease, any supplemental HVAC unit servicing the Premises (the "Supplemental HVAC Unit") shall be the sole responsibility of

Tenant and Landlord shall have no obligations with respect thereto. Tenant shall, at its sole cost and expense, maintain and keep the Supplemental HVAC Unit in good condition and repair and shall otherwise be solely responsible for any repair, maintenance and/or replacement costs with respect to the Supplemental HVAC Unit. Tenant shall keep in full force and effect during the Term (and occupancy by Tenant, if any, after termination of this Lease) a preventative maintenance contract for quarterly, semi-annual, and annual Supplemental HVAC Unit inspections and maintenance using a qualified, licensed, bonded service provider reasonably approved by Landlord. If requested in writing by Landlord, Tenant shall provide to Landlord copies of the Supplemental HVAC Unit maintenance contracts and the Supplemental HVAC Unit maintenance reports on a quarterly basis. In the event Landlord determines that Tenant is not properly maintaining the Supplemental HVAC Unit, Landlord may take over Tenant's responsibilities with respect to the Supplemental HVAC Unit. Any such costs or expenses incurred, or payments made by Landlord as a result of Tenant failing to properly maintain the Supplemental HVAC Unit, shall be deemed to be Additional Rent payable by Tenant outside of Operating Expenses within thirty (30) days of receiving an invoice therefor. Notwithstanding anything to the contrary in this Lease, commencing on the Term Commencement Date and continuing throughout the Term, Tenant shall pay to Landlord (as Additional Rent outside of Operating Expenses within thirty (30) days of receiving an invoice therefor) all utility charges in connection with the Supplemental HVAC Unit. Tenant shall pay to Landlord (as Additional Rent outside of Operating Expenses within thirty (30) days of receiving an invoice therefor) an amount equal to all costs paid or incurred by Landlord in connection with purchasing, installing and monitoring any metering equipment Landlord deems reasonably necessary to monitor utility consumption with respect to the Supplemental HVAC Unit. Upon Landlord's demand, utility charges in connection with the Supplemental HVAC Unit (if separately metered) shall be paid by Tenant directly to the supplier of such utilities. Notwithstanding anything to the contrary in this Lease, Landlord shall have no liability, and Tenant shall have no right or remedy, on account of any interruption or impairment with respect to the Supplemental HVAC Unit.

17. Alterations.

17.1. Tenant shall make no alterations, additions or improvements in or to the Premises or engage in any construction, demolition, reconstruction, renovation or other work (whether major or minor) of any kind in, at or serving the Premises ("Alterations") without Landlord's prior written approval, which approval Landlord shall not unreasonably withhold, condition or delay; provided, however, that, in the event any proposed Alteration affects (a) any structural portions of the Building, including exterior walls, the roof, the foundation or slab, foundation or slab systems (including barriers and subslab systems) or the core of the Building, (b) the exterior of the Building or (c) any Building systems, including elevator, plumbing, HVAC, electrical, security, life safety and power, then Landlord may withhold its approval in its sole and absolute discretion. Tenant shall, in making any Alterations, use only those architects, contractors, suppliers and mechanics of which Landlord has given prior written approval, which approval shall not be unreasonably withheld, conditioned or delayed. In seeking Landlord's approval, Tenant shall provide Landlord, at least twenty (20) days in advance of any proposed construction, with plans, specifications, bid proposals, certified stamped engineering drawings and calculations by Tenant's engineer of record or architect of record (including connections to the Building's structural system, modifications to the Building's envelope, non-structural penetrations in slabs or walls, and modifications or tie-ins to life safety systems), work contracts,

requests for laydown areas and such other information concerning the nature and cost of the Alterations as Landlord may reasonably request. In no event shall Tenant use or Landlord be required to approve any architects, consultants, contractors, subcontractors or material suppliers that Landlord reasonably believes could cause labor disharmony, and Tenant agrees to comply with all reasonable requirements of Landlord relative to union and non-union labor. Notwithstanding the foregoing, Tenant may make strictly cosmetic changes to the Premises that do not require any permits or more than three (3) total contractors and subcontractors ("Cosmetic Alterations") without Landlord's consent; provided that (y) the cost of any Cosmetic Alterations does not exceed Forty Thousand Dollars ($40,000) annually, (z) such Cosmetic Alterations do not (i) require any structural or other substantial modifications to the Premises, (ii) require any changes to or adversely affect the Building systems, (iii) affect the exterior of the Building or (iv) trigger any requirement under Applicable Laws that would require Landlord to make any alteration or improvement to the Premises, the Building or the Project. Tenant shall give Landlord at least ten (10) days' prior written notice of any Cosmetic Alterations.

17.2. Tenant shall not construct or permit to be constructed partitions or other obstructions that might interfere with free access to mechanical installation or service facilities of the Building or with other tenants' components located within the Building, or interfere with the moving of Landlord's equipment to or from the enclosures containing such installations or facilities.

17.3. Tenant shall accomplish any work performed on the Premises or the Building in such a manner as to permit any life safety systems to remain fully operable at all times.

17.4. Any work performed on the Premises, the Building or the Project by Tenant or Tenant's contractors shall be done at such times and in such manner as Landlord may from time to time reasonably designate, provided that except for noisy or disruptive work (as reasonably determined by Landlord), all such work may be performed during normal business hours. Tenant covenants and agrees that all work done by Tenant or Tenant's contractors shall be performed in full compliance with Applicable Laws. Within thirty (30) days after completion of any Alterations, and to the extent applicable (as reasonably determined by Landlord), Tenant shall provide Landlord with complete "as built" drawing print sets and electronic CADD files on disc (or files in such other current format in common use as Landlord reasonably approves or requires) showing any changes in the Premises, as well as a commissioning report prepared by a licensed, qualified third-party commissioning agent hired by Tenant and reasonably approved by Landlord for all new or affected mechanical, electrical and plumbing systems. Any such "as built" plans shall show the applicable Alterations as an overlay on the Building as-built plans; provided that Landlord provides the Building "as built" plans to Tenant.

17.5. Before commencing any Alterations, Tenant shall give Landlord at least twenty (20) days' prior written notice of the proposed commencement of such work and shall, if required by Landlord, secure, at Tenant's own cost and expense, a completion and lien indemnity bond satisfactory to Landlord for such work.

17.6. Tenant shall repair any damage to the Premises caused by Tenant's removal of any property from the Premises. During any such restoration period extending after the expiration or earlier termination of the Term, Tenant shall pay Rent to Landlord as provided

herein as if such space were otherwise occupied by Tenant. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

17.7. The Premises plus any Alterations, Signage, Tenant Improvements, attached equipment, decorations, fixtures, casework and related appliances, trade fixtures, and additions and improvements attached to or built into the Premises made by either of the parties (including all floor and wall coverings; paneling; sinks and related plumbing fixtures; ductwork; conduits; electrical panels and circuits; business and trade fixtures; attached machinery and equipment; and built-in furniture and cabinets, in each case, together with all additions and accessories thereto), shall (unless, prior to construction or installation thereof, Landlord elects otherwise in writing) at all times remain the property of Landlord, shall remain in the Premises and shall (unless, prior to such construction or installation, Landlord elects otherwise in writing) be surrendered to Landlord upon the expiration or earlier termination of this Lease. For the avoidance of doubt, the items, if any, listed on Exhibit H attached hereto (which Exhibit H may be updated by Tenant from and after the Term Commencement Date, subject to Landlord's written consent) constitute Tenant's property and shall be removed by Tenant upon the expiration or earlier termination of the Lease.

17.8. Notwithstanding any other provision of this Article to the contrary, in no event shall Tenant remove any improvement from the Premises as to which Landlord contributed payment, including the Tenant Improvements, without Landlord's prior written consent, which consent Landlord may withhold in its sole and absolute discretion. Landlord shall not require Tenant to remove any of the initial Tenant Improvements.

17.9. If Tenant shall fail to remove any of its property from the Premises prior to the expiration or earlier termination of this Lease, then Landlord may, at its option, remove the same in any manner that Landlord shall choose and store such effects without liability to Tenant for loss thereof or damage thereto, and Tenant shall pay Landlord, within thirty (30) days after written demand (accompanied by reasonable supporting documentation thereof), any costs and expenses incurred due to such removal and storage or Landlord may to the extent permitted by Applicable Laws, at its sole option and without notice to Tenant, sell such property or any portion thereof at private sale for such price as Landlord may obtain and apply the proceeds of such sale against any (a) amounts due by Tenant to Landlord under this Lease and (b) any expenses incident to the removal, storage and sale of such personal property.

17.10. Tenant shall pay to Landlord as Additional Rent an amount equal to three percent (3%) of the cost to Tenant of all Alterations to cover Landlord's overhead and expenses for plan review, engineering review, coordination, scheduling and supervision thereof. For purposes of payment of such sum, Tenant shall submit to Landlord copies of all bills, invoices and statements covering the costs of such charges, accompanied by payment to Landlord of the fee set forth in this Section. Tenant shall reimburse Landlord for any extra expenses incurred by Landlord by reason of faulty work done by Tenant or its contractors, or by reason of delays caused by such work, or by reason of inadequate clean-up.

17.11. Within sixty (60) days after final completion of any Alterations performed by Tenant with respect to the Premises, Tenant shall submit to Landlord documentation showing the amounts expended by Tenant with respect to such Alterations, together with supporting

documentation showing payment thereof and waiver of liens by all involved contractors, subcontractors and material suppliers, as reasonably acceptable to Landlord.

17.12. Tenant shall take, and shall cause its contractors to take, commercially reasonable steps to protect the Premises during the performance of any Alterations, including covering or temporarily removing any window coverings so as to guard against dust, debris or damage.

17.13. Tenant shall require its contractors and subcontractors performing work on the Premises to name Landlord and all Landlord Parties (as defined below) as additional insureds on their respective insurance policies.

18. Repairs and Maintenance.

18.1. Landlord shall repair and maintain the structural and exterior portions and Common Area of the Building and the Project, including roofing and covering materials; foundations (excluding any architectural slabs, but including any structural slabs); exterior walls; plumbing; fire sprinkler systems (if any); HVAC systems (excluding the Supplemental HVAC Unit); elevators; and electrical systems installed or furnished by Landlord. In addition, Landlord shall provide (a) janitorial services for the Premises comparable to the janitorial services provided by comparable owners of comparable properties in the Rockville, Maryland, area, which services shall be provided five (5) nights per week, Monday through Friday, excluding holidays recognized as such by the federal government and/or the State of Maryland, and (b) Building standard lightbulb changes to the Premises.

18.2. Except for services of Landlord, if any, required by Section 18.1, Tenant shall at Tenant's sole cost and expense maintain and keep the Premises and every part thereof in good condition and repair, damage thereto from ordinary wear and tear excepted, and shall, within ten (10) days after receipt of written notice from Landlord, provide to Landlord any maintenance records that Landlord reasonably requests. Tenant shall, upon the expiration or sooner termination of the Term, surrender the Premises to Landlord in as good a condition as when received, ordinary wear and tear excepted; and shall, at Landlord's request and Tenant's sole cost and expense, remove all telephone and data systems, wiring and equipment from the Premises, and repair any damage to the Premises caused thereby. Landlord shall have no obligation to alter, remodel, improve, repair, decorate or paint the Premises or any part thereof, other than as described in Exhibit B.

18.3. Landlord shall not be liable for any failure to make any repairs or to perform any maintenance that is Landlord's obligation pursuant to this Lease unless such failure shall persist for an unreasonable time after Tenant provides Landlord with written notice of the need of such repairs or maintenance. Tenant waives its rights under Applicable Laws now or hereafter in effect to make repairs at Landlord's expense.

18.4. If any excavation shall be made upon land adjacent to or under the Building, or shall be authorized to be made, Tenant shall afford to the person causing or authorized to cause such excavation, license to enter the Premises upon reasonable prior notice for the purpose of performing such work as such person shall reasonably deem necessary or desirable to preserve and protect the Building from injury or damage and to support the same by proper foundations,

without any claim for damages or liability against Landlord and without reducing or otherwise affecting Tenant's obligations under this Lease; provided, however, that all such activities shall be conducted only to the extent and for the time reasonably required and in such a manner so as to cause as little interference to Tenant as is reasonably possible.

18.5. This Article relates to repairs and maintenance arising in the ordinary course of operation of the Building and the Project. In the event of a casualty described in Article 24, Article 24 shall apply in lieu of this Article. In the event of eminent domain, Article 25 shall apply in lieu of this Article.

18.6. Costs incurred by Landlord pursuant to this Article shall constitute Operating Expenses to the extent permitted by Article 9.

19. Liens.

19.1. Subject to the immediately succeeding sentence, Tenant shall keep the Premises, the Building and the Project free from any liens arising out of work or services performed, materials furnished to or obligations incurred by Tenant. Tenant further covenants and agrees that any mechanic's or materialman's lien filed against the Premises, the Building or the Project for work or services claimed to have been done for, or materials claimed to have been furnished to, or obligations incurred by Tenant shall be discharged or bonded by Tenant within ten (10) business days after the filing thereof, at Tenant's sole cost and expense.

19.2. Should Tenant fail to discharge or bond against any lien of the nature described in Section 19.1, Landlord may, at Landlord's election, pay such claim or post a statutory lien bond or otherwise provide security to eliminate the lien as a claim against title, and Tenant shall within five (5) days after written demand (accompanied by reasonable supporting documentation) reimburse Landlord for the costs thereof as Additional Rent. Tenant shall indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold the Landlord Indemnitees harmless from and against any Claims arising from any such liens, including any administrative, court or other legal proceedings related to such liens.

19.3. In the event that Tenant leases or finances the acquisition of office equipment, furnishings or other personal property of a removable nature utilized by Tenant in the operation of Tenant's business, Tenant warrants that any Uniform Commercial Code financing statement shall, upon its face or by exhibit thereto, indicate that such financing statement is applicable only to removable personal property of Tenant located within the Premises. In no event shall the address of the Premises, the Building or the Project be furnished on a financing statement without qualifying language as to applicability of the lien only to removable personal property located in an identified suite leased by Tenant. Should any holder of a financing statement record or place of record a financing statement that appears to constitute a lien against any interest of Landlord or against equipment that may be located other than within an identified suite leased by Tenant, Tenant shall, within ten (10) business days after filing such financing statement, cause (a) a copy of the lender security agreement or other documents to which the financing statement pertains to be furnished to Landlord to facilitate Landlord's ability to demonstrate that the lien of such financing statement is not applicable to Landlord's interest and

(b) Tenant's lender to amend such financing statement and any other documents of record to clarify that any liens imposed thereby are not applicable to any interest of Landlord in the Premises, the Building or the Project.

20. Estoppel Certificate. Tenant shall, within ten (10) business days after receipt of written notice from Landlord, execute, acknowledge and deliver a statement in writing substantially in the form attached to this Lease as Exhibit I, or on any other form reasonably requested by a current or proposed Lender or encumbrancer or proposed purchaser, (a) certifying that this Lease is unmodified and in full force and effect (or, if modified, stating the nature of such modification and certifying that this Lease as so modified is in full force and effect) and the dates to which rental and other charges are paid in advance, if any, (b) acknowledging that there are not, to Tenant's knowledge, any uncured defaults on the part of Landlord hereunder, or specifying such defaults if any are claimed, and (c) setting forth such further information with respect to this Lease or the Premises as may be requested thereon. Any such statement may be relied upon by any prospective purchaser or encumbrancer of all or any portion of the Property. If Tenant fails to deliver such statement within the prescribed time set forth in the first grammatical sentence of this Section, then Landlord shall deliver written notice to Tenant of such failure and if Tenant fails to deliver such statement within five (5) days of receiving such written notice, Tenant's failure to deliver such statement shall, at Landlord's option, constitute a Default (as defined below) under this Lease.

21. Hazardous Materials.

21.1. Tenant shall not cause or permit any Hazardous Materials (as defined below) to be brought upon, kept or used in or about the Premises, the Building or the Project by Tenant or any of its employees, agents, contractors or invitees (collectively with Tenant, each a "Tenant Party"), except for small amounts of Hazardous Materials included in typical office and cleaning supplies and equipment (provided that the same are stored, used and disposed of in accordance with Applicable Laws). If (a) Tenant breaches such obligation, (b) the presence of Hazardous Materials as a result of such a breach results in contamination of the Project, any portion thereof, or any adjacent property, (c) contamination of the Premises otherwise occurs during the Term or any extension or renewal hereof or holding over hereunder (other than if such contamination results from (i) migration of Hazardous Materials from outside the Premises not caused by a Tenant Party or (ii) to the extent such contamination is caused by Landlord's gross negligence or willful misconduct) or (d) contamination of the Project occurs as a result of Hazardous Materials that are placed on or under or are released into the Project by a Tenant Party, then Tenant shall indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold the Landlord Indemnitees harmless from and against any and all Claims of any kind or nature, including (w) diminution in value of the Project or any portion thereof, (x) damages for the loss or restriction on use of rentable or usable space or of any amenity of the Project, (y) damages arising from any adverse impact on marketing of space in the Project or any portion thereof and (z) sums paid in settlement of Claims that arise before, during or after the Term as a result of such breach or contamination. This indemnification by Tenant includes costs incurred in connection with any investigation of site conditions or any clean-up, remedial, removal or restoration work required by any Governmental Authority because of Hazardous Materials present in the air, soil or groundwater above, on, under or about the Project. Without limiting the foregoing, if the presence of any Hazardous Materials in, on, under or about the

Project, any portion thereof or any adjacent property caused or permitted by any Tenant Party results in any contamination of the Project, any portion thereof or any adjacent property, then Tenant shall promptly take all actions at its sole cost and expense as are necessary to return the Project, any portion thereof or any adjacent property to its respective condition existing prior to the time of such contamination; provided that Landlord's written approval of such action shall first be obtained, which approval Landlord shall not unreasonably withhold, condition or delay; and provided, further, that it shall be reasonable for Landlord to withhold its consent if such actions could have a material adverse long-term or short-term effect on the Project, any portion thereof or any adjacent property. Tenant's obligations under this Section shall not be affected, reduced or limited by any limitation on the amount or type of damages, compensation or benefits payable by or for Tenant under workers' compensation acts, disability benefit acts, employee benefit acts or similar legislation.

21.2. If (a) on the Execution Date, the Premises contains Hazardous Materials in violation of Applicable Laws, or (b) after the Execution Date, Landlord causes the Premises to contain Hazardous Materials in violation of Applicable Laws, then Landlord shall cause such Hazardous Materials to be removed and/or abated in accordance with all Applicable Laws and in a manner which minimizes disruption of Tenant's access to and use and occupancy of the Premises, at no cost to Tenant (either directly or as an Operating Expense).

21.3. [Intentionally omitted]

21.4. At any time, and from time to time, prior to the expiration of the Term, Landlord shall, subject to the provisions of Section 14.4 if entry into the Premises is required, have the right to conduct appropriate tests of the Project or any portion thereof to demonstrate that Hazardous Materials are present or that contamination has occurred due to the acts or omissions of a Tenant Party. Tenant shall pay all reasonable costs of such tests if such tests reveal that Hazardous Materials exist at the Project in violation of Section 21.1 of this Lease.

21.5. [Intentionally omitted]

21.6. Tenant shall promptly report to Landlord any actual or suspected presence of mold or water intrusion at the Premises.

21.7. Landlord's and Tenant's obligations under this Article shall survive the expiration or earlier termination of the Lease. During any period of time needed by Tenant or Landlord after the termination of this Lease to complete the removal from the Premises of any such Hazardous Materials resulting from a violation of Tenant's obligations under Section 21.1 of this Lease, Tenant shall be deemed a holdover tenant and subject to the provisions of Article 27.

21.8. As used herein, the term "Hazardous Material" means any toxic, explosive, corrosive, flammable, infectious, radioactive, carcinogenic, mutagenic or otherwise hazardous substance, material or waste that is or becomes regulated by Applicable Laws or any Governmental Authority.

22. Odors and Exhaust. Tenant acknowledges that Landlord would not enter into this Lease with Tenant unless Tenant assured Landlord that under no circumstances will any other

occupants of the Building or the Project (including persons legally present in any outdoor areas of the Project) be subjected to odors or fumes (whether or not noxious), and that the Building and the Project will not be damaged by any exhaust, in each case from Tenant's operations. Landlord and Tenant therefore agree as follows:

22.1. Tenant shall not cause or permit (or conduct any activities that would cause) any release of any odors or fumes of any kind from the Premises.

22.2. If the Building has a ventilation system that, in Landlord's judgment, is adequate, suitable, and appropriate to vent the Premises in a manner that does not release odors affecting any indoor or outdoor part of the Project, Tenant shall vent the Premises through such system. If Landlord at any time determines that any existing ventilation system is inadequate, or if no ventilation system exists, Tenant shall in compliance with Applicable Laws vent all fumes and odors from the Premises (and remove odors from Tenant's exhaust stream) as Landlord requires. The placement and configuration of all ventilation exhaust pipes, louvers and other equipment shall be subject to Landlord's approval. Tenant acknowledges Landlord's legitimate desire to maintain the Project (indoor and outdoor areas) in an odor-free manner, and Landlord may require Tenant to abate and remove all odors in a manner that goes beyond the requirements of Applicable Laws.

22.3. Tenant shall, at Tenant's sole cost and expense, provide odor eliminators and other devices (such as filters, air cleaners, scrubbers and whatever other equipment may in Landlord's judgment be necessary or appropriate from time to time) to completely remove, eliminate and abate any odors, fumes or other substances in Tenant's exhaust stream that, in Landlord's judgment, emanate from Tenant's Premises. Any work Tenant performs under this Section shall constitute Alterations.

22.4. Tenant's responsibility to remove, eliminate and abate odors, fumes and exhaust shall continue throughout the Term. Landlord's construction of the Tenant Improvements shall not preclude Landlord from requiring additional measures to eliminate odors, fumes and other adverse impacts of Tenant's exhaust stream (as Landlord may designate in Landlord's discretion). Tenant shall install additional equipment as Landlord requires from time to time under the preceding sentence. Such installations shall constitute Alterations.

22.5. If Tenant fails to install satisfactory odor control equipment within ten (10) business days after Landlord's demand made at any time, then Landlord may, without limiting Landlord's other rights and remedies, require Tenant to cease and suspend any operations in the Premises that, in Landlord's reasonable determination, cause odors, fumes or exhaust. For example, if Landlord reasonably determines that Tenant's production of a certain type of product causes odors, fumes or exhaust, and Tenant does not install satisfactory odor control equipment within ten (10) business days after Landlord's request, then Landlord may require Tenant to stop producing such type of product in the Premises unless and until Tenant has installed odor control equipment satisfactory to Landlord.

23. Insurance; Waiver of Subrogation.

23.1. Landlord shall maintain insurance for the Building and the Project in amounts equal to full replacement cost (exclusive of the costs of excavation, foundations and footings, engineering costs or such other costs to the extent the same are not incurred in the event of a rebuild and without reference to depreciation taken by Landlord upon its books or tax returns) or such lesser coverage as Landlord may elect, provided that such coverage shall not be less than the amount of such insurance Landlord's Lender, if any, requires Landlord to maintain, providing protection against any peril generally included within the classification "Fire and Extended Coverage," together with insurance against sprinkler damage (if applicable), vandalism and malicious mischief. Landlord, subject to availability thereof, shall further insure, if Landlord deems it appropriate, coverage against flood, environmental hazard, earthquake, loss or failure of building equipment, rental loss during the period of repairs or rebuilding, Workers' Compensation insurance and fidelity bonds for employees employed to perform services. Notwithstanding the foregoing, Landlord may, but shall not be deemed required to, provide insurance for any improvements installed by Tenant or that are in addition to the standard improvements customarily furnished by Landlord, without regard to whether or not such are made a part of or are affixed to the Building.

23.2. In addition, Landlord shall carry Commercial General Liability insurance with limits of not less than One Million Dollars ($1,000,000) per occurrence/general aggregate for bodily injury (including death), or property damage with respect to the Project.

23.3. Tenant shall, at its own cost and expense, procure and maintain during the Term the following insurance for the benefit of Tenant and Landlord (as their interests may appear) with insurers financially acceptable and lawfully authorized to do business in the state where the Premises are located:

(a) Commercial General Liability insurance on a broad-based occurrence coverage form, with coverages including but not limited to bodily injury (including death), property damage (including loss of use resulting therefrom), premises/operations, personal & advertising injury, and contractual liability with limits of liability of not less than $2,000,000 for bodily injury and property damage per occurrence, $2,000,000 general aggregate, which limits may be met by use of excess and/or umbrella liability insurance provided that such coverage is at least as broad as the primary coverages required herein.

(b) Commercial Automobile Liability insurance covering liability arising from the use or operation of any auto, including those owned, hired or otherwise operated or used by or on behalf of the Tenant. The coverage shall be on a broad-based occurrence form with combined single limits of not less than $1,000,000 per accident for bodily injury and property damage.

(c) Commercial Property insurance covering property damage to the full replacement cost value and business interruption. Covered property shall include all tenant improvements in the Premises (to the extent not insured by Landlord pursuant to Section 23.1) and Tenant's Property including personal property, furniture, fixtures, machinery, equipment, stock, inventory and improvements and betterments, which may be owned by Tenant or Landlord and required to be insured hereunder, or which may be leased, rented, borrowed or in

the care custody or control of Tenant, or Tenant's agents, employees or subcontractors. Such insurance, with respect only to all Tenant Improvements, Alterations or other work performed on the Premises by Tenant (collectively, "Tenant Work"), shall name Landlord and Landlord's current and future mortgagees as loss payees as their interests may appear. Such insurance shall be written on an "all risk" of physical loss or damage basis including the perils of fire, extended coverage, electrical injury, mechanical breakdown, windstorm, vandalism, malicious mischief, sprinkler leakage, back-up of sewers or drains, flood, earthquake, terrorism and such other risks Landlord may from time to time designate, for the full replacement cost value of the covered items with an agreed amount endorsement with no co-insurance. Business interruption coverage shall have limits sufficient to cover Tenant's lost profits and necessary continuing expenses, including rents due Landlord under the Lease. The minimum period of indemnity for business interruption coverage shall be twelve (12) months plus twelve (12) months' extended period of indemnity.

(d) Workers' Compensation insurance as is required by statute or law, or as may be available on a voluntary basis and Employers' Liability insurance with limits of not less than the following: each accident, Five Hundred Thousand Dollars ($500,000); disease ($500,000); disease (each employee), Five Hundred Thousand Dollars ($500,000).

(e) [Intentionally omitted]

(f) [Intentionally omitted]

(g) During all construction by Tenant at the Premises, with respect to tenant improvements being constructed (including any Alterations), insurance required in Exhibit B-l must be in place.

The insurance required of Tenant by this Article shall be with companies at all times having a current rating of not less than A- and financial category rating of at least Class VII in "A.M. Best's Insurance Guide" current edition. Tenant shall obtain for Landlord from the insurance companies/broker or cause the insurance companies/broker to furnish certificates of insurance evidencing all coverages required herein to Landlord. No such policy shall be cancelable or subject to reduction of coverage or other modification or cancellation except after twenty (20) days' prior written notice to Landlord from Tenant or its insurers (except in the event of non-payment of premium, in which case ten (10) days' written notice shall be given). All such policies shall be written as primary policies, not contributing with and not in excess of the coverage that Landlord may carry. Tenant's required policies shall contain severability of interests clauses stating that, except with respect to limits of insurance, coverage shall apply separately to each insured or additional insured. Tenant shall, prior to the expiration of such policies, furnish Landlord with renewal certificates of insurance or binders. Tenant agrees that if Tenant does not take out and maintain such insurance, Landlord may (but shall not be required to), following written notice to Tenant, procure such insurance on Tenant's behalf and at its cost to be paid by Tenant as Additional Rent. Commercial General Liability, Commercial Automobile Liability, Umbrella Liability and Pollution Legal Liability insurance as required above shall name Landlord, BioMed Realty, L.P., and BioMed Realty Trust, Inc., and their respective officers, employees, agents, general partners, members, subsidiaries, affiliates and Lenders ("Landlord Parties") as additional insureds as respects liability arising from work or

operations performed by or on behalf of Tenant, Tenant's use or occupancy of Premises, and ownership, maintenance or use of vehicles by or on behalf of Tenant.

23.4. In each instance where insurance is to name Landlord Parties as additional insureds, Tenant shall, upon Landlord's written request, also designate and furnish certificates evidencing such Landlord Parties as additional insureds to (a) any Lender of Landlord holding a security interest in the Building or the Project, (b) the landlord under any lease whereunder Landlord is a tenant of the real property upon which the Building is located if the interest of Landlord is or shall become that of a tenant under a ground lease rather than that of a fee owner and (c) any management company retained by Landlord to manage the Project.

23.5. Tenant assumes the risk of damage to any fixtures, goods, inventory, merchandise, equipment and leasehold improvements, and Landlord shall not be liable for injury to Tenant's business or any loss of income therefrom, relative to such damage, all as more particularly set forth within this Lease. Tenant shall, at Tenant's sole cost and expense, carry such insurance as Tenant desires for Tenant's protection with respect to personal property of Tenant or business interruption.

23.6. Tenant and its insurers hereby waive any and all rights of recovery or subrogation against the Landlord Parties with respect to any loss, damage, claims, suits or demands, howsoever caused, that are covered, or should have been covered, by valid and collectible insurance, including any deductibles or self-insurance maintained thereunder. If necessary, Tenant agrees to endorse the required insurance policies to permit waivers of subrogation as required hereunder and hold harmless and indemnify the Landlord Parties for any loss or expense incurred as a result of a failure to obtain such waivers of subrogation from insurers. Such waivers shall continue so long as Tenant's insurers so permit. Any termination of such a waiver shall be by written notice to Landlord, containing a description of the circumstances hereinafter set forth in this Section. Tenant, upon obtaining the policies of insurance required or permitted under this Lease, shall give notice to its insurance carriers that the foregoing waiver of subrogation is contained in this Lease. If such policies shall not be obtainable with such waiver or shall be so obtainable only at a premium over that chargeable without such waiver, then Tenant shall notify Landlord of such conditions. Landlord and its insurers hereby waive any and all rights of recovery or subrogation against Tenant with respect to any loss, damage, claims, suits or demands, howsoever caused, that are covered, or should have been covered, by valid and collectible insurance, including any deductibles or self-insurance maintained thereunder. If necessary, Landlord agrees to endorse the required insurance policies to permit waivers of subrogation as required hereunder and hold harmless and indemnify Tenant for any loss or expense incurred as a result of a failure to obtain such waivers of subrogation from insurers. Such waivers shall continue so long as Landlord's insurers so permit. Any termination of such a waiver shall be by written notice to Tenant, containing a description of the circumstances hereinafter set forth in this Section. Landlord, upon obtaining the policies of insurance required or permitted under this Lease, shall give notice to its insurance carriers that the foregoing waiver of subrogation is contained in this Lease. If such policies shall not be obtainable with such waiver or shall be so obtainable only at a premium over that chargeable without such waiver, then Landlord shall notify Tenant of such conditions.

23.7. Landlord may require insurance policy limits required under this Lease to be raised to conform with requirements of Landlord's Lender or to bring coverage limits to levels then being required of comparable new tenants within the Project.

23.8. Any costs incurred by Landlord pursuant to this Article shall constitute a portion of Operating Expenses to the extent permitted under Article 9.

23.9. The provisions of this Section shall survive the expiration or earlier termination of this Lease.

24. Damage or Destruction.

24.1. In the event of a partial destruction of (a) the Premises or (b) Common Area of the Building or the Project ((a) and (b) together, the "Affected Areas") by fire or other perils covered by extended coverage insurance not exceeding twenty-five percent (25%) of the full insurable value thereof, and provided that (x) the damage thereto is such that the Affected Areas may be repaired, reconstructed or restored within a period of six (6) months from the date of the happening of such casualty, (y) Landlord shall receive insurance proceeds sufficient to cover the cost of such repairs, reconstruction and restoration (except for any deductible amount provided by Landlord's policy, which deductible amount, if paid by Landlord, shall constitute an Operating Expense) and (z) such casualty was not intentionally caused by a Tenant Party, then Landlord shall commence and proceed diligently with the work of repair, reconstruction and restoration of the Affected Areas and this Lease shall continue in full force and effect.

24.2. In the event of any damage to or destruction of the Building or the Project other than as described in Section 24.1, Landlord may elect to repair, reconstruct and restore the Building or the Project, as applicable, in which case this Lease shall continue in full force and effect. If Landlord elects not to repair, reconstruct and restore the Building or the Project, as applicable, then this Lease shall terminate as of the date of such damage or destruction. In the event of any damage or destruction (regardless of whether such damage is governed by Section 24.1 or this Section), if (a) in Landlord's determination as set forth in the Damage Repair Estimate (as defined below), the Affected Areas cannot be repaired, reconstructed or restored within twelve (12) months after the date of the Damage Repair Estimate, (b) subject to Section 24.6, the Affected Areas are not actually repaired, reconstructed and restored within eighteen (18) months after the date of the Damage Repair Estimate, or (c) the damage and destruction occurs within the last twelve (12) months of the then-current Term, then Tenant shall have the right to terminate this Lease, effective as of the date of such damage or destruction, by delivering to Landlord its written notice of termination (a "Termination Notice") (y) with respect to Subsections 24.2(a) and (c), no later than fifteen (15) days after Landlord delivers to Tenant Landlord's Damage Repair Estimate and (z) with respect to Subsection 24.2(b), no later than fifteen (15) days after such eighteen (18) month period (as the same may be extended pursuant to Section 24.6) expires. If Tenant provides Landlord with a Termination Notice pursuant to Subsection 24.2(z), Landlord shall have an additional thirty (30) days after receipt of such Termination Notice to complete the repair, reconstruction and restoration. If Landlord does not complete such repair, reconstruction and restoration within such thirty (30) day period, then Tenant may terminate this Lease by giving Landlord written notice within two (2) business days after the expiration of such thirty (30) day period. If Landlord does complete such repair,

reconstruction and restoration within such thirty (30) day period, then this Lease shall continue in full force and effect.

24.3. As soon as reasonably practicable, but in any event within sixty (60) days following the date of damage or destruction, Landlord shall notify Tenant of Landlord's good faith estimate of the period of time in which the repairs, reconstruction and restoration will be completed (the "Damage Repair Estimate"), which estimate shall be based upon the opinion of a contractor reasonably selected by Landlord and experienced in comparable repair, reconstruction and restoration of similar buildings. Additionally, Landlord shall give written notice to Tenant within sixty (60) days following the date of damage or destruction of its election not to repair, reconstruct or restore the Building or the Project, as applicable.

24.4. Upon any termination of this Lease under any of the provisions of this Article, the parties shall be released thereby without further obligation to the other from the date possession of the Premises is surrendered to Landlord, except with regard to (a) items occurring prior to the damage or destruction and (b) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof.

24.5. In the event of repair, reconstruction and restoration as provided in this Article, all Rent to be paid by Tenant under this Lease shall be abated proportionately based on the extent to which Tenant's use of the Premises is impaired during the period of such repair, reconstruction or restoration, unless Landlord provides Tenant with other space during the period of repair, reconstruction and restoration that, in Tenant's reasonable opinion, is suitable for the temporary conduct of Tenant's business; provided, however, that the amount of such abatement shall be reduced by the proceeds of business interruption or loss of rental income insurance actually received by Tenant with respect to the Premises.

24.6. Notwithstanding anything to the contrary contained in this Article, should Landlord be delayed or prevented from completing the repair, reconstruction or restoration of the damage or destruction to the Premises after the occurrence of such damage or destruction by Force Majeure or delays caused by a Tenant Party, then the time for Landlord to commence or complete repairs, reconstruction and restoration shall be extended on a day-for-day basis; provided, however, that, at Landlord's election, Landlord shall be relieved of its obligation to make such repairs, reconstruction and restoration.

24.7. If Landlord is obligated to or elects to repair, reconstruct or restore as herein provided, then Landlord shall be obligated to make such repairs, reconstruction or restoration only with regard to (a) those portions of the Premises that were originally provided at Landlord's expense and (b) the Common Area portion of the Affected Areas. The repairs, reconstruction or restoration of improvements not originally provided by Landlord or at Landlord's expense shall be the obligation of Tenant. In the event Tenant has elected to upgrade certain improvements from the Building Standard, Landlord shall, upon the need for replacement due to an insured loss, provide only the Building Standard, unless Tenant again elects to upgrade such improvements and pay any incremental costs related thereto, except to the extent that excess insurance proceeds, if received, are adequate to provide such upgrades, in addition to providing for basic repairs, reconstruction and restoration of the Premises, the Building and the Project.

24.8. Notwithstanding anything to the contrary contained in this Article, Landlord shall not have any obligation whatsoever to repair, reconstruct or restore the Premises if the damage resulting from any casualty covered under this Article occurs during the last twenty-four (24) months of the Term or any extension thereof, or to the extent that insurance proceeds are not available therefor.

24.9. Landlord's obligation, should it elect or be obligated to repair, reconstruct or restore, shall be limited to the Affected Areas. Tenant shall, at its expense, replace or fully repair all of Tenant's personal property and any Alterations installed by Tenant existing at the time of such damage or destruction. If Affected Areas are to be repaired, reconstructed or restored in accordance with the foregoing, Landlord shall make available to Tenant any portion of insurance proceeds it receives that are allocable to the Alterations constructed by Tenant pursuant to this Lease; provided Tenant is not then in default under this Lease, and subject to the requirements of any Lender of Landlord.

24.10. This Article sets forth the terms and conditions upon which this Lease may terminate in the event of any damage or destruction. Accordingly, the parties hereby waive the provisions of any Applicable Laws (and any successor statutes) permitting the parties to terminate this Lease as a result of any damage or destruction.

25. Eminent Domain.

25.1. In the event (a) the whole of all Affected Areas or (b) such part thereof as shall substantially interfere with Tenant's use and occupancy of the Premises for the Permitted Use shall be taken for any public or quasi-public purpose by any lawful power or authority by exercise of the right of appropriation, condemnation or eminent domain, or sold to prevent such taking, Tenant or Landlord may terminate this Lease effective as of the date possession is required to be surrendered to such authority, except with regard to (y) items occurring prior to the taking and (z) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof.

25.2. In the event of a partial taking of (a) the Building or the Project or (b) drives, walkways or parking areas serving the Building or the Project for any public or quasi-public purpose by any lawful power or authority by exercise of right of appropriation, condemnation, or eminent domain, or sold to prevent such taking, then, without regard to whether any portion of the Premises occupied by Tenant was so taken, Landlord may elect to terminate this Lease (except with regard to (y) items occurring prior to the taking and (z) provisions of this Lease that, by their express terms, survive the expiration or earlier termination hereof) as of such taking if such taking is, in Landlord's sole opinion, of a material nature such as to make it uneconomical to continue use of the unappropriated portion for purposes of renting office or laboratory space.

25.3. Tenant shall be entitled to any award that is specifically awarded as compensation for (a) the taking of Tenant's personal property that was installed at Tenant's expense and (b) the costs of Tenant moving to a new location. Except as set forth in the previous sentence, any award for such taking shall be the property of Landlord.

25.4. If, upon any taking of the nature described in this Article, this Lease continues in effect, then Landlord shall promptly proceed to restore the Affected Areas to substantially their same condition prior to such partial taking. To the extent such restoration is infeasible, as determined by Landlord in its sole and absolute discretion, the Rent shall be decreased proportionately to reflect the loss of any portion of the Premises no longer available to Tenant.

25.5. This Article sets forth the terms and conditions upon which this Lease may terminate in the event of any damage or destruction. Accordingly, the parties hereby waive the provisions of any Applicable Laws (and any successor statutes) permitting the parties to terminate this Lease as a result of any damage or destruction.

26. Surrender.

26.1. At least ten (10) days prior to Tenant's surrender of possession of any part of the Premises, Tenant shall conduct a site inspection with Landlord.

26.2. No surrender of possession of any part of the Premises shall release Tenant from any of its obligations hereunder, unless such surrender is accepted in writing by Landlord.

26.3. The voluntary or other surrender of this Lease by Tenant shall not effect a merger with Landlord's fee title or leasehold interest in the Premises, the Building, the Property or the Project, unless Landlord consents in writing, and shall, at Landlord's option, operate as an assignment to Landlord of any or all subleases.

26.4. The voluntary or other surrender of any ground or other underlying lease that now exists or may hereafter be executed affecting the Building or the Project, or a mutual cancellation thereof or of Landlord's interest therein by Landlord and its lessor shall not effect a merger with Landlord's fee title or leasehold interest in the Premises, the Building or the Property and shall, at the option of the successor to Landlord's interest in the Building or the Project, as applicable, operate as an assignment of this Lease.

27. Holding Over.

27.1. If, with Landlord's prior written consent, Tenant holds possession of all or any part of the Premises after the Term, Tenant shall become a tenant from month to month after the expiration or earlier termination of the Term, and in such case Tenant shall continue to pay (a) Base Rent in accordance with Article 7 and (b) any amounts for which Tenant would otherwise be liable under this Lease if the Lease were still in effect, including payments for Additional Rent. Any such month-to-month tenancy shall be subject to every other term, covenant and agreement contained herein.

27.2. Notwithstanding the foregoing, if Tenant remains in possession of the Premises after the expiration or earlier termination of the Term without Landlord's prior written consent, (a) Tenant shall become a tenant at sufferance subject to the terms and conditions of this Lease, except that the monthly rent shall be equal to one hundred fifty percent (150%) of the Rent in effect during the last thirty (30) days of the Term, and (b) Tenant shall be liable to Landlord for any and all damages suffered by Landlord as a result of such holdover, including any lost rent or

consequential, special and indirect damages (in each case, regardless of whether such damages are foreseeable).

27.3. Acceptance by Landlord of Rent after the expiration or earlier termination of the Term shall not result in an extension, renewal or reinstatement of this Lease.

27.4. The foregoing provisions of this Article are in addition to and do not affect Landlord's right of reentry or any other rights of Landlord hereunder or as otherwise provided by Applicable Laws.

27.5. The provisions of this Article shall survive the expiration or earlier termination of this Lease.

28. Indemnification and Exculpation.

28.1. Tenant agrees to indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold the Landlord Indemnitees harmless from and against any and all Claims of any kind or nature, real or alleged, arising from injury to or death of any person or damage to any property occurring within or about the Premises, the Building, the Property or the Project, arising directly or indirectly out of (a) the presence at or use or occupancy of the Premises or Project by a Tenant Party, (b) an act or omission on the part of any Tenant Party, (c) a breach or default by Tenant in the performance of any of its obligations hereunder or (d) injury to or death of persons or damage to or loss of any property, real or alleged, arising from the serving of alcoholic beverages at the Premises or Project, including liability under any dram shop law, host liquor law or similar Applicable Law, except to the extent directly caused by Landlord's negligence or willful misconduct. Tenant's obligations under this Section shall not be affected, reduced or limited by any limitation on the amount or type of damages, compensation or benefits payable by or for Tenant under workers' compensation acts, disability benefit acts, employee benefit acts or similar legislation. Tenant's obligations under this Section shall survive the expiration or earlier termination of this Lease. Subject to Sections 23.6, 28.2 and 31.12, Landlord agrees to indemnify, save, defend (at Tenant's option and with counsel reasonably acceptable to Tenant) and hold the Tenant Parties harmless from and against any and all Claims arising from injury to or death of any person or damage to or loss of any physical property occurring within or about the Premises, the Building, the Property or the Project to the extent directly arising out of Landlord's gross negligence or willful misconduct.

28.2. Notwithstanding anything in this Lease to the contrary, Landlord shall not be liable to Tenant for and Tenant assumes all risk of (a) damage or losses caused by fire, electrical malfunction, gas explosion or water damage of any type (including broken water lines, malfunctioning fire sprinkler systems, roof leaks or stoppages of lines), unless any such loss is due to Landlord's willful disregard of written notice by Tenant of need for a repair that Landlord is responsible to make for an unreasonable period of time, and (b) damage to personal property or scientific research, including loss of records kept by Tenant within the Premises (in each case, regardless of whether such damages are foreseeable). Tenant further waives any claim for injury to Tenant's business or loss of income relating to any such damage or destruction of personal property as described in this Section. Notwithstanding anything in the foregoing or this Lease to

the contrary, except (x) as otherwise provided herein (including Section 27.2), (y) as may be provided by Applicable Laws or (z) in the event of Tenant's breach of Article 21 or Section 26.1, in no event shall Landlord or Tenant be liable to the other for any consequential, special or indirect damages arising out of this Lease, including lost profits (provided that this Subsection 28.2(z) shall not limit Tenant's liability for Base Rent or Additional Rent pursuant to this Lease).

28.3. Landlord shall not be liable for any damages arising from any act, omission or neglect of any other tenant in the Building or the Project, or of any other third party.

28.4. Tenant acknowledges that security devices and services, if any, while intended to deter crime, may not in given instances prevent theft or other criminal acts. Landlord shall not be liable for injuries or losses caused by criminal acts of third parties, and Tenant assumes the risk that any security device or service may malfunction or otherwise be circumvented by a criminal. If Tenant desires protection against such criminal acts, then Tenant shall, at Tenant's sole cost and expense, obtain appropriate insurance coverage. Tenant's security programs and equipment for the Premises shall be coordinated with Landlord and subject to Landlord's reasonable approval.

28.5. The provisions of this Article shall survive the expiration or earlier termination of this Lease.

29. Assignment or Subletting.

29.1. Except as hereinafter expressly permitted, none of the following (each, a "Transfer"), either voluntarily or by operation of Applicable Laws, shall be directly or indirectly performed without Landlord's prior written consent: (a) Tenant selling, hypothecating, assigning, pledging, encumbering or otherwise transferring this Lease or subletting the Premises or (b) a controlling interest in Tenant being sold, assigned or otherwise transferred (other than as a result of shares in Tenant being sold on a public stock exchange). For purposes of the preceding sentence, "control" means (a) owning (directly or indirectly) more than fifty percent (50%) of the stock or other equity interests of another person or (b) possessing, directly or indirectly, the power to direct or cause the direction of the management and policies of such person. Notwithstanding the foregoing, Tenant shall have the right to Transfer, without Landlord's prior written consent, Tenant's interest in this Lease or the Premises or any part thereof to any person that (i) as of the date of determination and at all times thereafter directly, or indirectly through one or more intermediaries, controls, is controlled by or is under common control with Tenant, (ii) is the successor entity to Tenant by merger or consolidation or (iii) acquires all or substantially all of Tenant's ownership interests or assets (any such entity, a "Tenant's Affiliate"); provided that Tenant shall notify Landlord in writing at least sixty (60) days prior to the effectiveness of such Transfer to Tenant's Affiliate (an "Exempt Transfer") and otherwise comply with the requirements of this Lease regarding such Transfer; and provided, further, that the person that will be the tenant under this Lease after the Exempt Transfer has a net worth and creditworthiness (as of both the day immediately prior to and the day immediately after the Exempt Transfer) that is equal to or greater than the net worth and creditworthiness (as of both the Execution Date and the date of the Exempt Transfer) of the transferring Tenant. For purposes of Exempt Transfers, "control" requires both (a) owning (directly or indirectly) more than fifty percent (50%) of the stock or other equity interests of another person and (b)

possessing, directly or indirectly, the power to direct or cause the direction of the management and policies of such person. In no event shall Tenant perform a Transfer to or with an entity that is a tenant at the Project or that is in discussions or negotiations with Landlord or an affiliate of Landlord to lease premises at the Project or a property owned by Landlord or an affiliate of Landlord.

29.2. In the event Tenant desires to effect a Transfer, then, at least sixty (60) but not more than ninety (90) days prior to the date when Tenant desires the Transfer to be effective (the "Transfer Date"), Tenant shall provide written notice to Landlord (the "Transfer Notice") containing information concerning the character of the proposed transferee, assignee or sublessee; the Transfer Date; current balance sheet for the Tenant and the proposed transferee, assignee or sublessee; the most recent two (2) years of unconsolidated financial statements of Tenant and of the proposed transferee, assignee or sublessee satisfying the requirements of Section 40.2 ("Required Financials"); any ownership or commercial relationship between Tenant and the proposed transferee, assignee or sublessee; and the consideration and all other material terms and conditions of the proposed Transfer, all in such detail as Landlord shall reasonably require.

29.3. Landlord, in determining whether consent should be given to a proposed Transfer (other than an Exempt Transfer), may give consideration to (a) the financial strength of Tenant and of such transferee, assignee or sublessee (notwithstanding Tenant remaining liable for Tenant's performance), (b) any change in use that such transferee, assignee or sublessee proposes to make in the use of the Premises and (c) Landlord's desire to exercise its rights under Section 29.7 to cancel this Lease. In no event shall Landlord be deemed to be unreasonable for declining to consent to a Transfer to a transferee, assignee or sublessee of poor reputation, lacking financial qualifications or seeking a change in the Permitted Use, or jeopardizing directly or indirectly the status of Landlord or any of Landlord's affiliates as a Real Estate Investment Trust under the Internal Revenue Code of 1986 (as the same may be amended from time to time, the "Revenue Code"). Notwithstanding anything contained in this Lease to the contrary, (w) no Transfer shall be consummated on any basis such that the rental or other amounts to be paid by the occupant, assignee, manager or other transferee thereunder would be based, in whole or in part, on the income or profits derived by the business activities of such occupant, assignee, manager or other transferee; (x) Tenant shall not furnish or render any services to an occupant, assignee, manager or other transferee with respect to whom transfer consideration is required to be paid, or manage or operate the Premises or any capital additions so transferred, with respect to which transfer consideration is being paid; (y) Tenant shall not consummate a Transfer with any person in which Landlord owns an interest, directly or indirectly (by applying constructive ownership rules set forth in Section 856(d)(5) of the Revenue Code); and (z) Tenant shall not consummate a Transfer with any person or in any manner that could cause any portion of the amounts received by Landlord pursuant to this Lease or any sublease, license or other arrangement for the right to use, occupy or possess any portion of the Premises to fail to qualify as "rents from real property" within the meaning of Section 856(d) of the Revenue Code, or any similar or successor provision thereto or which could cause any other income of Landlord to fail to qualify as income described in Section 856(c)(2) of the Revenue Code.

29.4. The following are conditions precedent to a Transfer or to Landlord considering a request by Tenant to a Transfer:

(a) Tenant shall remain fully liable under this Lease. Tenant agrees that it shall not be (and shall not be deemed to be) a guarantor or surety of this Lease, however, and waives its right to claim that is it is a guarantor or surety or to raise in any legal proceeding any guarantor or surety defenses permitted by this Lease or by Applicable Laws;

(b) If Tenant or the proposed transferee, assignee or sublessee does not or cannot deliver the Required Financials, then Landlord may elect to have either Tenant's ultimate parent company or the proposed transferee's, assignee's or sublessee's ultimate parent company provide a guaranty of the applicable entity's obligations under this Lease, in a form acceptable to Landlord, which guaranty shall be executed and delivered to Landlord by the applicable guarantor prior to the Transfer Date;

(c) In the case of an Exempt Transfer, Tenant shall provide Landlord with evidence reasonably satisfactory to Landlord that the Transfer qualifies as an Exempt Transfer;

(d) Tenant shall provide Landlord with evidence reasonably satisfactory to Landlord that the value of Landlord's interest under this Lease shall not be diminished or reduced by the proposed Transfer. Such evidence shall include evidence respecting the relevant business experience and financial responsibility and status of the proposed transferee, assignee or sublessee;

(e) Tenant shall reimburse Landlord for Landlord's actual costs and expenses, including reasonable attorneys' fees, charges and disbursements incurred in connection with the review, processing and documentation of such request;

(f) If Tenant's transfer of rights or sharing of the Premises provides for the receipt by, on behalf of or on account of Tenant of any consideration of any kind whatsoever (including a premium rental for a sublease or lump sum payment for an assignment, but excluding Tenant's reasonable costs in marketing and subleasing the Premises) in excess of the rental and other charges due to Landlord under this Lease, Tenant shall pay fifty percent (50%) of all of such excess to Landlord, after making deductions for any reasonable marketing expenses, tenant improvement funds expended by Tenant, alterations, cash concessions, brokerage commissions, attorneys' fees and free rent actually paid by Tenant. If such consideration consists of cash paid to Tenant, payment to Landlord shall be made upon receipt by Tenant of such cash payment;

(g) The proposed transferee, assignee or sublessee shall agree that, in the event Landlord gives such proposed transferee, assignee or sublessee notice that Tenant is in default under this Lease, such proposed transferee, assignee or sublessee shall thereafter make all payments otherwise due Tenant directly to Landlord, which payments shall be received by Landlord without any liability being incurred by Landlord, except to credit such payment against those due by Tenant under this Lease, and any such proposed transferee, assignee or sublessee shall agree to attorn to Landlord or its successors and assigns should this Lease be terminated for

any reason; provided, however, that in no event shall Landlord or its Lenders, successors or assigns be obligated to accept such attornment;

(h) Landlord's consent to any such Transfer shall be effected on Landlord's forms;

(i) Tenant shall not then be in Default hereunder in any respect;

(j) Such proposed transferee, assignee or sublessee's use of the Premises shall be the same as the Permitted Use;

(k) Landlord shall not be bound by any provision of any agreement pertaining to the Transfer, except for Landlord's written consent to the same;

(l) Tenant shall pay all transfer and other taxes (including interest and penalties) assessed or payable for any Transfer;

(m) Landlord's consent (or waiver of its rights) for any Transfer shall not waive Landlord's right to consent or refuse consent to any later Transfer; and

(n) Tenant shall deliver to Landlord one executed copy of any and all written instruments evidencing or relating to the Transfer.

29.5. Any Transfer that is not in compliance with the provisions of this Article or with respect to which Tenant does not fulfill its obligations pursuant to this Article shall be void and shall, at the option of Landlord, terminate this Lease.

29.6. Notwithstanding any Transfer, Tenant shall remain fully and primarily liable for the payment of all Rent and other sums due or to become due hereunder, and for the full performance of all other terms, conditions and covenants to be kept and performed by Tenant. The acceptance of Rent or any other sum due hereunder, or the acceptance of performance of any other term, covenant or condition thereof, from any person or entity other than Tenant shall not be deemed a waiver of any of the provisions of this Lease or a consent to any Transfer.

29.7. If Tenant delivers to Landlord a Transfer Notice indicating a desire to transfer this Lease to a proposed transferee, assignee or sublessee other than pursuant to an Exempt Transfer, then Landlord shall have the option, exercisable by giving notice to Tenant at any time within ten (10) days after Landlord's receipt of such Transfer Notice, to terminate this Lease as of the date specified in the Transfer Notice as the Transfer Date, except for those provisions that, by their express terms, survive the expiration or earlier termination hereof. If Landlord exercises such option, then Tenant shall have the right to withdraw such Transfer Notice by delivering to Landlord written notice of such election within ten (10) days after Landlord's delivery of notice electing to exercise Landlord's option to terminate this Lease. In the event Tenant withdraws the Transfer Notice as provided in this Section, this Lease shall continue in full force and effect. No failure of Landlord to exercise its option to terminate this Lease shall be deemed to be Landlord's consent to a proposed Transfer.

29.8. If Tenant sublets the Premises or any portion thereof, Tenant hereby immediately and irrevocably assigns to Landlord, as security for Tenant's obligations under this Lease, all rent from any such subletting, and appoints Landlord as assignee and attorney-in-fact for Tenant, and Landlord (or a receiver for Tenant appointed on Landlord's application) may collect such rent and apply it toward Tenant's obligations under this Lease; provided that, until the occurrence of a Default (as defined below) by Tenant, Tenant shall have the right to collect such rent.

30. Subordination and Attornment.

30.1. This Lease shall be subject and subordinate to the lien of any mortgage, deed of trust, or lease in which Landlord is tenant now or hereafter in force against the Building or the Project and to all advances made or hereafter to be made upon the security thereof without the necessity of the execution and delivery of any further instruments on the part of Tenant to effectuate such subordination. As of the Execution Date, there are no mortgages, deeds of trust or ground leases encumbering the Project. The automatic subordination to any future mortgage, deed of trust or lease provided for in this Section is expressly conditioned upon the holder of such mortgage, deed of trust or lease, agreeing that as long as no Default occurs under this Lease, the holder of such mortgage, deed of trust or lease will not disturb Tenant's rights of possession under this Lease.

30.2. Notwithstanding the foregoing, Tenant shall execute and deliver upon demand such further instrument or instruments evidencing such subordination of this Lease to the lien of any such mortgage or mortgages or deeds of trust or lease in which Landlord is tenant as may be required by Landlord. If any such mortgagee, beneficiary or landlord under a lease wherein Landlord is tenant (each, a "Mortgagee") so elects, however, this Lease shall be deemed prior in lien to any such lease, mortgage, or deed of trust upon or including the Premises regardless of date and Tenant shall execute a statement in writing to such effect at Landlord's request. For the avoidance of doubt, "Mortgagees" shall also include historic tax credit investors and new market tax credit investors.

30.3. Upon written request of Landlord and opportunity for Tenant to review, Tenant agrees to execute any Lease amendments not materially altering the terms of this Lease, if required by a Mortgagee incident to the financing of the real property of which the Premises constitute a part.

30.4. In the event any proceedings are brought for foreclosure, or in the event of the exercise of the power of sale under any mortgage or deed of trust made by Landlord covering the Premises, Tenant shall at the election of the purchaser at such foreclosure or sale attorn to the purchaser upon any such foreclosure or sale and recognize such purchaser as Landlord under this Lease.

31. Defaults and Remedies.

31.1. Late payment by Tenant to Landlord of Rent and other sums due shall cause Landlord to incur costs not contemplated by this Lease, the exact amount of which shall be extremely difficult and impracticable to ascertain. Such costs include processing and accounting charges and late charges that may be imposed on Landlord by the terms of any mortgage or trust

deed covering the Premises. Therefore, if any installment of Rent due from Tenant is not received by Landlord within five (5) days after the date such payment is due, Tenant shall pay to Landlord (a) an additional sum of five percent (5%) of the overdue Rent as a late charge plus (b) interest at an annual rate (the "Default Rate") equal to the lesser of (a) nine percent (9%) and (b) the highest rate permitted by Applicable Laws. The parties agree that this late charge represents a fair and reasonable estimate of the costs that Landlord shall incur by reason of late payment by Tenant and shall be payable as Additional Rent to Landlord due with the next installment of Rent or within seven (7) days after Landlord's written demand, whichever is earlier. Landlord's acceptance of any Additional Rent (including a late charge or any other amount hereunder) shall not be deemed an extension of the date that Rent is due or prevent Landlord from pursuing any other rights or remedies under this Lease, at law or in equity.

31.2. No payment by Tenant or receipt by Landlord of a lesser amount than the Rent payment herein stipulated shall be deemed to be other than on account of the Rent, nor shall any endorsement or statement on any check or any letter accompanying any check or payment as Rent be deemed an accord and satisfaction, and Landlord may accept such check or payment without prejudice to Landlord's right to recover the balance of such Rent or pursue any other remedy provided in this Lease or in equity or at law. If a dispute shall arise as to any amount or sum of money to be paid by Tenant to Landlord hereunder, Tenant shall have the right to make payment "under protest," such payment shall not be regarded as a voluntary payment, and there shall survive the right on the part of Tenant to institute suit for recovery of the payment paid under protest.

31.3. If Tenant fails to pay any sum of money required to be paid by it hereunder or perform any other act on its part to be performed hereunder, in each case within the applicable notice and cure period (if any) described in Section 31.4, then Landlord may (but shall not be obligated to), without waiving or releasing Tenant from any obligations of Tenant, make such payment or perform such act; provided that such failure by Tenant unreasonably interfered with the use of the Building or the Project by any other tenant or with the efficient operation of the Building or the Project, or resulted or could have resulted in a violation of Applicable Laws or the cancellation of an insurance policy maintained by Landlord. Notwithstanding the foregoing, in the event of an emergency, Landlord shall have the right to enter the Premises and act in accordance with its rights as provided elsewhere in this Lease. In addition to the late charge described in Section 31.1, Tenant shall pay to Landlord as Additional Rent all sums so paid or incurred by Landlord, together with interest at the Default Rate, computed from the date such sums were paid or incurred.

31.4. The occurrence of any one or more of the following events shall constitute a "Default" hereunder by Tenant:

(a) Tenant abandons the Premises;

(b) Tenant fails to make any payment of Rent, as and when due, or to satisfy its obligations under Article 19, where such failure shall continue for a period of five (5) days after written notice thereof from Landlord to Tenant;

(c) Tenant fails to observe or perform any obligation or covenant contained herein (other than described in Sections 31.4(a) and 31.4(b)) to be performed by Tenant, where such failure continues for a period of fifteen (15) days after written notice thereof from Landlord to Tenant; provided that, if the nature of Tenant's default is such that it reasonably requires more than fifteen (15) days to cure, Tenant shall not be deemed to be in Default if Tenant commences such cure within such fifteen (15) day period and thereafter diligently prosecutes the same to completion; and provided, further, that such cure is completed no later than thirty (30) days after Tenant's receipt of written notice from Landlord;

(d) Tenant makes an assignment for the benefit of creditors;

(e) A receiver, trustee or custodian is appointed to or does take title, possession or control of all or substantially all of Tenant's assets;

(f) Tenant files a voluntary petition under the United States Bankruptcy Code or any successor statute (as the same may be amended from time to time, the "Bankruptcy Code") or an order for relief is entered against Tenant pursuant to a voluntary or involuntary proceeding commenced under any chapter of the Bankruptcy Code;

(g) Any involuntary petition is filed against Tenant under any chapter of the Bankruptcy Code and is not dismissed within one hundred twenty (120) days;

(h) Tenant fails to deliver an estoppel certificate in accordance with Article 20; or

(i) Tenant's interest in this Lease is attached, executed upon or otherwise judicially seized and such action is not released within one hundred twenty (120) days of the action.

Notices given under this Section shall specify the alleged default and shall demand that Tenant perform the provisions of this Lease or pay the Rent that is in arrears, as the case may be, within the applicable period of time, or quit the Premises. No such notice shall be deemed a forfeiture or a termination of this Lease unless Landlord elects otherwise in such notice.

31.5. In the event of a Default by Tenant, and at any time thereafter, with or without notice or demand and without limiting Landlord in the exercise of any right or remedy that Landlord may have, Landlord has the right to do any or all of the following:

(a) Halt any Tenant Improvements and Alterations and order Tenant's contractors, subcontractors, consultants, designers and material suppliers to stop work;

(b) Terminate Tenant's right to possession of the Premises by written notice to Tenant or by any lawful means, in which case Tenant shall immediately surrender possession of the Premises to Landlord. In such event, Landlord shall have the immediate right to re-enter and remove all persons and property, and such property may be removed and stored in a public warehouse or elsewhere at the cost and for the account of Tenant, all without service of notice or

resort to legal process and without being deemed guilty of trespass or becoming liable for any loss or damage that may be occasioned thereby; and

(c) Terminate this Lease, in which event Tenant shall immediately surrender possession of the Premises to Landlord. In such event, Landlord shall have the immediate right to re-enter and remove all persons and property, and such property may be removed and stored in a public warehouse or elsewhere at the cost and for the account of Tenant, all without service of notice or resort to legal process and without being deemed guilty of trespass or becoming liable for any loss or damage that may be occasioned thereby. In the event that Landlord shall elect to so terminate this Lease, then Landlord shall be entitled to recover from Tenant all damages incurred by Landlord by reason of Tenant's default, including:

(i) The sum of:

A. The worth at the time of award of any unpaid Rent that had accrued at the time of such termination; plus

B. The worth at the time of award of the amount by which the unpaid Rent that would have accrued during the period commencing with termination of the Lease and ending at the time of award exceeds that portion of the loss of Landlord's rental income from the Premises that Tenant proves to Landlord's reasonable satisfaction could have been reasonably avoided; plus

C. The worth at the time of award of the amount by which the unpaid Rent for the balance of the Term after the time of award exceeds that portion of the loss of Landlord's rental income from the Premises that Tenant proves to Landlord's reasonable satisfaction could have been reasonably avoided; plus

D. Any other amount necessary to compensate Landlord for all the detriment caused by Tenant's failure to perform its obligations under this Lease or that in the ordinary course of things would be likely to result therefrom, including the cost of restoring the Premises to the condition required under the terms of this Lease, including any rent payments not otherwise chargeable to Tenant (e.g., during any "free" rent period or rent holiday); plus

E. At Landlord's election, such other amounts in addition to or in lieu of the foregoing as may be permitted from time to time by Applicable Laws; or

(ii) At Landlord's election, as minimum liquidated damages in addition to any (A) amounts paid or payable to Landlord pursuant to Section 31.5(c)(i)(A) prior to such election and (B) costs of restoring the Premises to the condition required under the terms of this Lease, an amount (the "Election Amount") equal to either (Y) the positive difference (if any, and measured at the time of such termination) between (1) the then-present value of the total Rent and other benefits that would have accrued to Landlord under this Lease for the remainder of the Term if Tenant had fully complied with the Lease minus (2) the then-present cash rental value of the Premises as determined by Landlord for what would be the then-unexpired Term if the Lease remained in effect, computed using the discount rate of the Federal Reserve Bank of San Francisco at the time of the award plus one (1) percentage point (the

"Discount Rate") or (Z) twelve (12) months (or such lesser number of months as may then be remaining in the Term) of Base Rent and Additional Rent at the rate last payable by Tenant pursuant to this Lease, in either case as Landlord specifies in such election. Landlord and Tenant agree that the Election Amount represents a reasonable forecast of the minimum damages expected to occur in the event of a breach, taking into account the uncertainty, time and cost of determining elements relevant to actual damages, such as fair market rent, time and costs that may be required to re-lease the Premises, and other factors; and that the Election Amount is not a penalty.

As used in Sections 31.5(c)(i)(A) and (B), "worth at the time of award" shall be computed by allowing interest at the Default Rate. As used in Section 31.5(c)(i)(C), the "worth at the time of the award" shall be computed by taking the present value of such amount, using the Discount Rate.

31.6. In addition to any other remedies available to Landlord at law or in equity and under this Lease, Landlord may continue this Lease in effect after Tenant's Default or abandonment and recover Rent as it becomes due. In addition, Landlord shall not be liable in any way whatsoever for its failure or refusal to relet the Premises, unless or to the extent required by Applicable Law. For purposes of this Section, the following acts by Landlord will not constitute the termination of Tenant's right to possession of the Premises:

(a) Acts of maintenance or preservation or efforts to relet the Premises, including alterations, remodeling, redecorating, repairs, replacements or painting as Landlord shall consider advisable for the purpose of reletting the Premises or any part thereof; or

(b) The appointment of a receiver upon the initiative of Landlord to protect Landlord's interest under this Lease or in the Premises.

Notwithstanding the foregoing, in the event of a Default by Tenant, Landlord may elect at any time to terminate this Lease and to recover damages to which Landlord is entitled.

31.7. If Landlord does not elect to terminate this Lease as provided in Section 31.5, then Landlord may, from time to time, recover all Rent as it becomes due under this Lease. At any time thereafter, Landlord may elect to terminate this Lease and to recover damages to which Landlord is entitled.

31.8. In the event Landlord elects to terminate this Lease and relet the Premises, Landlord may execute any new lease in its own name. Tenant hereunder shall have no right or authority whatsoever to collect any Rent from such tenant. The proceeds of any such reletting shall be applied as follows:

(a) First, to the payment of any indebtedness other than Rent due hereunder from Tenant to Landlord, including storage charges or brokerage commissions owing from Tenant to Landlord as the result of such reletting;

(b) Second, to the payment of the costs and expenses of reletting the Premises, including (i) alterations and repairs that Landlord deems reasonably necessary and

advisable and (ii) reasonable attorneys' fees, charges and disbursements incurred by Landlord in connection with the retaking of the Premises and such reletting;

(c) Third, to the payment of Rent and other charges due and unpaid hereunder; and

(d) Fourth, to the payment of future Rent and other damages payable by Tenant under this Lease.

31.9. All of Landlord's rights, options and remedies hereunder shall be construed and held to be nonexclusive and cumulative. Landlord shall have the right to pursue any one or all of such remedies, or any other remedy or relief that may be provided by Applicable Laws, whether or not stated in this Lease. No waiver of any default of Tenant hereunder shall be implied from any acceptance by Landlord of any Rent or other payments due hereunder or any omission by Landlord to take any action on account of such default if such default persists or is repeated, and no express waiver shall affect defaults other than as specified in such waiver. Notwithstanding any provision of this Lease to the contrary, in no event shall Landlord be required to mitigate its damages with respect to any default by Tenant, unless or to the extent required by Applicable Law. Any obligation imposed by Applicable Law upon Landlord to relet the Premises after any termination of this Lease shall be subject to the reasonable requirements of Landlord to (a) lease to high quality tenants on such terms as Landlord may from time to time deem appropriate in its discretion and (b) develop the Project in a harmonious manner with a mix of uses, tenants, floor areas, terms of tenancies, etc., as determined by Landlord. Landlord shall not be obligated to relet the Premises to any party to whom Landlord or an affiliate of Landlord may desire to lease other available space in the Project or at another property owned by Landlord or an affiliate of Landlord.

31.10. Landlord's termination of (a) this Lease or (b) Tenant's right to possession of the Premises shall not relieve Tenant of any liability to Landlord that has previously accrued or that shall arise based upon events that occurred prior to the later to occur of (y) the date of Lease termination and (z) the date Tenant surrenders possession of the Premises.

31.11. To the extent permitted by Applicable Laws, Tenant waives any and all rights of redemption granted by or under any present or future Applicable Laws if Tenant is evicted or dispossessed for any cause, or if Landlord obtains possession of the Premises due to Tenant's default hereunder or otherwise.

31.12. Landlord shall not be in default or liable for damages under this Lease unless Landlord fails to perform obligations required of Landlord within a reasonable time, but in no event shall such failure continue for more than thirty (30) days after written notice from Tenant specifying the nature of Landlord's failure; provided, however, that if the nature of Landlord's obligation is such that more than thirty (30) days are required for its performance, then Landlord shall not be in default if Landlord commences performance within such thirty (30) day period and thereafter diligently prosecutes the same to completion. In no event shall Tenant have the right to terminate or cancel this Lease or to withhold or abate rent or to set off any Claims against Rent as a result of any default or breach by Landlord of any of its covenants, obligations,

representations, warranties or promises hereunder, except as may otherwise be expressly set forth in this Lease.

31.13. In the event of any default by Landlord, Tenant shall give notice by registered or certified mail or by a reputable international overnight delivery service, such as FedEx, to any (a) beneficiary of a deed of trust or (b) mortgagee under a mortgage covering the Premises, the Building or the Project and to any landlord of any lease of land upon or within which the Premises, the Building or the Project is located, and shall offer such beneficiary, mortgagee or landlord a reasonable opportunity to cure the default, including time to obtain possession of the Building or the Project by power of sale or a judicial action if such should prove necessary to effect a cure; provided that Landlord shall furnish to Tenant in writing, upon written request by Tenant, the names and addresses of all such persons who are to receive such notices.

32. Bankruptcy. In the event a debtor, trustee or debtor in possession under the Bankruptcy Code, or another person with similar rights, duties and powers under any other Applicable Laws, proposes to cure any default under this Lease or to assume or assign this Lease and is obliged to provide adequate assurance to Landlord that (a) a default shall be cured, (b) Landlord shall be compensated for its damages arising from any breach of this Lease and (c) future performance of Tenant's obligations under this Lease shall occur, then such adequate assurances shall include any or all of the following, as designated by Landlord in its sole and absolute discretion:

32.1. Those acts specified in the Bankruptcy Code or other Applicable Laws as included within the meaning of "adequate assurance," even if this Lease does not concern a shopping center or other facility described in such Applicable Laws;

32.2. A prompt cash payment to compensate Landlord for any monetary defaults or actual damages arising directly from a breach of this Lease;

32.3. A cash deposit in an amount at least equal to the then-current amount of the Security Deposit; or

32.4. The assumption or assignment of all of Tenant's interest and obligations under this Lease.

33. Brokers.

33.1. Tenant represents and warrants that it has had no dealings with any real estate broker or agent in connection with the negotiation of this Lease other than Scheer Partners ("Broker"), and that it knows of no other real estate broker or agent that is or might be entitled to a commission in connection with this Lease. Landlord shall compensate Broker in relation to this Lease pursuant to a separate agreement between Landlord and Broker.

33.2. Tenant represents and warrants that no broker or agent has made any representation or warranty relied upon by Tenant in Tenant's decision to enter into this Lease, other than as contained in this Lease.

33.3. Tenant acknowledges and agrees that the employment of brokers by Landlord is for the purpose of solicitation of offers of leases from prospective tenants and that no authority is

granted to any broker to furnish any representation (written or oral) or warranty from Landlord unless expressly contained within this Lease. Landlord is executing this Lease in reliance upon Tenant's representations, warranties and agreements contained within Sections 33.1 and 33.2.

33.4. Tenant agrees to indemnify, save, defend (at Landlord's option and with counsel reasonably acceptable to Landlord) and hold the Landlord Indemnitees harmless from any and all cost or liability for compensation claimed by any broker or agent, other than Broker, employed or engaged by Tenant or claiming to have been employed or engaged by Tenant.

33.5. Landlord represents and warrants that it has had no dealings with any real estate broker or agent in connection with the negotiation of this Lease other than Broker, and that it knows of no other real estate broker or agent that is or might be entitled to a commission in connection with this Lease. Landlord agrees to indemnify, save, defend (at Tenant's option and with counsel reasonably acceptable to Tenant) and hold Tenant harmless from any and all cost or liability for compensation claimed by any broker or agent, other than Broker, employed or engaged by Landlord or claiming to have been employed or engaged by Landlord.

34. Definition of Landlord. With regard to obligations imposed upon Landlord pursuant to this Lease, the term "Landlord," as used in this Lease, shall refer only to Landlord or Landlord's then-current successor-in-interest. In the event of any transfer, assignment or conveyance of Landlord's interest in this Lease or in Landlord's fee title to or leasehold interest in the Property, as applicable, Landlord herein named (and in case of any subsequent transfers or conveyances, the subsequent Landlord) shall be automatically freed and relieved, from and after the date of such transfer, assignment or conveyance, from all liability for the performance of any covenants or obligations contained in this Lease thereafter to be performed by Landlord and, without further agreement, the transferee, assignee or conveyee of Landlord's in this Lease or in Landlord's fee title to or leasehold interest in the Property, as applicable, shall be deemed to have assumed and agreed to observe and perform any and all covenants and obligations of Landlord hereunder during the tenure of its interest in the Lease or the Property. Landlord or any subsequent Landlord may transfer its interest in the Premises or this Lease without Tenant's consent.

35. Limitation of Landlord's Liability.

35.1. If Landlord is in default under this Lease and, as a consequence, Tenant recovers a monetary judgment against Landlord, the judgment shall be satisfied only out of (a) the proceeds of sale received on execution of the judgment and levy against the right, title and interest of Landlord in the Building and the Project, (b) rent or other income from such real property receivable by Landlord, (c) the consideration received by Landlord from the sale, financing, refinancing or other disposition of all or any part of Landlord's right, title or interest in the Building or the Project and/or (d) any insurance proceeds or condemnation awards received by Landlord in connection with a casualty loss or condemnation of the Building or Project.

35.2. Neither Landlord nor any of its affiliates, nor any of their respective partners, shareholders, directors, officers, employees, members or agents shall be personally liable for Landlord's obligations or any deficiency under this Lease, and service of process shall not be

made against any shareholder, director, officer, employee or agent of Landlord or any of Landlord's affiliates. No partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates shall be sued or named as a party in any suit or action, and service of process shall not be made against any partner or member of Landlord except as may be necessary to secure jurisdiction of the partnership, joint venture or limited liability company, as applicable. No partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates shall be required to answer or otherwise plead to any service of process, and no judgment shall be taken or writ of execution levied against any partner, shareholder, director, officer, employee, member or agent of Landlord or any of its affiliates.

35.3. Each of the covenants and agreements of this Article shall be applicable to any covenant or agreement either expressly contained in this Lease or imposed by Applicable Laws and shall survive the expiration or earlier termination of this Lease.

36. Joint and Several Obligations. If more than one person or entity executes this Lease as Tenant, then:

36.1. Each of them is jointly and severally liable for the keeping, observing and performing of all of the terms, covenants, conditions, provisions and agreements of this Lease to be kept, observed or performed by Tenant, and such terms, covenants, conditions, provisions and agreements shall be binding with the same force and effect upon each and all of the persons executing this Agreement as Tenant; and

36.2. The term "Tenant." as used in this Lease, shall mean and include each of them, jointly and severally. The act of, notice from, notice to, refund to, or signature of any one or more of them with respect to the tenancy under this Lease, including any renewal, extension, expiration, termination or modification of this Lease, shall be binding upon each and all of the persons executing this Lease as Tenant with the same force and effect as if each and all of them had so acted, so given or received such notice or refund, or so signed.

37. Representations. Tenant guarantees, warrants and represents that (a) Tenant is duly incorporated or otherwise established or formed and validly existing under the laws of its state of incorporation, establishment or formation, (b) Tenant has and is duly qualified to do business in the state in which the Property is located, (c) Tenant has full corporate, partnership, trust, association or other appropriate power and authority to enter into this Lease and to perform all Tenant's obligations hereunder, (d) each person (and all of the persons if more than one signs) signing this Lease on behalf of Tenant is duly and validly authorized to do so and (e) neither (i) the execution, delivery or performance of this Lease nor (ii) the consummation of the transactions contemplated hereby will violate or conflict with any provision of documents or instruments under which Tenant is constituted or to which Tenant is a party. In addition, Tenant guarantees, warrants and represents that none of (x) it, (y) its affiliates or partners nor (z) to the best of its knowledge (without independent investigation), its members, shareholders or other equity owners or any of their respective employees, officers, directors, representatives or agents is a person or entity with whom U.S. persons or entities are restricted from doing business under regulations of the Office of Foreign Asset Control ("OFAC") of the Department of the Treasury (including those named on OFAC's Specially Designated and Blocked Persons List) or under any statute, executive order (including the September 24, 2001, Executive Order Blocking

Property and Prohibiting Transactions with Persons Who Commit, Threaten to Commit, or Support Terrorism) or other similar governmental action. Landlord guarantees, warrants and represents that (aa) Landlord is duly incorporated or otherwise established or formed and validly existing under the laws of its state of incorporation, establishment or formation, (bb) Landlord has and is duly qualified to do business in the state in which the Property is located, (cc) Landlord has full corporate, partnership, trust, association or other appropriate power and authority to enter into this Lease and to perform all Landlord's obligations hereunder, (dd) each person (and all of the persons if more than one signs) signing this Lease on behalf of Landlord is duly and validly authorized to do so and (ee) neither (i) the execution, delivery or performance of this Lease nor (ii) the consummation of the transactions contemplated hereby will violate or conflict with any provision of documents or instruments under which Landlord is constituted or to which Landlord is a party. In addition, Landlord represents that, to its current, actual knowledge (without duty of inquiry), it is not an entity with whom U.S. persons or entities are restricted from doing business under regulations of OFAC (including those named on OFAC's Specially Designated and Blocked Persons List) or under any statute, executive order (including the September 24, 2001, Executive Order Blocking Property and Prohibiting Transactions with Persons Who Commit, Threaten to Commit, or Support Terrorism) or other similar governmental action.

38. Confidentiality. Tenant shall keep the terms and conditions of this Lease and any information provided to Tenant or its employees, agents or contractors pursuant to Article 9 confidential and shall not (a) disclose to any third party any terms or conditions of this Lease or any other Lease-related document (including subleases, assignments, work letters, construction contracts, letters of credit, subordination agreements, non-disturbance agreements, brokerage agreements or estoppels) or (b) provide to any third party an original or copy of this Lease (or any Lease-related document). Landlord shall not release to any third party any non-public financial information or non-public information about Tenant's ownership structure that Tenant gives Landlord. Notwithstanding the foregoing, confidential information under this Section may be released by Landlord or Tenant under the following circumstances: (x) if required by Applicable Laws or in any judicial proceeding; provided that the releasing party has given the other party reasonable notice of such requirement, if feasible, (y) to a party's attorneys, accountants, brokers and other bona fide consultants or advisers (with respect to this Lease only); provided such third parties agree to be bound by this Section or (z) to bona fide prospective assignees or subtenants of this Lease; provided they agree in writing to be bound by this Section. For avoidance of doubt, if and only to the extent required by Applicable Laws, Tenant may disclose this Lease (including filing a copy of the Lease if, and only if, required by Applicable Laws) in its 8-K filing with the Securities and Exchange Commission.

39. Notices. Except as otherwise stated in this Lease, any notice, consent, demand, invoice, statement or other communication required or permitted to be given hereunder shall be in writing and shall be given by (a) personal delivery, (b) overnight delivery with a reputable international overnight delivery service, such as FedEx, or (c) facsimile or email transmission, so long as such transmission is followed within one (1) business day by delivery utilizing one of the methods described in Subsection 39(a) or (b). Any such notice, consent, demand, invoice, statement or other communication shall be deemed delivered (x) upon receipt, if given in accordance with Subsection 39(a); (y) one (1) business day after deposit with a reputable international overnight

delivery service, if given if given in accordance with Subsection 39(b); or (z) upon transmission, if given in accordance with Subsection 39(c). Except as otherwise stated in this Lease, any notice, consent, demand, invoice, statement or other communication required or permitted to be given pursuant to this Lease shall be addressed to Tenant at the Premises, or to Landlord or Tenant at the addresses shown in Sections 2.9 and 2.10 or 2.11, respectively. Either party may, by notice to the other given pursuant to this Section, specify additional or different addresses for notice purposes.

40. Miscellaneous.

40.1. Landlord reserves the right to change the name of the Building or the Project in its sole discretion.

40.2. To induce Landlord to enter into this Lease, Tenant agrees that it shall promptly furnish to Landlord, from time to time, upon Landlord's written request (but no more than one (1) time per calendar year), the most recent year-end unconsolidated financial statements reflecting Tenant's current financial condition audited by a nationally recognized accounting firm. Tenant shall, within ninety (90) days after the end of Tenant's financial year, furnish Landlord with a certified copy of Tenant's year-end unconsolidated financial statements for the previous year audited by a nationally recognized accounting firm. Tenant represents and warrants that all financial statements, records and information furnished by Tenant to Landlord in connection with this Lease are true, correct and complete in all respects. If audited financials are not otherwise prepared, unaudited financials complying with generally accepted accounting principles and certified by the chief financial officer of Tenant as true, correct and complete in all respects shall suffice for purposes of this Section. The provisions of this Section shall not apply at any time while Tenant is a corporation whose shares are traded on any nationally recognized stock exchange.

40.3. Submission of this instrument for examination or signature by Tenant does not constitute a reservation of or option for a lease, and shall not be effective as a lease or otherwise until execution by and delivery to both Landlord and Tenant.

40.4. The terms of this Lease are intended by the parties as a final, complete and exclusive expression of their agreement with respect to the terms that are included herein, and may not be contradicted or supplemented by evidence of any other prior or contemporaneous agreement.

40.5. Landlord may, but shall not be obligated to, record a short form or memorandum hereof (at Landlord's sole cost and expense) without Tenant's consent. Within ten (10) days after receipt of written request from Landlord, Tenant shall execute a termination of any short form or memorandum of lease recorded with respect hereto. Neither party shall record this Lease.

40.6. Where applicable in this Lease, the singular includes the plural and the masculine or neuter includes the masculine, feminine and neuter. The words "include," "includes," "included" and "including" mean "'include,' etc., without limitation." The word "shall" is mandatory and the word "may" is permissive. The section headings of this Lease are not a part

of this Lease and shall have no effect upon the construction or interpretation of any part of this Lease. Landlord and Tenant have each participated in the drafting and negotiation of this Lease, and the language in all parts of this Lease shall be in all cases construed as a whole according to its fair meaning and not strictly for or against either Landlord or Tenant.

40.7. Except as otherwise expressly set forth in this Lease, each party shall pay its own costs and expenses incurred in connection with this Lease and such party's performance under this Lease; provided that, if either party commences an action, proceeding, demand, claim, action, cause of action or suit against the other party arising out of or in connection with this Lease, then the substantially prevailing party shall be reimbursed by the other party for all reasonable costs and expenses, including reasonable attorneys' fees and expenses, incurred by the substantially prevailing party in such action, proceeding, demand, claim, action, cause of action or suit, and in any appeal in connection therewith (regardless of whether the applicable action, proceeding, demand, claim, action, cause of action, suit or appeal is voluntarily withdrawn or dismissed).

40.8. Time is of the essence with respect to the performance of every provision of this Lease.

40.9. Each provision of this Lease performable by Tenant shall be deemed both a covenant and a condition.

40.10. Notwithstanding anything to the contrary contained in this Lease, Tenant's obligations under this Lease are independent and shall not be conditioned upon performance by Landlord.

40.11. Whenever consent or approval of either party is required, that party shall not unreasonably withhold, condition or delay such consent or approval, except as may be expressly set forth to the contrary.

40.12. Any provision of this Lease that shall prove to be invalid, void or illegal shall in no way affect, impair or invalidate any other provision hereof, and all other provisions of this Lease shall remain in full force and effect and shall be interpreted as if the invalid, void or illegal provision did not exist.

40.13. Each of the covenants, conditions and agreements herein contained shall inure to the benefit of and shall apply to and be binding upon the parties hereto and their respective heirs; legatees; devisees; executors; administrators; and permitted successors and assigns. This Lease is for the sole benefit of the parties and their respective heirs, legatees, devisees, executors, administrators and permitted successors and assigns, and nothing in this Lease shall give or be construed to give any other person or entity any legal or equitable rights. Nothing in this Section shall in any way alter the provisions of this Lease restricting assignment or subletting.

40.14. This Lease shall be governed by, construed and enforced in accordance with the laws of the state in which the Premises are located, without regard to such state's conflict of law principles.

40.15. Tenant guarantees, warrants and represents that the individual or individuals signing this Lease have the power, authority and legal capacity to sign this Lease on behalf of and to bind all entities, corporations, partnerships, limited liability companies, joint venturers or other organizations and entities on whose behalf such individual or individuals have signed. Landlord guarantees, warrants and represents that the individual or individuals signing this Lease have the power, authority and legal capacity to sign this Lease on behalf of and to bind all entities, corporations, partnerships, limited liability companies, joint venturers or other organizations and entities on whose behalf such individual or individuals have signed.

40.16. This Lease may be executed in one or more counterparts, each of which, when taken together, shall constitute one and the same document. Signature pages may be detached from the counterparts and attached to a single copy of this Lease to form one (1) document. A facsimile, electronic or portable document format (PDF) signature on this Lease shall be equivalent to, and have the same force and effect as, an original signature.

40.17. No provision of this Lease may be modified, amended or supplemented except by an agreement in writing signed by Landlord and Tenant.

40.18. No waiver of any term, covenant or condition of this Lease shall be binding upon Landlord unless executed in writing by Landlord. No waiver of any term, covenant or condition of this Lease shall be binding upon Tenant unless executed in writing by Tenant. The waiver by Landlord or Tenant of any breach or default of any term, covenant or condition contained in this Lease shall not be deemed to be a waiver of any preceding or subsequent breach or default of such term, covenant or condition or any other term, covenant or condition of this Lease.

40.19. To the extent permitted by Applicable Laws, the parties waive trial by jury in any action, proceeding or counterclaim brought by the other party hereto related to matters arising out of or in any way connected with this Lease; the relationship between Landlord and Tenant; Tenant's use or occupancy of the Premises; or any claim of injury or damage related to this Lease or the Premises.

41. [Intentionally omitted]

42. Options to Extend Term. Tenant shall have two (2) options (each, an "Option") to extend the Term by three (3) years each as to the entire Premises (and no less than the entire Premises) upon the following terms and conditions. Any extension of the Term pursuant to an Option shall be on all the same terms and conditions as this Lease, except as follows:

42.1. Base Rent during each Option term (including annual escalations, if any) shall equal the then-current fair market value for comparable office space in the Rockville, Maryland submarket of comparable age, quality, level of finish and proximity to amenities and public transit ("FMV"). Tenant may, no more than twelve (12) months prior to the date the Term is then scheduled to expire, request Landlord's estimate of the FMV for the next Option term. Landlord shall, within fifteen (15) days after receipt of such request, give Tenant a written proposal of such FMV. If Tenant gives written notice to exercise an Option, such notice shall specify whether Tenant accepts Landlord's proposed estimate of FMV. If Tenant does not accept the FMV, then the parties shall endeavor to agree upon the FMV, taking into account all

relevant factors, including (a) the size of the Premises, (b) the length of the Option term, (c) rent in comparable buildings in the relevant submarket, including concessions offered to new tenants, such as free rent, tenant improvement allowances and moving allowances, (d) Tenant's creditworthiness and (e) the quality and location of the Building and the Project. In the event that the parties are unable to agree upon the FMV within thirty (30) days after Tenant notifies Landlord that Tenant is exercising an Option, then either party may request that the same be determined as follows: a senior officer of a nationally recognized leasing brokerage firm with local knowledge of the Rockville, Maryland office leasing submarket (the "Baseball Arbitrator") shall be selected and paid for jointly by Landlord and Tenant. If Landlord and Tenant are unable to agree upon the Baseball Arbitrator, then the same shall be designated by the local chapter of the Judicial Arbitration and Mediation Services or any successor organization thereto (the "JAMS"). The Baseball Arbitrator selected by the parties or designated by JAMS shall (y) have at least ten (10) years' experience in the leasing of office space in the Rockville, Maryland submarket and (z) not have been employed or retained by either Landlord or Tenant or any affiliate of either for a period of at least ten (10) years prior to appointment pursuant hereto. Each of Landlord and Tenant shall submit to the Baseball Arbitrator and to the other party its determination of the FMV. The Baseball Arbitrator shall grant to Landlord and Tenant a hearing and the right to submit evidence. The Baseball Arbitrator shall determine which of the two (2) FMV determinations more closely represents the actual FMV. The arbitrator may not select any other FMV for the Premises other than one submitted by Landlord or Tenant. The FMV selected by the Baseball Arbitrator shall be binding upon Landlord and Tenant and shall serve as the basis for determination of Base Rent payable for the applicable Option term. If, as of the commencement date of an Option term, the amount of Base Rent payable during the Option term shall not have been determined, then, pending such determination, Tenant shall pay Base Rent equal to the Base Rent payable with respect to the last year of the then-current Term. After the final determination of Base Rent payable for the Option term, the parties shall promptly execute a written amendment to this Lease specifying the amount of Base Rent to be paid during the applicable Option term and any overpayment or underpayment of Base Rent for the Option term prior to such determination shall be adjusted between the parties. Any failure of the parties to execute such amendment shall not affect the validity of the FMV determined pursuant to this Section.

42.2. No Option is assignable separate and apart from this Lease.

42.3. An Option is conditional upon Tenant giving Landlord written notice of its election to exercise such Option at least nine (9) months prior to the end of the expiration of the then-current Term. Time shall be of the essence as to Tenant's exercise of an Option. Tenant assumes full responsibility for maintaining a record of the deadlines to exercise an Option. Tenant acknowledges that it would be inequitable to require Landlord to accept any exercise of an Option after the date provided for in this Section.

42.4. Notwithstanding anything contained in this Article to the contrary, Tenant shall not have the right to exercise an Option:

(a) During the time commencing from the date Landlord delivers to Tenant a written notice that Tenant is in default under any provisions of this Lease and continuing until Tenant has cured the specified default; or

(b) At any time after any Default as described in Article 31 of the Lease (provided, however, that, for purposes of this Section 42.4(b), Landlord shall not be required to provide Tenant with a separate notice of such Default) and continuing until Tenant cures any such Default, if such Default is susceptible to being cured; or

(c) In the event that Tenant has defaulted in the performance of its obligations under this Lease three (3) or more times during the twelve (12)-month period immediately prior to the date that Tenant intends to exercise an Option, whether or not Tenant has cured such defaults.

42.5. The period of time within which Tenant may exercise an Option shall not be extended or enlarged by reason of Tenant's inability to exercise such Option because of the provisions of Section 42.4.

42.6. All of Tenant's rights under the provisions of an Option shall terminate and be of no further force or effect even after Tenant's due and timely exercise of such Option if, after such exercise, but prior to the commencement date of the new term, (a) Tenant fails to pay to Landlord a monetary obligation of Tenant for a period of twenty (20) days after written notice from Landlord to Tenant, (b) Tenant fails to commence to cure a default (other than a monetary default) within thirty (30) days after the date Landlord gives notice to Tenant of such default or (c) Tenant has defaulted under this Lease three (3) or more times and a service or late charge under Section 31.1 has become payable for any such default, whether or not Tenant has cured such defaults.

43. Right of First Refusal. Commencing upon the Term Commencement Date, but subject to any other parties' pre-existing rights existing on the Term Commencement Date with respect to Available ROFR Premises (as defined below) (including, without limitation, any such rights granted to GlycoMimetics, Inc.), Tenant shall have a right of first refusal ("ROFR") as to any rentable premises on the second (2nd) floor of the building located at 9714 Medical Center Drive, Rockville, Maryland, for which Landlord is seeking a tenant ("Available ROFR Premises"); provided, however, that in no event shall Landlord be required to lease any Available ROFR Premises to Tenant for any period past the date on which this Lease expires or is terminated pursuant to its terms. To the extent that Landlord renews or extends a then-existing lease with any then-existing tenant or subtenant of any space, or enters into a new lease with such then-existing tenant or subtenant for the same premises, the affected space shall not be deemed to be Available ROFR Premises. In the event Landlord receives from a third party a bona fide offer to lease Available ROFR Premises, Landlord shall provide written notice thereof to Tenant (the "Notice of Offer"), specifying the terms and conditions of a proposed lease to Tenant of the Available ROFR Premises.

43.1. Within ten (10) days following its receipt of a Notice of Offer, Tenant shall advise Landlord in writing whether Tenant elects to lease all (not just a portion) of the Available ROFR Premises on the terms and conditions set forth in the Notice of Offer. If Tenant fails to notify

Landlord of Tenant's election within such ten (10) day period, then Tenant shall be deemed to have elected not to lease the Available ROFR Premises.

43.2. If Tenant timely notifies Landlord that Tenant elects to lease the Available ROFR Premises on the terms and conditions set forth in the Notice of Offer, then Landlord shall lease the Available ROFR Premises to Tenant upon the terms and conditions set forth in the Notice of Offer.

43.3. If Tenant notifies Landlord that Tenant elects not to lease the Available ROFR Premises on the terms and conditions set forth in the Notice of Offer, or if Tenant fails to notify Landlord of Tenant's election within the ten (10)-day period described above, then Landlord shall have the right to consummate the lease of the Available ROFR Premises on the same terms as set forth in the Notice of Offer following Tenant's election (or deemed election) not to lease the Available ROFR Premises. If Landlord does not lease the Available ROFR Premises within one hundred eighty (180) days after Tenant's election (or deemed election) not to lease the Available ROFR Premises, then the ROFR shall be fully reinstated, and Landlord shall not thereafter lease the Available ROFR Premises without first complying with the procedures set forth in this Article.

43.4. Notwithstanding anything in this Article to the contrary, Tenant shall not exercise the ROFR during such period of time that Tenant is in Default under any provision of this Lease. Any attempted exercise of the ROFR during a period of time in which Tenant is so in Default shall be void and of no effect. In addition, Tenant shall not be entitled to exercise the ROFR if Landlord has given Tenant two (2) or more notices of default under this Lease, whether or not the defaults are cured, during the twelve (12) month period prior to the date on which Tenant seeks to exercise the ROFR.

43.5. Notwithstanding anything in this Lease to the contrary, Tenant shall not assign or transfer the ROFR, either separately or in conjunction with an assignment or transfer of Tenant's interest in the Lease, without Landlord's prior written consent, which consent Landlord may withhold in its sole and absolute discretion.

43.6. If Tenant exercises the ROFR, Landlord does not guarantee that the Available ROFR Premises will be available on the anticipated commencement date for the Lease as to such Available ROFR Premises due to a holdover by the then-existing occupants of the Available ROFR Premises or for any other reason beyond Landlord's reasonable control.

43.7. At any time following the Term Commencement Date, upon Tenant's request, Landlord shall advise Tenant of any other parties holding pre-existing rights with respect to Available ROFR Premises.

44. Existing Furniture. Concurrently with the execution of this Lease, Landlord and Tenant shall execute and deliver a Bill of Sale substantially in the form of attached Exhibit D, whereby Landlord shall convey to Tenant any rights Landlord has in certain personal property.

[REMAINDER OF THIS PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the parties hereto have executed this Lease as of the date first above written.

LANDLORD:

BMR-MEDICAL CENTER DRIVE LLC,
a Delaware limited liability company

By: /s/ Kevin M. Simonsen
Name: Kevin M. Simonsen
Title: Sr. VP, Real Estate Legal

TENANT:

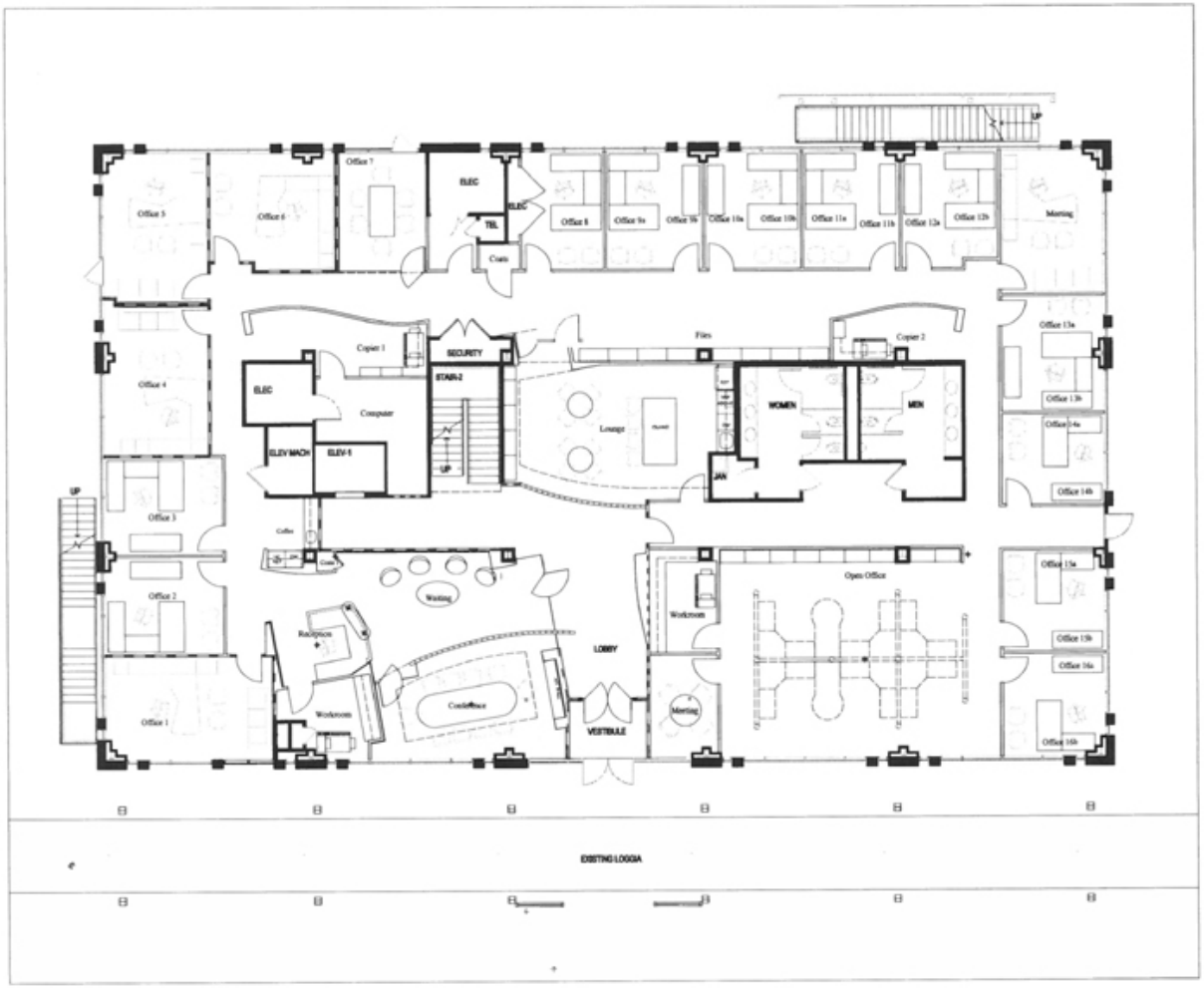
REGENXBIO INC.,
a Delaware corporation

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

EXHIBIT A

PREMISES

A-l



Glenn Forrest AIA Architect
8120 Woodmont Avenue
Woodmont Plaza Suite 500
Bethesda, MD 20814
Fax 301.951.0041
301.951.0500

dx
STANDARDS

Suite 100

USDS, Inc.
9712 Medical Center Dr.
Suite 100
Montgomery County
Rockville, MD 20850

Furniture Plan

Ground Floor

A2.6

EXHIBIT B

TENANT IMPROVEMENT PLANS

PERKINS
+WILL

REGENX
TENANT
IMPROVEMENT

LEASE EXHIBIT

1 LEVEL 01 PLAN

Scope of Work

1. Repaint walls throughout Premises
2. Install door in hallway near security and electrical
3. Install 1-2 ton HVAC in server room with humidification to support equipment in chart below
4. Install additional power and backup power in server room to support equipment in chart below

BRAND	EQUIPMENT	VALUES PER ITEM									QTY	EXTENDED VALUES			Outlets (Per Rack)
		DIMENSIONS (in.)			Weight (Lbs)	Volts (V)	Amps (A)	Electrical Load		Heat Load (BTU/Hr)		Weight (Lbs)	Electrical Load (Total KVA)	Heat Load (BTU/Hr)	
		H	W	D				(VA)	(KVA)						
Server Cabinet 1															
TBD	Cabinet	84.00	24.00	48.00	100	N/A	N/A	N/A	N/A	N/A	1	100	N/A	N/A	(2) L6-30 (4) L5-30
Cisco	MCS 7828-H5 (Call Manager)	1.70	16.73	24.00	27	120	4.5	540	0.54	1,841	1	27	0.54	1,841	
Liebert	PSI-XR PSI2T0XRT3	3.50	17.70	27.60	93	120	16.0	2160	2.16	7,366	1	93	2.16	7,366	
Liebert	PSI-XR PSI2T0XRT3	3.50	17.70	27.60	93	120	16.0	2160	2.16	7,366	1	93	2.16	7,366	
Cisco	2821 ISR (Router)	1.75	17.50	9.40	8	120	6.0	720	0.72	2,455	1	8	0.72	2,455	
Cisco	3560-48PS (Switch)	1.73	17.50	16.90	13	120	4.0	480	0.48	1,637	2	26	0.96	3,274	
TBD	Spare					120	5.0	600	0.60	2,046	1	10	0.60	2,046	
TBD	Spare					120	5.0	600	0.60	2,046	1	10	0.60	2,046	
TBD	Keyes	4	16	22	70	120	5.0	1200	2.2	2,046	1	70	2.2	7,502	
										Total:		437	9.94	33,898	

5. Install new refrigerator in kitchen area of similar quality/style to existing appliances
6. Repair coat closet in reception area

EXHIBIT B-1

TENANT WORK INSURANCE SCHEDULE

Tenant shall be responsible for requiring all of Tenant contractors doing construction or renovation work to purchase and maintain such insurance as shall protect it from the claims set forth below which may arise out of or result from any Tenant Work whether such Tenant Work is completed by Tenant or by any Tenant contractors or by any person directly or indirectly employed by Tenant or any Tenant contractors, or by any person for whose acts Tenant or any Tenant contractors may be liable:

1. Claims under workers' compensation, disability benefit and other similar employee benefit acts which are applicable to the Tenant Work to be performed.

2. Claims for damages because of bodily injury, occupational sickness or disease, or death of employees under any applicable employer's liability law.

3. Claims for damages because of bodily injury, or death of any person other than Tenant's or any Tenant contractors' employees.

4. Claims for damages insured by usual personal injury liability coverage which are sustained (a) by any person as a result of an offense directly or indirectly related to the employment of such person by Tenant or any Tenant contractors or (b) by any other person.

5. Claims for damages, other than to the Tenant Work itself, because of injury to or destruction of tangible property, including loss of use therefrom.

6. Claims for damages because of bodily injury or death of any person or property damage arising out of the ownership, maintenance or use of any motor vehicle.

Tenant contractors' Commercial General Liability Insurance shall include premises/operations (including explosion, collapse and underground coverage if such Tenant Work involves any underground work), elevators, independent contractors, products and completed operations, and blanket contractual liability on all written contracts, all including broad form property damage coverage.

Tenant contractors' Commercial General, Automobile, Employers and Umbrella Liability Insurance shall be written for not less than limits of liability as follows:

a.	Commercial General Liability: Bodily Injury and Property Damage	Commercially reasonable amounts, but in any event no less than $1,000,000 per occurrence and $2,000,000 general aggregate, with $2,000,000 products and completed operations aggregate.

b.	Commercial Automobile Liability: Bodily Injury and Property Damage	$1,000,000 per accident
c.	Employer's Liability: Each Accident Disease – Policy Limit Disease – Each Employee	$500,000 $500,000 $500,000
d.	Umbrella Liability: Bodily Injury and Property Damage	Commercially reasonable amounts (excess of coverages a, b and c above), but in any event no less than $5,000,000 per occurrence / aggregate.

All subcontractors for Tenant contractors shall carry the same coverages and limits as specified above, unless different limits are reasonably approved by Landlord. The foregoing policies shall contain a provision that coverages afforded under the policies shall not be canceled or not renewed until at least thirty (30) days' prior written notice has been given to the Landlord. Certificates of insurance including required endorsements showing such coverages to be in force shall be filed with Landlord prior to the commencement of any Tenant Work and prior to each renewal. Coverage for completed operations must be maintained for the lesser of ten (10) years and the applicable statue of repose following completion of the Tenant Work, and certificates evidencing this coverage must be provided to Landlord. The minimum A.M. Best's rating of each insurer shall be A- VII. Landlord and its mortgagees shall be named as an additional insureds under Tenant contractors' Commercial General Liability, Commercial Automobile Liability and Umbrella Liability Insurance policies as respects liability arising from work or operations performed, or ownership, maintenance or use of autos, by or on behalf of such contractors. Each contractor and its insurers shall provide waivers of subrogation with respect to any claims covered or that should have been covered by valid and collectible insurance, including any deductibles or self-insurance maintained thereunder.

If any contractor's work involves the handling or removal of asbestos (as determined by Landlord in its sole and absolute discretion), such contractor shall also carry Pollution Legal Liability insurance. Such coverage shall include bodily injury, sickness, disease, death or mental anguish or shock sustained by any person; property damage, including physical injury to or destruction of tangible property (including the resulting loss of use thereof), clean-up costs and the loss of use of tangible property that has not been physically injured or destroyed; and defense costs, charges and expenses incurred in the investigation, adjustment or defense of claims for such damages. Coverage shall apply to both sudden and non-sudden pollution conditions including the discharge, dispersal, release or escape of smoke, vapors, soot, fumes, acids, alkalis, toxic chemicals, liquids or gases, waste materials or other irritants, contaminants or pollutants into or upon land, the atmosphere or any watercourse or body of water. Claims-made coverage is permitted, provided the policy retroactive date is continuously maintained prior to the Term Commencement Date, and coverage is continuously maintained during all periods in which

Tenant occupies the Premises. Coverage shall be maintained with limits of not less than $1,000,000 per incident with a $2,000,000 policy aggregate.

EXHIBIT C

ACKNOWLEDGEMENT OF TERM COMMENCEMENT DATE
AND TERM EXPIRATION DATE

THIS ACKNOWLEDGEMENT OF TERM COMMENCEMENT DATE AND TERM EXPIRATION DATE is entered into as of [_____], 20[__], with reference to that certain Lease (the "Lease") dated as of [_____], 2015, by REGENXBIO INC., a Delaware corporation ("Tenant"), in favor of BMR-MEDICAL CENTER DRIVE LLC, a Delaware limited liability company ("Landlord"). All capitalized terms used herein without definition shall have the meanings ascribed to them in the Lease.

Tenant hereby confirms the following:

1. Tenant accepted possession of the Premises for use in accordance with the Permitted Use on [_____], 20[__]. Tenant first occupied the Premises for the Permitted Use on [_____], 20[__].

2. To Tenant's knowledge, the Premises are in good order, condition and repair.

3. The Tenant Improvements are Substantially Complete.

4. To Tenant's knowledge, all conditions of the Lease to be performed by Landlord as a condition to the full effectiveness of the Lease have been satisfied, and Landlord has fulfilled all of its duties in the nature of inducements offered to Tenant to lease the Premises.

5. In accordance with the provisions of Article 4 of the Lease, the Term Commencement Date is [_____], 20[__], and, unless the Lease is terminated prior to the Term Expiration Date pursuant to its terms, the Term Expiration Date shall be [_____], 20[__].

6. The Lease is in full force and effect, and the same represents the entire agreement between Landlord and Tenant concerning the Premises[, except [_____]].

7. To Tenant's knowledge, Tenant has no existing defenses against the enforcement of the Lease by Landlord, and, to Tenant's knowledge, there exist no offsets or credits against Rent owed or to be owed by Tenant.

8. The obligation to pay Rent is presently in effect and all Rent obligations on the part of Tenant under the Lease commenced to accrue on [_____], 20[__], with Base Rent payable on the dates and amounts set forth in the chart below:

Dates	Approximate Square Feet of Rentable Area	Base Rent per Square Foot of Rentable Area	Monthly Base Rent	Annual Base Rent
[__]/[__]/[__]- [__]/[__]/[__]	[ ]	$[_____] annually	[ ]	[ ]

9. The undersigned Tenant has not made any prior assignment, transfer, hypothecation or pledge of the Lease or of the rents thereunder or sublease of the Premises or any portion thereof.

[REMAINDER OF THIS PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, Tenant has executed this Acknowledgment of Term Commencement Date and Term Expiration Date as of the date first written above.

TENANT:

REGENXBIO INC.,
a Delaware corporation

By: _____
Name: _____
Title: _____

EXHIBIT D

BILL OF SALE

This instrument (this "Bill of Sale") dated as of March 6, 2015, is executed by and between BMR-MEDICAL CENTER DRIVE LLC, a Delaware limited liability company ("Seller"), and REGENXBIO INC., a Delaware corporation ("Purchaser").

1. Sale of Personalty. As part of the consideration for Tenant's agreement to enter into that certain Lease of even date herewith between Seller, as Landlord, and Purchaser, as Tenant, for certain space in the building located at 9712 Medical Center Drive, Rockville, Maryland (the "Building"), Seller hereby transfers, sets over and conveys to Purchaser all right, title and interest of Seller in and to all tangible personal property described in Schedule A attached hereto (the "Personal Property") from the Building.

2. "As Is" Sale. Purchaser accepts the Personal Property in its condition "as is" and with all faults, patent or latent, as of the date hereof, and Seller hereby disclaims any warranties, related to the Personal Property, including, without limitation, warranties of merchantability and fitness for a particular purpose. Purchaser hereby acknowledges that Seller has not made, and does not make any representations or warranties, of any kind, express or implied, regarding the Personal Property, and Purchaser waives and releases any and all claims of any kind arising from the sale or condition of the Personal Property.

3. Successors and Assigns. This Bill of Sale is binding upon, and shall inure to the benefit of Seller and Purchaser and their respective heirs, legal representatives, successors and assigns.

4. Counterparts. This Bill of Sale may be executed in counterparts, each of which shall be deemed an original, but all of which, together, shall constitute one and the same instrument. Signature pages may be detached from the counterparts and attached to a single copy of this Bill of Sale to form one (1) document. A facsimile, electronic or portable document format (PDF) signature on this Bill of Sale shall be equivalent to, and have the same force and effect as, an original signature.

5. Governing Law. This Bill of Sale shall be governed by, interpreted under, and construed and enforceable in accordance with, the laws of the State of Maryland.

6. Attorneys' Fees. Should either party employ attorneys to enforce any of the provisions hereof, the substantially prevailing party shall be entitled to receive from the other party all reasonable costs, charges, and expenses, including reasonable attorneys' fees, expended or incurred by the substantially prevailing party in connection therewith.

IN WITNESS WHEREOF, the undersigned have caused this Bill of Sale to be executed as of the date written above.

SELLER:
BMR-MEDICAL CENTER DRIVE LLC,
a Delaware limited liability company

By: /s/ Kevin M. Simonsen
Name: Kevin M. Simonsen
Title: Sr. VP, Real Estate Legal

PURCHASER:
REGENXBIO INC.,
a Delaware corporation

By: /s/ Kenneth Mills
Name: Kenneth Mills
Title: President & CEO

SCHEDULE A

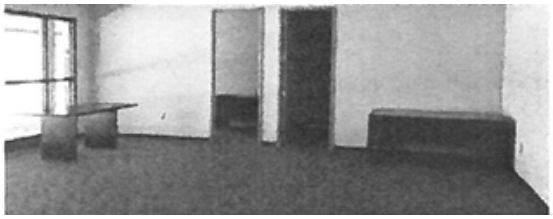
PERSONAL PROPERTY

(attached)

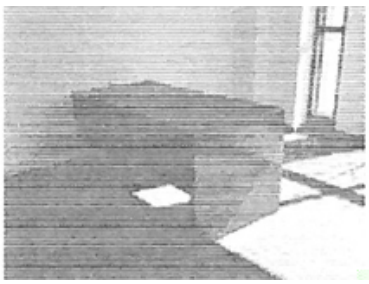
Personal Property List for 9712 Medical Center Drive

Kitchenette:
1 Round Kitchenette Table
8 Plastic and Metal Chairs
3 Plastic and Metal Highboy Chairs

Open Office Area:
1 Dark Wood Veneer Desk
1 Dark Wood Veneer Table

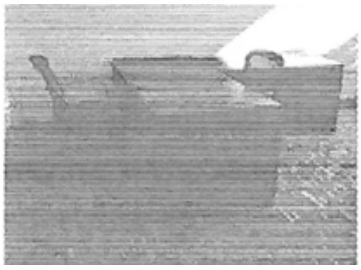


Offices:
Dark Wood Veneer Desk

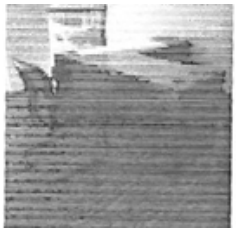


2 Dark Wood Veneer Bookcases
1 Dark Wood Desk and Return
1 Desk Chair

1 Dark Wood Color Desk and Return
2 Desk Chairs

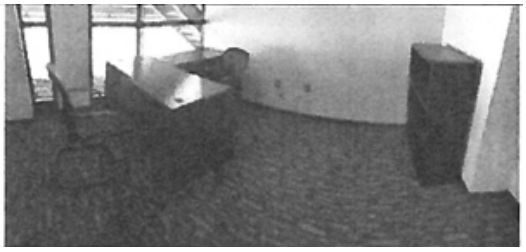


1 Dark Wood Color Desk and Return
1 Desk Chair

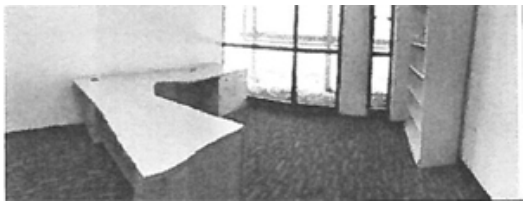


1 Dark Wood Desk and Return
2 Dark Wood Bookcases
1 Dark Wood Bilateral File Cabinet
1 Dark Wood Color Round Table
2 Black Leather Side Chairs

1 Dark Wood Color Desk and Return
1 Desk Chair
1 Black Desk Chair With Wheels
1 Dark Wood Color Bookcase

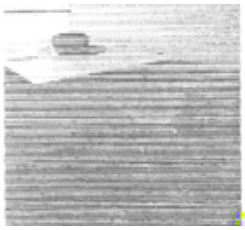


1 Light Wood Color Desk and Return
1 Tall Light Wood Bookcase

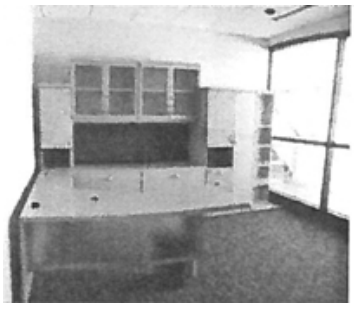


1 Light Wood Color Desk and Return
1 Tall Light Wood Bookcase
1 Black Desk Chair with Wheels

1 Light Wood Color Desk and Return
1 Desk Chair
1 Tall Light Wood Bookcase
2 Side Chairs

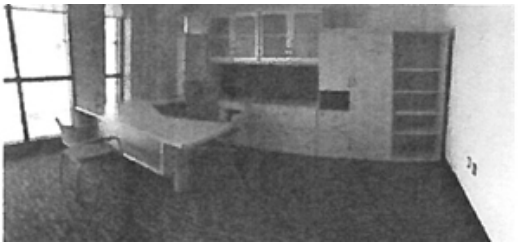


1 Light Wood Desk and Return with Attached Credenza
1 Light Wood Corner Bookshelf



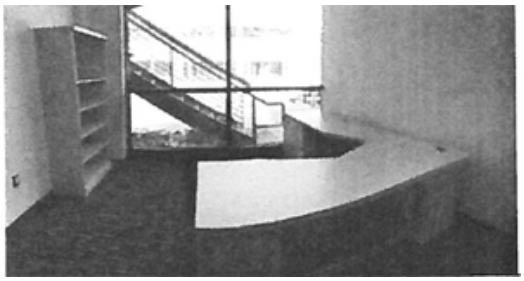
1 Light Wood Color Desk and Return
1 Tall Light Wood Bookcase

1 Side Chair
1 Executive Desk Chair
1 Light Wood Desk and Return with Attached Credenza
1 Light Wood Corner Bookshelf

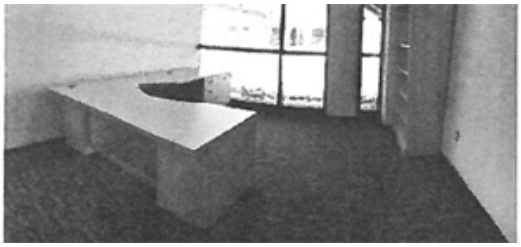


1 Executive Desk Chair
1 Light Wood Desk and Return with Attached Credenza
1 Light Wood Corner Bookshelf

1 Tall Light Wood Color Bookcase
1 Light Wood Desk and Return



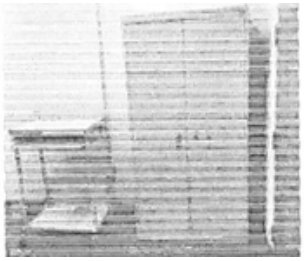
1 Tall Light Wood Color Bookcase
1 Light Wood Desk and Return



Small Conference Room :
4 Leather Side Chairs

1 Side Chair
1 Executive Desk Chair
1 Light Wood Desk and Return with Attached Credenza
1 Light Wood Credenza
1 Light Wood Corner Bookshelf

Filing Room:
1 Metal Computer Cart
1 Metal Filing Cabinet



Reception Area
1 Side Chair

Conference Room
1 Light Wood Conference Table With Double Utility Jacks
5 Executive Desk Chairs

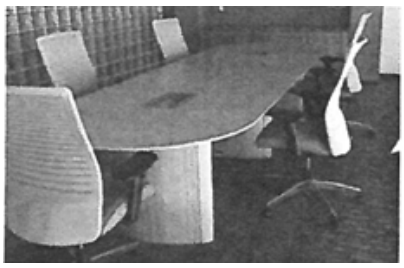


EXHIBIT E

FORM OF LETTER OF CREDIT

[On letterhead or L/C letterhead of Issuer]

LETTER OF CREDIT

Date: _____, 20__

_____ (the "Beneficiary")
_____
_____
Attention:_____
L/C. No.: _____
Loan No. :_____

Ladies and Gentlemen:

We establish in favor of Beneficiary our irrevocable and unconditional Letter of Credit numbered as identified above (the "L/C") for an aggregate amount of $_____, expiring at _____ :00 p.m. on _____ or, if such day is not a Banking Day, then the next succeeding Banking Day (such date, as extended from time to time, the "Expiry Date"). "Banking Day" means a weekday except a weekday when commercial banks in _____ are authorized or required to close.

We authorize Beneficiary to draw on us (the "Issuer") for the account of _____ (the "Account Party"), under the terms and conditions of this L/C.

Funds under this L/C are available by presenting the following documentation (the "Drawing Documentation"): (a) the original L/C and (b) a sight draft substantially in the form of Attachment 1, with blanks filled in and bracketed items provided as appropriate. No other evidence of authority, certificate, or documentation is required.

Drawing Documentation must be presented at Issuer's office at _____ on or before the Expiry Date by personal presentation, courier or messenger service, or fax. Presentation by fax shall be effective upon electronic confirmation of transmission as evidenced by a printed report from the sender's fax machine. After any fax presentation, but not as a condition to its effectiveness, Beneficiary shall with reasonable promptness deliver the original Drawing Documentation by any other means. Issuer will on request issue a receipt for Drawing Documentation.

We agree, irrevocably, and irrespective of any claim by any other person, to honor drafts drawn under and in conformity with this L/C, within the maximum amount of this L/C, presented to us on or before the Expiry Date, provided we also receive (on or before the Expiry Date) any other Drawing Documentation this L/C requires.

We shall pay this L/C only from our own funds by check or wire transfer, in compliance with the Drawing Documentation.

If Beneficiary presents proper Drawing Documentation to us on or before the Expiry Date, then we shall pay under this L/C at or before the following time (the "Payment Deadline"): (a) if presentment is made at or before noon of any Banking Day, then the close of such Banking Day; and (b) otherwise, the close of the next Banking Day. We waive any right to delay payment beyond the Payment Deadline. If we determine that Drawing Documentation is not proper, then we shall so advise Beneficiary in writing, specifying all grounds for our determination, within one Banking Day after the Payment Deadline.

Partial drawings are permitted. This L/C shall, except to the extent reduced thereby, survive any partial drawings.

We shall have no duty or right to inquire into the validity of or basis for any draw under this L/C or any Drawing Documentation. We waive any defense based on fraud or any claim of fraud.

The Expiry Date shall automatically be extended by one year (but never beyond _____ (the "Outside Date")) unless, on or before the date 90 days before any Expiry Date, we have given Beneficiary notice that the Expiry Date shall not be so extended (a "Nonrenewal Notice"). We shall promptly upon request confirm any extension of the Expiry Date under the preceding sentence by issuing an amendment to this L/C, but such an amendment is not required for the extension to be effective. We need not give any notice of the Outside Date.

Beneficiary may from time to time without charge transfer this L/C, in whole but not in part, to any transferee (the "Transferee"). Issuer shall look solely to Account Party for payment of any fee for any transfer of this L/C. Such payment is not a condition to any such transfer. Beneficiary or Transferee shall consummate such transfer by delivering to Issuer the original of this L/C and a Transfer Notice substantially in the form of Attachment 2, purportedly signed by Beneficiary, and designating Transferee. Issuer shall promptly reissue or amend this L/C in favor of Transferee as Beneficiary. Upon any transfer, all references to Beneficiary shall automatically refer to Transferee, who may then exercise all rights of Beneficiary. Issuer expressly consents to any transfers made from time to time in compliance with this paragraph.

Any notice to Beneficiary shall be in writing and delivered by hand with receipt acknowledged or by overnight delivery service such as FedEx (with proof of delivery) at the above address, or such other address as Beneficiary may specify by written notice to Issuer. A copy of any such notice shall also be delivered, as a condition to the effectiveness of such notice, to: _____ (or such replacement as Beneficiary designates from time to time by written notice).

No amendment that adversely affects Beneficiary shall be effective without Beneficiary's written consent.

This L/C is subject to and incorporates by reference: (a) the International Standby Practices 98 ("ISP 98"); and (b) to the extent not inconsistent with ISP 98, Article 5 of the Uniform Commercial Code of the State of New York.

Very truly yours,

[Issuer Signature]

ATTACHMENT 1 TO EXHIBIT E

FORM OF SIGHT DRAFT

[BENEFICIARY LETTERHEAD]

TO:

[Name and Address of Issuer]

SIGHT DRAFT

AT SIGHT, pay to the Order of _____, the sum of _____ United States Dollars ($_____). Drawn under [Issuer] Letter of Credit No. _____ dated _____.

[Issuer is hereby directed to pay the proceeds of this Sight Draft solely to the following account: _____.]

[Name and signature block, with signature or purported signature of Beneficiary]

Date: _____

ATTACHMENT 2 TO EXHIBIT E

FORM OF TRANSFER NOTICE

[BENEFICIARY LETTERHEAD]

TO:

[Name and Address of Issuer] (the "Issuer")

TRANSFER NOTICE

By signing below, the undersigned, Beneficiary (the "Beneficiary") under Issuer's Letter of Credit No. _____ dated _____ (the "L/C"), transfers the L/C to the following transferee (the "Transferee"):

[Transferee Name and Address]

The original L/C is enclosed. Beneficiary directs Issuer to reissue or amend the L/C in favor of Transferee as Beneficiary. Beneficiary represents and warrants that Beneficiary has not transferred, assigned, or encumbered the L/C or any interest in the L/C, which transfer, assignment, or encumbrance remains in effect.

[Name and signature block, with signature or purported signature of Beneficiary]

Date: _____

EXHIBIT F

RULES AND REGULATIONS

NOTHING IN THESE RULES AND REGULATIONS ("RULES AND REGULATIONS") SHALL SUPPLANT ANY PROVISION OF THE LEASE. IN THE EVENT OF A CONFLICT OR INCONSISTENCY BETWEEN THESE RULES AND REGULATIONS AND THE LEASE, THE LEASE SHALL PREVAIL.

1. No Tenant Party shall encumber or obstruct the common entrances, lobbies, elevators, sidewalks and stairways of the Building(s) or the Project or use them for any purposes other than ingress or egress to and from the Building(s) or the Project.

2. Except as specifically provided in the Lease, no sign, placard, picture, advertisement, name or notice shall be installed or displayed on any part of the outside of the Premises or the Building(s) without Landlord's prior written consent. Landlord shall have the right to remove, at Tenant's sole cost and expense and without notice, any sign installed or displayed in violation of this rule.

3. If Landlord objects in writing to any curtains, blinds, shades, screens, hanging plants or other similar objects attached to or used in connection with any window or door of the Premises or placed on any windowsill, and (a) such window, door or windowsill is visible from the exterior of the Premises and (b) such curtain, blind, shade, screen, hanging plant or other object is not included in plans approved by Landlord or is other than Landlord's standard window coverings, then Tenant shall promptly remove such curtains, blinds, shades, screens, hanging plants or other similar objects at its sole cost and expense.

4. No deliveries shall be made that impede or interfere with other tenants in or the operation of the Project. Movement of furniture, office equipment or any other large or bulky material(s) through the Common Area shall be restricted to such hours as Landlord may reasonably designate and shall be subject to reasonable restrictions that Landlord may impose.

5. Tenant shall not place a load upon any floor of the Premises that exceeds the load per square foot that (a) such floor was designed to carry or (b) is allowed by Applicable Laws. Fixtures and equipment that cause noises or vibrations that may be transmitted to the structure of the Building(s) to such a degree as to be objectionable to other tenants shall be placed and maintained by Tenant, at Tenant's sole cost and expense, on vibration eliminators or other devices sufficient to eliminate such noises and vibrations to levels reasonably acceptable to Landlord and the affected tenants of the Project.

6. Tenant shall not use any method of HVAC other than that approved in writing by Landlord or present at the Project and serving the Premises as of the Term Commencement Date.

7. Tenant shall not install any radio, television or other antennae; cell or other communications equipment; or other devices on the roof or exterior walls of the Premises except in accordance with the Lease. Tenant shall not interfere with radio, television or other digital or electronic communications at the Project or elsewhere.

8. Canvassing, peddling, soliciting and distributing handbills or any other written material within, on or around the Project (other than within the Premises) are prohibited. Tenant shall cooperate with Landlord to prevent such activities by any Tenant Party.

9. Tenant shall store all of its trash and garbage in receptacles within its Premises or in receptacles designated by Landlord outside of the Premises. Tenant shall not place in any such receptacle any material that cannot be disposed of in the ordinary and customary manner of trash and garbage disposal. Tenant shall be responsible, at its sole cost and expense, for Tenant's removal of its trash and garbage. Tenant is encouraged to participate in the waste removal and recycling program in place at the Project.

10. The Premises shall not be used for lodging or for any improper, immoral or objectionable purpose. No cooking shall be done or permitted in the Premises; provided, however, that Tenant may use (a) equipment approved in accordance with the requirements of insurance policies that Landlord or Tenant is required to purchase and maintain pursuant to the Lease for brewing coffee, tea, hot chocolate and similar beverages, (b) microwave ovens for employees' and invitees' use and (c) equipment shown on plans approved by Landlord; provided, further, that any such equipment and microwave ovens are used in accordance with Applicable Laws.

11. Tenant shall not, without Landlord's prior written consent, use the name of the Project, if any, in connection with or in promoting or advertising Tenant's business except as Tenant's address.

12. Tenant shall comply with all safety, fire protection and evacuation procedures and regulations established by any Governmental Authority or as reasonably established by Landlord on a Building-wide or Project-wide basis.

13. Tenant assumes any and all responsibility for protecting the Premises from theft, robbery and pilferage, which responsibility includes keeping doors locked and other means of entry to the Premises closed.

14. Tenant shall not modify any locks to the Premises without Landlord's prior written consent, which consent Landlord shall not unreasonably withhold, condition or delay. Tenant shall furnish Landlord with copies of keys, pass cards or similar devices for locks to the Premises.

15. Tenant shall cooperate and participate in all reasonable Building-wide or Project-wide security programs affecting the Premises.

16. Tenant shall not permit any animals in the Building, other than service animals.

17. Bicycles shall not be taken into the Building (including the elevators and stairways of the Building) except into areas designated by Landlord.

18. The water and wash closets and other plumbing fixtures shall not be used for any purposes other than those for which they were constructed, and no sweepings, rubbish, rags or other substances shall be deposited therein.

19. Discharge of industrial sewage shall only be permitted if Tenant, at its sole expense, first obtains all necessary permits and licenses therefor from all applicable Governmental Authorities.

20. Smoking is prohibited inside the Building, but is permitted in designated outdoor areas of the Project.

21. The Project's hours of operation are normal business hours of business days as defined in Section 16.9 of this Lease.

22. Tenant shall comply with all orders, requirements and conditions now or hereafter imposed by Applicable Laws or Landlord ("Waste Regulations") regarding the collection, sorting, separation and recycling of waste products, garbage, refuse and trash generated by Tenant (collectively, "Waste Products"), including (without limitation) the separation of Waste Products into receptacles reasonably approved by Landlord and the removal of such receptacles in accordance with any collection schedules prescribed by Waste Regulations.

23. Tenant, at Tenant's sole cost and expense, shall cause the Premises to be exterminated on a periodic basis (to the extent necessary) to Landlord's reasonable satisfaction and shall cause all portions of the Premises used for the storage, preparation, service or consumption of food or beverages to be cleaned daily in a manner reasonably satisfactory to Landlord, and to be treated against infestation by insects, rodents and other vermin and pests whenever there is evidence of any infestation. Tenant shall not permit any person to enter the Premises or the Project for the purpose of providing such extermination services, unless such persons have been approved by Landlord.

24. If Tenant desires to use any portion of the Common Area for a Tenant-related event, Tenant must notify Landlord in writing at least thirty (30) days prior to such event on the form attached as Attachment 1 to this Exhibit, which use shall be subject to Landlord's prior written consent, not to be unreasonably withheld, conditioned or delayed. Notwithstanding anything in this Lease or the completed and executed Attachment to the contrary, Tenant shall be solely responsible for setting up and taking down any equipment or other materials required for the event, and shall promptly pick up any litter and report any property damage to Landlord related to the event. Any use of the Common Area pursuant to this Section shall be subject to the provisions of Article 28 of the Lease.

Landlord may waive any one or more of these Rules and Regulations for the benefit of Tenant or any other tenant, but no such waiver by Landlord shall be construed as a waiver of such Rules and Regulations in favor of Tenant or any other tenant, nor prevent Landlord from thereafter enforcing any such Rules and Regulations against any or all of the tenants of the Project, including Tenant. These Rules and Regulations are in addition to, and shall not be construed to in any way modify or amend, in whole or in part, the terms covenants, agreements and conditions of the Lease. Landlord reserves the right to make such other and reasonable rules and regulations as, in its judgment, may from time to time be needed for safety and security, the care and cleanliness of the Project, or the preservation of good order therein; provided, however, that such additional rules and regulations shall be subject to Section 13.1 of the Lease. Tenant agrees to abide by these Rules and Regulations and any additional rules and regulations issued or

adopted by Landlord subject to Section 13.1 of the Lease. Tenant shall be responsible for the observance of these Rules and Regulations by all Tenant Parties.

ATTACHMENT 1 TO EXHIBIT F

REQUEST FOR USE OF COMMON AREA

[TENANT LETTERHEAD]

VIA [_____]

[Date]

BMR-MEDICAL CENTER DRIVE LLC
17190 Bernardo Center Drive
San Diego, California 92128
Attn: Senior Director, East Coast Operations

Re: Notice of Request to Use Common Area

To Whom It May Concern:

REGENXBIO Inc., requests that it have use of the common area as described below: _____

Event Description: _____

Date: _____

Location at Property: _____

Number of Attendees: _____

Open to the Public? [__] YES [__] NO

Food and/or Beverages? [__] YES [__] NO

If YES:

- will alcohol be served *(Note: Proof of an insurance endorsement for serving alcohol must be provided)* [__] YES [__] NO
- please describe: _____

Other Amenities (tent, band, etc.): _____

Other Event Details: _____

Please let us know at your earliest convenience whether such use is approved.

Sincerely,

[Name]
[Title]

To Be Completed by Landlord:

[__] APPROVED DENIED [__]

The following conditions apply to approval (if approved):

1. _____

2. _____

3. _____

4. _____

5. _____

BMR-MEDICAL CENTER DRIVE LLC

By: _____
Name: _____
Its: _____
Date: _____

EXHIBIT G

[Intentionally omitted]

EXHIBIT H

TENANT'S PROPERTY

EXHIBIT I

FORM OF ESTOPPEL CERTIFICATE

To: BMR-Medical Center Drive LLC
17190 Bernardo Center Drive
San Diego, California 92128
Attention: Vice President, Real Estate Legal

BioMed Realty, L.P.
17190 Bernardo Center Drive
San Diego, California 92128

Re: [PREMISES ADDRESS] (the "Premises") at 9712 Medical Center Drive, Rockville, Maryland (the "Property")

The undersigned tenant ("Tenant") hereby certifies to you as follows:

1. Tenant is a tenant at the Property under a lease (the "Lease") for the Premises dated as of [_____], 2015. The Lease has not been cancelled, modified, assigned, extended or amended [except as follows: [_____]], and there are no other agreements, written or oral, affecting or relating to Tenant's lease of the Premises or any other space at the Property. The lease term expires on [_____], 20[__].

2. Tenant took possession of the Premises, currently consisting of [_____] square feet, on [_____], 20[__], and commenced to pay rent on [_____], 20[__]. Tenant has full possession of the Premises, has not assigned the Lease or sublet any part of the Premises, and does not hold the Premises under an assignment or sublease[, except as follows: [_____]].

3. All base rent, rent escalations and additional rent under the Lease have been paid through [_____], 20[__]. There is no prepaid rent[, except $[_____]][, and the amount of security deposit is $[_____] [in cash][OR][in the form of a letter of credit]]. Tenant currently has no right to any future rent abatement under the Lease [except as follows: [_____]].

4. Base rent is currently payable in the amount of $[_____] per month.

5. Tenant is currently paying estimated payments of additional rent of $[_____] per month on account of real estate taxes, insurance, management fees and Common Area maintenance expenses.

6. All work to be performed for Tenant under the Lease has, to the actual knowledge of Tenant, been performed as required under the Lease and has been accepted by Tenant[, except [_____]], and all allowances to be paid to Tenant, including allowances for tenant improvements, moving expenses or other items, have been paid.

7. The Lease is in full force and effect, and to Tenant's knowledge, is free from default and free from any event that could become a default under the Lease, and to Tenant's knowledge, Tenant has no claims against the landlord or offsets or defenses against rent, and to Tenant's

knowledge, there are no disputes with the landlord. Tenant has received no notice of prior sale, transfer, assignment, hypothecation or pledge of the Lease or of the rents payable thereunder[, except [_____]].

8. [Tenant has the following expansion rights or options for the Property: [_____].][OR][Tenant has no rights or options to purchase the Property.]

9. To Tenant's knowledge, no Hazardous Materials have been generated, treated, stored or disposed of by or on behalf of Tenant in, on or around the Premises or the Project in violation of any Applicable Laws.

10. The undersigned has executed this Estoppel Certificate with the knowledge and understanding that [INSERT NAME OF LANDLORD, PURCHASER OR LENDER, AS APPROPRIATE] or its assignee is [acquiring the Property/making a loan secured by the Property] in reliance on this certificate and that the undersigned shall be bound by this certificate. The statements contained herein may be relied upon by [INSERT NAME OF PURCHASER OR LENDER, AS APPROPRIATE], [LANDLORD], BioMed Realty, L.P., BioMed Realty Trust, Inc., and any [other] mortgagee of the Property and their respective successors and assigns.

Any capitalized terms not defined herein shall have the respective meanings given in the Lease.

Dated this [____] day of [_____], 20[__].

[_____],

a[_____]

By: _____
Name: _____
Title: _____

EXHIBIT J

[Intentionally omitted]

EXHIBIT K

JANITORIAL SPECIFICATIONS

Office Areas

- Dusting unobstructed furniture, office equipment, window sills etc. - weekly
- Non-carpeted floors dust mopped, i.e. under desks & furniture - daily
- Tile floors (i.e. kitchen areas) wet moped - daily
- Trash cans/recycle cans emptied (plastic lines replaced where applicable) - daily
- Vacuuming all carpeted areas - daily
- Spot cleaning of carpeted areas - as necessary
- Spot clean all glass surfaces - weekly
- High dusting of ledges, moldings, picture frames etc. - quarterly
- Window blinds to be dusted - quarterly
- Areas around air conditioning grills/return ducts cleaned - monthly
- Kitchen (if any) - all horizontal surfaces cleaned & sanitize - daily

Restroom Cleaning

- Sweep floors - daily
- Damp mop floors - daily
- Dust light fixtures & air vents - as needed but not less than weekly
- Clean toilets/urinals and flush to remove water stains - daily
- Clean doors and other surfaces - daily
- Sanitize & spot clean walls & partitions - daily
- Remove any and all trash - daily
- Supply paper products, hand soap & refill as necessary
- Clean and dry countertops, clean out sink basins - daily
- Clean all mirrors - daily

CONFIDENTIAL TREATMENT REQUESTED

DEVELOPMENT, MANUFACTURING, AND TESTING STANDARD TERMS AND CONDITIONS

This Development, Manufacturing and Testing Standard Terms and Conditions together with any Work Orders attached hereto is made and entered into as of April 3, 2015 (Effective Date) by and between WuXi AppTec, Inc., a corporation organized under the laws of Delaware ("WuXi AppTec"), and REGENXBIO Inc., a corporation organized under the laws of Delaware (Customer), Customer and WuXi AppTec are referred to herein individually as a "Party" and collectively as the "Parties".

The Parties agree as follows:

1. Definitions

1.1 Defined Terms. The following terms (whether or not underscored) when used in this Agreement, shall, except where the context otherwise requires, have the following meanings:

1.1.1 "Affiliate" means any company, partnership or other entity which directly or indirectly controls, Is controlled by or is under common control with the relevant party to this Agreement. "Control" means the ownership of at least fifty per cent (50%) of the equity of the entity or the legal power to direct the general management and policies of the entity.

1.1.2 "Agreement' means these Terms and Conditions together with an applicable work order.

1.1.3 "Batch" means the total Product obtained from one run for cell growth at scale from cell culture flasks, hyperstacks, bioreactor or other device and associated purification using the Process and carried out in accordance with cGMP or non cGMP if so identified in the Work Order.

1.1.4 "Cell Line" means the cell line used to produce Product, particulars of which are set out in Work Orders.

1.1.5 "Certificate of Analysis" means a certificate of analysis as to testing of Specifications of any Product in form and substance agreed to by WuXi AppTec and Customer,

1.1.6 "cGMP" means current Good Manufacturing Practices and General Biologics Products Standards as promulgated under the US Federal Food Drug and Cosmetic Act at 21 CFR (Chapters 210, 211, 600 and 610), the Guide to Good Manufacturing Practices for Medicinal Products as promulgated under European Directive 91/356/EEC and ICH Guidance Q7A (Good Manufacturing Practice Guidance for Active Pharmaceutical Ingredients. WuXi AppTec's operational quality standards are defined in internal GMP policy documents.

1.1.7 "cGMP Product" means Product which may be required under Work Orders to be manufactured in accordance with cGMP.

1.1.8 "Customer" means REGENXBIO Inc., Inc. and its successors and assigns.

1.1.9 "Customer Information" means all technical and other information from time to time supplied by Customer to WuXi AppTec, which at the time of supply by Customer is not (i) already in the public domain or (ii) already known by WuXi AppTec at the time of disclosure as established by written records.

1.1.10 "Customer Know-How" means all technical and other information relating to the Product or the Process known to Customer from time to time other than WuXi AppTec Know-How and information in the public domain.

1.1.11 "Customer Materials" means the materials supplied by Customer to WuXi AppTec and identified as such in Work Orders hereto.

1.1.12 "Customer Patent Rights" means all patents and patent applications of any kind throughout the world that are necessary or useful in performance of the Services, or related to the Products or the Process, which from time to time Customer is the owner of or is entitled to use.

1.1.13 "Deliver," "Delivered" or "Delivery" has the meaning ascribed to it by Section 5.1.

1.1.14 "Price" means the price specified in Work Orders for the Services.

1.1.15 "Process" means the process for the production and purification of the Product from the Cell Line and the virus bank or plasmids to generate viral or vector product(s), including any improvements or modifications thereto from time to time.

1.1.16 "Product" means all or any part of the product manufactured (including any sample thereof), particulars of which are set out in Work Orders and includes all derivatives thereof.

1.1.17 "Services" means all or any part of the services that are the subject of the Agreement, particulars of which are set out in Work Orders.

1.1.18 "Specification" means the specification for Product or Services, as applicable, particulars of which are set out in Work Orders.

1.1.19 "Terms of Payment" means the terms of payment specified in Work Orders.

1.1.20 "Testing Laboratories" means any third party instructed to carry out tests on the Cell Line or the Product

1.1.21 "Work Order" means any such appendix to this Agreement specifying Services. Work Order(s) shall be attached to this Agreement and shall when approved in writing by both Parties be deemed an integral part hereof. Work Order(s) may be updated from time to time by mutual agreement.

1.1.22 "WuXi AppTec" means WuXi AppTec, Inc. and its successors and assigns.

1.1.23 "'WuXi AppTec Know-How" means all technical and other information and materials, ideas, concepts, methods, procedures, designs, documents, data, inventions, discoveries and works of authorship (in each case, whether or not patentable) known to WuXi AppTec from time to time other than confidential Customer Information and information in the public domain.

1.1.24 "WuXi AppTec Patent Rights" means all patents and patent applications of any kind throughout the world relating to WuXi AppTec Know-How or to the Process which from time to time WuXi AppTec is the owner of or is entitled to use

1.2 Use of Definitions. Unless the context requires otherwise, words and phrases defined in any other part of the Agreement shall bear the same meanings in these Standard Terms and

Conditions, references to the singular number include the plural and vice versa, references to Work Orders are references to work orders to the Agreement, and references to Sections are references to sections of these Standard Terms and Conditions.

1.3 Conflicting Definitions. In the event of a conflict between a term in any executed Work Order or any supplemental or additional term agreed to in writing from time to time between the parties and these Standard Terms and Conditions, any Work Order and any supplemental or additional term agreed to in writing after the date hereof shall prevail.

2. Applicability of Standard Terms and Conditions

These Terms and Conditions will not be effective until it (or a counterpart of it) has been signed on behalf of both Parties. Customer and WuXi AppTec must complete and execute a Work Order before Services are provided. Each Work Order will include information relating to the specific Services agreed to by the Parties and price for Services. Once signed, a Work Order becomes a part of the Agreement, although the terms in a Work Order will govern only Services described in that Work Order. To initiate the provision of Services under a Work Order, Customer must issue a purchase order. Neither a Work Order nor a purchase order will change any term in the Agreement. In the event of any inconsistency between the Agreement and any Work Order, the Agreement will prevail. No variation of or addition to the Agreement or any part thereof shall be effective unless in writing and signed on behalf of both Parties. Notwithstanding the above, the Parties hereby confirm that amendments to the Specification shall be effective if reduced to writing and signed by the quality and/or regulatory representative of both Parties, which quality and/or regulatory representative shall be nominated from time to time by each Party. Any such amendments to Specifications must also reflect, in writing, any corresponding changes to the timing of the Services and any changes to the Pricing detailed in the applicable Work Order.

3. Representations and Warranties

3.1 WuXi AppTec Warranties. WuXi AppTec represents and warrants that:

3.1.1 The Services will be performed in accordance with the Terms and Conditions of this Agreement;

3.1.2 It will use reasonable endeavors to keep the Cell Line and virus/vector plasmids or banks (if applicable) and/or other Customer Materials and/or the Customer Know-How secure and safe from loss and damage in such manner as WuXi AppTec stores its own material of similar nature;

3.1.3 It will not part with possession of the. Cell Line and virus/vector plasmids or banks (if applicable) and/or other Customer Materials or the Product, save for the purpose of tests at any third party Testing Laboratories that may be required and only with Customer’s written permission; and

3.1.4 It will use only Testing Laboratories bound to obligations of confidence substantially similar to those obligations of confidence imposed on WuXi AppTec under these Standard Terms and Conditions.

3.1.5 Subject to Section 13, unencumbered title to Product will be conveyed to Customer upon Delivery;

3.1.6 As of the date of this Agreement, to the best of WuXi AppTec’s knowledge without independent investigation, the WuXi AppTec Patent Rights and the WuXi AppTec Know-How are owned by WuXi AppTec or WuXi AppTec is otherwise entitled to use them for the purposes of providing Services under this Agreement and during the term of this Agreement, WuXi

AppTec shall not do or cause anything to be done which would adversely affect their ownership or entitlement to use the same for those purposes;

3.1.7 WuXi AppTec has the necessary corporate authorizations to enter into this Agreement;

3.1.8 As of the date of this Agreement to the best of WuXi AppTec's knowledge without independent investigation, the use by WuXi AppTec of the Process (excluding any modifications or steps made or developed by Customer, Customer Materials, Customer Information and Customer Patent Rights) and WuXi AppTec Patent Rights and WuXi AppTec Know-How for the performance of the Services as provided herein will not infringe any rights (including without limitation any intellectual or industrial property rights) vested in any third party;

3.1.9 WuXi AppTec will notify Customer in writing immediately if it receives or is notified of a claim from a third party that the use by WuXi AppTec of the Process and/or the WuXi AppTec Know-How or the WuXi AppTec Patents Rights for Services infringes any intellectual property rights vested in such third party;

3.2 DISCLAIMER. SECTION 3.1 IS IN LIEU OF ALL CONDITIONS, WARRANTIES AND STATEMENTS IN RESPECT OF THE SERVICES AND/OR THE PRODUCT WHETHER EXPRESSED OR IMPLIED BY STATUTE, CUSTOM OF THE TRADE OR OTHERWISE (INCLUDING BUT WITHOUT LIMITATION ANY SUCH CONDITION, WARRANTY OR STATEMENT RELATING TO THE DESCRIPTION OR QUALITY OF THE PRODUCT, ITS FITNESS OR SUITABILITY FOR A PARTICULAR PURPOSE OR USE UNDER ANY CONDITIONS WHETHER OR NOT KNOWN TO WUXI APPTEC) AND ANY SUCH CONDITION, WARRANTY OR STATEMENT IS HEREBY EXCLUDED AND DISCLAIMED.

3.3 Representations and Warranties of Customer. Customer represents and warrants to WuXi AppTec that:

3.3.1 Customer shall supply to WuXi AppTec the Customer Information, together with full details of any hazards relating to the Cell Line and virus/vector plasmids or banks (if applicable) and/or other Customer Materials, their storage and use. Upon review of this Customer Information, the Cell Line and virus/vector plasmids or banks (if applicable) and/or other Customer Materials and/or the Customer Know-How will be provided to WuXi AppTec at WuXi AppTec's reasonable request. The Cell Line Line and virus/vector plasmids or banks (if applicable) and/or other Customer Materials and/or the Customer Information and/or the Customer Know-How supplied to WuXi AppTec will remain the property of Customer.

3.3.2 Customer hereby grants WuXi AppTec the non-exclusive right to use the Cell Line, the virus/vector plasmids or banks (if applicable) and/or other Customer Materials, the Customer Know-How and the Customer Information for the purpose of the Agreement. WuXi AppTec hereby undertakes not to use the Cell Line, virus/vector plasmids or banks (if applicable) and/or other Customer Materials, the Customer Know-How or the Customer Information (or any part thereof) for any other purpose.

3.3.3 Customer has the necessary corporate authority to enter into this Agreement;

3.3.4 To Customer's knowledge without independent investigation, as of the date of this Agreement, Customer has the right to supply the Cell Line, and virus/vector plasmids or banks (if applicable) and/or other Customer Materials and the Customer Information to WuXi AppTec and the necessary rights to license or permit WuXi AppTec to use the same for the purpose of the Services; and Customer shall not do or cause anything to be done which would adversely affect their ownership or entitlement to use the same for those purposes;

3.3.5 To Customer's knowledge and belief without independent investigation, as of the date of this Agreement, the use by WuXi AppTec of the Cell Line, virus/vector plasmids or banks (if applicable) and/or other Customer Materials, Customer Information and Customer Patent Rights for the Services (including without limitation the manufacture of the Product) will not infringe any intellectual property rights of any third party; and Customer shall not do or cause anything to be done which would adversely affect such use;

3.3.6 Customer will promptly notify WuXi AppTec in writing if it receives or is notified of a claim from a third party that the Cell Line, and virus/vector plasmids or banks (if applicable) and/or other Customer Materials, Customer Information or the Customer Patent Rights or that the-use by WuXi AppTec thereof for the provision of the Services infringes any intellectual property rights of such third party;

4. Provision of the Services

4.1 Services. WuXi AppTec shall carry out the Services as provided in applicable Work Orders and shall use reasonable efforts to achieve the estimated time schedule thereto or as agreed to by the Parties.

4.2 Specification. Specifications will be agreed to by the Parties prior to initiation of a manufacturing run or other Services, as appropriate.

4.3 Time Limitations. Due to the unpredictable nature of the technical transfer, Process development and assay development Services the time schedule set down for the performance of these Services is estimated only. Upon completion or near completion of these Services the project teams will work to finalize the pilot or engineering and cGMP manufacturing schedules.

5. Delivery, Transportation of Product

5.1 Delivery. Product will be delivered Ex Works WuXi AppTec premises which means (a) when WuXi AppTec places Product at the disposal of Customer at WuXi AppTec's premises and (b) risk and title to Product pass to Customer upon delivery ("Deliver," "Delivery," or "Delivered," as appropriate). Subject to Section 5.2, WuXi AppTec shall deliver to Customer the Certificate of Analysis not later than the date of Delivery. Transportation of Product, whether or not under any arrangements made by WuXi AppTec on behalf of Customer, shall be made at the sole risk and expense of Customer.

5.2 Delivery Without Certificate of Analysis. At Customer's request, WuXi AppTec will Deliver Product in quarantine prior to delivery of the Certificate of Analysis. Such request shall be accompanied by Customer's written acknowledgement that the Product has been Delivered without the transmittal to Customer of a Certificate of Analysis, that accordingly the Product cannot be administered to humans until transmittal of the Certificate of Analysis, and that Customer nevertheless accepts full risk of loss, title and ownership of the Product. The Delivery of Product in quarantine will be subject to such testing requirements as WuXi AppTec may reasonably require, and the **** period referred to in Section 5.8 will run from Delivery in quarantine by Customer of the Product.

5.3 Packaging and Labeling. Unless otherwise agreed, WuXi AppTec shall package and label Product for Delivery in accordance with its standard operating procedures and in accordance with required shipping conditions. It shall be the responsibility of Customer to inform WuXi AppTec in writing in advance of any special packaging and labeling requirements for Product. All additional costs and expenses of whatever nature incurred by WuXi AppTec in complying with such special requirements must be agreed to in advance in writing and will be charged to Customer in addition to the Price.

5.4 Insurance. If requested in writing by Customer, WuXi AppTec will (acting as agent for Customer) arrange for insurance of Product while held by WuXi AppTec after Delivery

(awaiting transportation) for a maximum of **** on terms equivalent to those under which WuXi AppTec insures product prior to Delivery. Third party expenses incurred by WuXi AppTec in arranging such insurance must be agreed to in advance in writing and will be charged to Customer in addition to the Price.

5.5 Transportation. If requested in writing by Customer, WuXi AppTec will (acting as agent of Customer for such purpose) arrange the transportation of Product from WuXi AppTec's premises to the destination indicated by Customer together with insurance coverage for Product in transit at its invoiced value. All additional costs and expenses of whatever nature incurred by WuXi AppTec in arranging such transportation and insurance must be agreed to in advance in writing and will be charged to Customer in addition to the Price.

5.6 Acceptance of Delivery. Where WuXi AppTec has made arrangements for the transportation of Product, Customer shall diligently examine the Product as soon as practicable after receipt. Notice of all claims (time being of the essence) arising out of:

5.6.1 Visible damage to or total or partial loss of Product in transit will be given in writing to WuXi AppTec and the carrier within **** of receipt by Customer; or

5.6.2 Non-delivery will be given in writing to WuXi AppTec within **** after the receipt by Customer of WuXi AppTec's dispatch notice,

5.7 Damage Claims. Customer shall make damaged Product and associated packaging materials available for inspection and shall comply with the reasonable requirements of any insurance policy covering the Product, for which notification has been given by WuXi AppTec to Customer. WuXi AppTec shall offer Customer all reasonable assistance in pursuing any claims arising out of the transportation of Product.

6. Non-Conforming Product or Services

6.1 Non-conforming Product. Promptly following Delivery of Product, Customer may carry out any of the tests outlined or referred to in the Specifications. If such tests show that the Product fails to meet Specification, Customer shall give WuXi AppTec written notice thereof within **** from the date of Delivery and shall return such Product to WuXi AppTec's premises, at WuXi AppTec's expense, for further testing. In the absence of such written notice, Product shall be deemed to have been accepted by Customer as meeting Specification. If Product returned to WuXi AppTec fails to meet Specification and such failure is due to the negligence of Wuxi AppTec, WuXi AppTec shall refund that part of the Price that relates to the production of such Product or initiate a manufacturing run to replace such Product at its own cost and expense.

6.2 Non-Conforming Testing Services. If, within **** of receiving a valid result from testing Services, Customer notifies WuXi AppTec in writing that the result is unexpected, WuXi AppTec will initiate a laboratory investigation of the result, The Customer and WuXi AppTec will agree on an appropriate course of action pending the results of the laboratory investigation. If WuXi AppTec observes an Out Of Specification (OOS) result it will notify Customer as soon as reasonable but in any case within **** of learning of such result. Customer and WuXi AppTec will agree on the appropriate course of action to investigate the OOS result. If WuXi AppTec determines that an unexpected, invalid, or OOS result is due to the inherent condition of the sample matrix, or to the act, omission, direction, or negligence of Customer, Customer shall be liable to WuXi AppTec for the price of the Services performed, including any additional testing or retests, and materials, reagents, expenses consumed, employed, or specially obtained during the course of the laboratory investigation. If the unexpected, invalid, or OOS result was caused by a combination of the inherent property of the sample matrix or the act, omission, direction, or negligence of Customer and WuXi AppTec error, or a reasonable determination of cause cannot be ascertained, Customer shall be liable for **** of the price of the Services performed, including any additional testing or retests, and **** of the cost of any materials or reagents specially obtained by WuXi AppTec during the course of the laboratory

investigation. Customer is not liable to WuXi AppTec for unexpected, invalid, or OOS results due to WuXi AppTec error and WuXi AppTec shall make a refund of any payments made by Customer for the Services giving rise to the unexpected, invalid, or OOS results. Should Customer request a repeat or retest of such non-conforming Services Customer shall be liable for the price of a successful repeat or retest of such non-conforming Services.

7. Records

Records of Services are available for Customer review at the WuXi AppTec facility where the Services were performed. WuXi AppTec will retain batch, laboratory and other technical records ("Records") of Services for the longer of **** or for the minimum period required by applicable law and consistent with FDA regulations and guidance relating to the manufacture or testing of products intended to support an application for regulatory approval. To the extent that raw data from Services or descriptions of any of WuXi AppTec's protocols, test methods, or SOPS are not included in the Customer-approved protocol Work Order, or Report pertaining to any particular Service and are required by a competent regulatory authority, WuXi AppTec will upon written request by Customer provide a copy of such raw data or relevant portions of such protocols, test methods, or SOPs to be used solely for purposes of such regulatory submission under the provisions of Confidentiality described in the following section. In the event WuXi AppTec proposes to dispose of Records WuXi AppTec shall provide Customer written notice thereof. If within **** after such notice Customer requests any Records, WuXi AppTec shall provide to Customer at Customer's expense such Records rather than disposing thereof. WuXi AppTec may, however, retain copies of any Records as are reasonably necessary for regulatory or insurance purposes, subject to WuXi AppTec's obligation of confidentiality.

8. Price and Terms of Payment

8.1 Price. Customer shall pay the Price in accordance with the Price detailed in Work Orders attached hereto.

8.2 Payment. Payment will be made in accordance with Work Orders attached hereto. Unless otherwise indicated in a Work Order, all prices and charges are exclusive of any applicable taxes, levies, duties and fees of whatever nature imposed by or under the authority of any government or public authority, which shall be paid by Customer (other than taxes on WuXi AppTec's income). Payment must be made within **** of receipt by Customer of a correct invoice. Payment shall be made without deduction, deferment, set-off, lien or counterclaim of any nature.

8.3 Payment Default. In the event of a default of payment on due date:

8.3.1 Interest shall accrue on any amount overdue at the annual rate of **** above the prime rate of interest published from time to time in the Wall Street Journal, interest to accrue on a day to day basis both before and after judgment; and

8.3.2 WuXi AppTec shall, at its sole discretion, and without prejudice to any other of its accrued rights, be entitled to suspend the provision of the Services or to treat the Agreement as repudiated on not less than **** prior notice in writing to Customer given at any time after the due date.

9. Indemnification and Limitation of Liability

9.1 WuXi AppTec Indemnity. WuXi AppTec shall indemnify and hold Customer harmless against all claims, actions, costs, expenses (including court costs and reasonable attorney's fees) or other liabilities (collectively, "Losses") whatsoever to, from or in favor of third parties, to the extent such Losses are in respect of WuXi AppTec's ****.

9.2 Customer Indemnity. Customer shall indemnify and hold WuXi AppTec harmless against all Losses whatsoever to, from or in favor of third parties, to the extent such Losses are in respect of Customer's ****.

9.3 Limitation. Except for the above indemnification obligations, neither Party shall be liable for any penalties, liquidated, special, consequential, incidental or indirect damages arising out of or in connection with this Agreement (or the termination hereof), including, without limitation, loss of profits or anticipated sales to the fullest extent permitted by law, and the total liability, in the aggregate, of either Party and its agents to the other Party and anyone claiming by or through the other Party, for any and all claims, losses, costs or damages, including without limitation, attorneys' fees and costs and expert-witness fees and costs of any nature whatsoever or claims expenses resulting from or in any way related to this Agreement from any cause or causes shall not exceed the fees paid or owed under this Agreement for the portion of the Services under which such liability arises. Except as otherwise provided, it is intended that this limitation apply to any and all liability or cause of action however alleged or arising, including without limitation, negligence, professional errors and omissions, breach of contract, unless otherwise prohibited by law. For the avoidance of doubt, the foregoing shall not limit either Party's ability to obtain equitable relief of any type.

9.4 Further Limitation. The obligation of WuXi AppTec under Section 9.1 and Customer under Section 9.2 is limited to **** per claim, except that this limitation will not apply with respect to any indemnifiable claim arising out of or relating to fraud or willful misconduct by the indemnifying Party under this Agreement Except for claims arising under indemnities contained herein, any claim must be brought by either Party within **** from the completion of Services under which such claim arises or such claim will be forever barred.

9.5 Limitation Exception. Nothing contained in these Standard Terms and Conditions shall purport to exclude or restrict any liability for death or personal injury resulting directly from gross negligence by a Party in carrying out their obligations in breach of the terms of this Agreement.

9.6 Survival. The obligations of WuXi AppTec and Customer and under this Section 9 shall survive the termination or expiration of this Agreement.

10. Confidentiality

10.1 Confidential Information. The Parties will exchange proprietary and confidential information during the term of this Agreement, including without limitation, the existence and terms of this Agreement. The parties will identify, in writing, such information as confidential and/or proprietary. Notwithstanding the foregoing, Customer Confidential Information will also include Customer Information, Customer Materials, and Customer Know-How, and WuXi AppTec Confidential Information will include WuXi AppTec Know-How, development and manufacturing processing know-how, study designs, pricing information, and test protocols. Customer acknowledges that WuXi AppTec Confidential Information and WuXi AppTec acknowledges that Customer Confidential Information, with which it is supplied by the other pursuant to the Agreement is supplied subject to Sections 10.5 and 10.6 in circumstances imparting an obligation of confidence. Each Party agrees to keep the other Party's confidential information secret and confidential and to respect the other's proprietary rights therein and not at any time for any reason whatsoever to disclose or permit the other party's confidential information to be disclosed to any third party save as expressly provided herein.

10.2 Obligations of Confidentiality. Customer and WuXi AppTec shall each cause all their respective employees, consultants, contractors and persons for whom it is responsible having access to WuXi AppTec Confidential Information or Customer Confidential Information to be subject to the same obligations of confidence as Customer and WuXi AppTec pursuant to Sections 10.1 and 10.3 and shall be bound by confidentiality agreements in support of such obligations. WuXi AppTec and

Customer each undertake not to disclose or permit to be disclosed to any third party, or otherwise make use of or permit to be made use of (a) any trade secrets or confidential information relating to the technology, business affairs or finances of the other, any subsidiary, holding company or subsidiary or any such holding company of the other, or of any suppliers, agents, distributors, licensees or other customers of the other which comes into its possession under this Agreement, or (b) the commercial terms of this Agreement; except to the extent that the same is required to be disclosed pursuant tosubpoena, court order, judicial process or otherwise by law, provided the receiving party provides prompt notice to the disclosing Party of such requirement in order to give the disclosing party an opportunity to timely seek a protective order or other appropriate judicial relief. In the event the disclosing Party is unable to obtain a protective order or other appropriate judicial relief, the receiving party shall disclose only that portion of the disclosing Party's confidential information which is legally required to be disclosed, and that the disclosing party shall be given an opportunity to review the confidential information prior to its disclosure.

10.3 Limitations. The obligations of confidentiality referred to in this Section 9 shall not extend to any information which:

10.3.1 Is or becomes generally available to the public otherwise than by reason of a breach by the recipient Party of the provisions of this Section 10;

10.3.2 Is known to the receiving Party and is at its free disposal prior to its receipt from he disclosing Party, as established by written records;

10.3.3 Is subsequently disclosed to the receiving Party without being made subject to an obligation of confidence by a third party, as established by written records;

10.3.4 Is required to be disclosed by WuXi AppTec or Customer under any statutory, regulatory or similar legislative requirement, subject to the imposition of obligations of confidentiality wherever possible in that relation; or

10.3.5 Is developed by any servant or agent of the recipient Party without access to or use or knowledge of the information by the disclosing party, as established by written records.

10.4 Remedies. Without prejudice to any other rights and remedies that the Parties may have, the Parties agree that the confidential information is valuable and that damages may not be an adequate remedy for any breach of the provisions of Sections 10.1, 10.2, or 10.3. The Parties agree that the relevant party will be entitled without proof of special damage to seek the remedies of an injunction and other equitable relief for any actual or threatened breach by the other Party,

10.5 WuXi AppTec Confidential Information. Customer acknowledges that Customer shall not at any time have any right, title, license or interest in or to WuXi AppTec Confidential Information the WuXi AppTec Patent Rights or any other intellectual property rights relating to the Services which are vested in WuXi AppTec or to which WuXi AppTec is otherwise entitled.

10.6 Customer Confidential Information. WuXi AppTec acknowledges that save as provided herein WuXi AppTec shall not at any time have any right, title, license or interest in or to the Customer Confidential Information, Customer Patent Rights, Customer Know-How, or any other intellectual property rights vested in Customer or to which Customer is entitled.

10.7 Survival. The obligations of WuXi AppTec and Customer under this Section 10 shall survive the termination or expiration of this Agreement.

11. Term and Termination

11.1 Term. This Agreement will expire on the later of (a) two (2) years from the Effective Date or (b) the completion of all Services under the last Work Order executed by the Parties prior to the second anniversary of the Effective Date. The Agreement may be extended by mutual agreement of the parties or earlier terminated in accordance with Section 11.2. or 11.3.

11.2 Termination without Cause.

11.2.1 Customer may in its sole discretion terminate this Agreement or Work Order at any time for any reason or no reason by giving not less than **** notice in writing to WuXi AppTec. In the event of termination pursuant to this Section 10.2.1 Customer shall pay WuXi AppTec for Services performed up to the date of termination, and any applicable cancellation fees that the Parties have agreed to in the applicable Statement of Work. In addition, Customer shall reimburse WuXi AppTec for expenses incurred or irrevocably committed to third parties in accordance with this Agreement and the Price for any cell banks, toxicology studies, or manufacturing Batches that are in-progress.

11.2.2 WuXi AppTec may in its sole discretion terminate this Agreement or any Work Order at any time for any reason or no reason by giving not less than **** notice in writing to Customer. During such notice period, WuXi AppTec shall continue all work in progress and both Parties shall remain liable to each other for their respective obligations under this Agreement. In the event of termination pursuant to this Section 11.2.2 Customer shall pay WuXi AppTec for Services performed and for expenses incurred or irrevocably committed to third parties.

11.3 Termination for Cause. WuXi AppTec and Customer may each terminate the Agreement forthwith by notice in writing to the other upon the occurrence of any of the following events:

11.3.1 If the other commits a material breach of the Agreement which in the case of a breach capable of remedy is not remedied to the reasonable satisfaction of the non-breaching Party within **** of the receipt by the other of written notice identifying the breach and requiring its remedy; or

11.3.2 Any party may terminate this Agreement at any time by giving notice in writing to the other party, if the other party files a petition of any type as to its bankruptcy, is declared bankrupt, becomes insolvent makes an assignment for the benefit of creditors, goes into liquidation or receivership, otherwise loses legal control of its business or ceases to carry on its business.

11.4 Rights and Obligations upon Termination. Upon the termination of the Agreement for whatever reason:

11.4.1 Subject to Section 7, WuXi AppTec shall promptly return to Customer all Customer Know-How, Customer Information and shall dispose of or return to Customer the Customer Materials (and where supplied by Customer the Cell Line) and any materials therefrom, as directed by Customer;

11.4.2 Customer shall promptly return to WuXi AppTec all WuXi AppTec Know-How and WuXi AppTec Confidential Information it has received from WuXi AppTec;

11.4.3 Customer shall not thereafter use or exploit WuXi AppTec Confidential Information, the WuXi AppTec Patent Rights or the WuXi AppTec Know-How in any way whatsoever;

11.4.4 WuXi AppTec shall not thereafter use or exploit the Customer Patent Rights, Customer Know-How or the Customer Information in any way whatsoever;

11.4.5 WuXi AppTec and Customer shall do all such acts and things and shall sign and execute all such deeds and documents as the other may reasonably require to evidence compliance with this Section 10.4.

12. Force Majeure

12.1 Force Majeure Rights. If either Party is prevented or delayed in the performance of any of its obligations under the Agreement by Force Majeure such Party shall give written notice thereof to the other Party specifying the matters constituting Force Majeure together with such evidence as reasonably can give and specifying the period for which it is estimated that such prevention or delay will continue, the Party claiming Force Majeure shall be excused from the performance or the punctual performance of such obligations as the case may be from the date of such notice for so long as such cause of prevention or delay shall continue. Notwithstanding the foregoing, if the Party claiming Force Majeure estimates that the delay will exceed ****, or if the delay has, in fact, exceeded ****, the other Party may terminate this Agreement for cause as set forth in Section 9.3, including an additional **** notice to remedy the breach,

12.2 Force Majeure Definition. The expression "Force Majeure" shall be deemed to include any cause affecting the performance by either Party of the Agreement arising from or attributable to acts, events, acts of God, omissions or accidents beyond the reasonable control of the Party claiming the Force Majeure.

13. Inventions.

13.1 All information, data and writings provided to WuXi AppTec by and/or on behalf of Customer in connection with this Agreement, including Customer Know-How, Customer Information, Customer Patent Rights, and Customer Materials which were owned by or licensed to Customer prior to being provided to WuXi AppTec, shall remain the property of Customer (the "Customer Data"). WuXi AppTec shall acquire no right, title or interest in the Customer Data as a result of its performance of the Services.

13.2 Any and all intellectual property including without limitation WuXi AppTec Patent Rights, WuXi AppTec Know-How, trade secrets, and proprietary information, whether tangible or intangible, which was in WuXi AppTec 's possession on the Effective Date or which is later generated or acquired by WuXi AppTec outside the scope of activities under this Agreement (collectively, the "WuXi AppTec Property"), shall be the sole and exclusive property of WuXi AppTec,

13.3 All information, data, writings, inventions and other work product, in any form whatsoever, both tangible and intangible, developed as a result of WuXi AppTec's performance of the Services (collectively, the "Works"), shall be the sole and exclusive property of Customer. Customer shall be the sole owner of all the rights to such Works in any form and in all fields of use known or hereafter existing. Provided that Customer has fulfilled all of its payment obligations to WuXi AppTec, Customer may transfer such Works or use the Works for any purpose without further payment to WuXi AppTec. In the event new intellectual property emerges in the course of WuXi AppTec providing services to Customer which do not use or incorporate Customer Data provided by Customer and is generally valuable to WuXi AppTec in the conduct of its business as a contact service organization, Customer and WuXi AppTec agree that Customer will own such new intellectual property and Customer will hereby grant WuXi AppTec a non-exclusive, world-wide, fully paid-up, royalty-free, perpetual, irrevocable license to any and all portions of such new intellectual property.

13.4 Protocols, methods, controls, SOPS, specifications, or documents generally used by WuXi AppTec in the normal course of its business that are used by WuXi AppTec for Services (collectively, "Service Instruments") are furnished solely with respect to Services, and WuXi AppTec will retain all common law, statutory, ownership, and other reserved rights in such Service Instruments. For the avoidance of doubt, Service Instruments does not include Customer-specific batch records, data, reports, or other similar documents containing Customer-specific information produced by WuXi AppTec as a result of Services.

13.5 Neither Party shall acquire any right, title or interest in any of the trademarks, service marks or copyrights belonging to the other Party. No right or license, whether express or implied, is granted to one Party by the other Party, except to the extent expressly authorized by this Agreement.

14. Mediation, Arbitration, Governing Law, Jurisdiction, and Enforceability

14.1 Mediation. In the event of any dispute, controversy or claim arising out of or relating to this Agreement, or the breach, termination or invalidity thereof, each Party shall by written notice to the other have the right to have such dispute referred to the senior management of WuXi AppTec and Customer for attempted resolution by good faith negotiations within **** after such notice is received. If such senior management are unable to resolve such dispute within the **** period, and before arbitration is initiated, the Parties shall participate in a mediation that will last no less than **** unless the dispute is resolved before such time. Notwithstanding the requirement for the parties to submit to mediation for a minimum of ****, neither party will be required to participate in mediation for longer than ****. Any mediation will take place a mutually agreeable venue, and will be officiated by a mutually agreeable mediator identified and engaged by the Parties, the cost and fees for whom shall be borne equally by the Parties. In the event the Parties' efforts to reach an amicable resolution through mediation or other informal means are unsuccessful, either party may invoke the provisions of Section 14.2. Any settlement reached by the Parties under this Section shall not be binding until reduced to writing and signed by the above-specified management of WuXi AppTec and Customer, When reduced to writing, such agreement shall supersede all other agreements, written or oral, to the extent such agreements specifically pertain to the matters so settled.

14.2 Arbitration. In the event of the failure to reach a resolution pursuant to Section 14.1, any dispute, controversy or claim arising out of or relating to this Agreement, or the breach, termination or invalidity thereof, shall be finally settled by binding arbitration in accordance with the complex rules of the Commercial Arbitration Rules of the American Arbitration Association in effect on the date of this Agreement by a panel of three arbitrators who shall be experienced in the biopharmaceutical industry and who will be appointed in accordance with such rules. The place of arbitration will be Philadelphia, Pennsylvania, and the Parties shall **** filing fees, arbitrator fees or other costs of such proceedings, except that each Party **** attorney's fees, and other out-of-pocket arbitration expenses, unless the arbitrators decides otherwise.

14.3 Governing Law and Jurisdiction. The construction, validity and performance of the Agreement shall be governed by the laws of the Commonwealth of Pennsylvania.

14.4 Waiver. No failure or delay on the part of either WuXi AppTec or Customer to exercise or enforce any rights conferred on it by the Agreement shall be construed or operate as a waiver thereof nor shall any single or partial exercise of any right, power or privilege or further exercise thereof operate so as to bar the exercise or enforcement thereof at any time or times thereafter.

14.5 Severability. The illegality or invalidity of any provision (or any part thereof) of the Agreement or these Standard Terms and Conditions shall not affect the legality, validity or enforceability of the remainder of its provisions or the other parts of such provision as the case may be.

15. Miscellaneous

15.1 Assignment. Neither party shall be entitled to assign, transfer, charge or in any way make over the benefit and/or the burden of this Agreement without the prior written consent of the other which consent shall not be unreasonably withheld or delayed, save that either party shall be entitled without the prior written consent of the other party to assign, transfer, charge, sub-contract, deal with or in any other manner make over the benefit and/or burden of this Agreement to an Affiliate or to any company with which such assigning party may merge or to any company to which such assigning party may transfer its assets and undertakings.

15.2 Press Releases. The text of any press release or other communication to be published by or in the media concerning the subject matter of the Agreement shall require the prior written approval of WuXi AppTec and Customer.

15.3 Entire Agreement. The Agreement, the Work Orders attached hereto embody the entire understanding of WuXi AppTec and Customer and there are no promises, terms, conditions or obligations, oral or written, expressed on implied, other than those contained in the Agreement. The terms of the Agreement shall supersede all previous agreements (if any) which may exist or have existed between WuXi AppTec and Customer relating to the Services. In the event the Parties desire to enter into a Commercial Manufacturing Agreement with each other, such Commercial Manufacturing Agreement shall be on separate terms and conditions from this Agreement

15.4 No Third Party Beneficiaries. The parties to this Agreement do not intend that any terms hereof should be enforceable by any person who is not a party to this Agreement.

15.5 Counterparts. This Agreement may be executed in two or more counterparts, and each such counterpart shall be deemed an original thereof.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by its duly authorized representatives as of the Effective Date.

WuXi AppTec, Inc.

By: /s/ W. Alan Moore
Name: W. Alan Moore
Title: Vice President, Cell Manuf.
Date: April 6, 2015

REGENXBIO Inc.

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President & CEO
Date: April 3, 2015

CONFIDENTIAL TREATMENT REQUESTED

COOPERATION AGREEMENT

This **AGREEMENT** is entered into as of date of signature hereof by and between:

WuXI AppTec, Inc., having a mailing address of 4751 League Island Blvd., Philadelphia, PA 19112, USA (WUXI APPTEC) and,

REGENXBIO Inc., having a principal place of business at 9712 Medical Center Drive, Suite 100, Rockville, MD 20850 (REGENX).

Hereinafter individually referred to as "Party" and collectively as "Parties".

WHEREAS, WUXI APPTEC is a service laboratory which offers contract testing, process development, and regulatory compliant manufacturing services for biopharmaceuticals, medical devices, cellular therapeutics, gene therapies, and tissue-based products; and

WHEREAS, REGENX is an organization that drives the development of NAV® gene therapies through its own development, manufacturing and commercialization of NAV gene therapy treatments for a number of diseases including lysosomal storage disorders and ocular diseases and the licensing of NAV Technology to ther companies,.

WHEREAS, WUXI APPTEC and REGENX intend to enter into a collaboration that will take advantage of the complementary strengths of both companies within the AAV gene therapy area; and

WHEREAS, the purpose of the intended collaboration is to offer REGENX-established NAV gene therapy programs, and REGENX licensees (collectively, Customers) at their choice, specialized contract services for the efficient progression of these gene therapy programs through the various stages of preclinical and clinical development and manufacturing to the benefit of both companies and Customers; and

WHEREAS, the Parties do not intend or wish to restrict or control in any way the decision of Customers to utilize either REGENX or WUXI APPTEC.

Now Therefore, the parties hereby agree as follows:

Article 1 Definitions

1.1 "**Confidential Information**" means (i) the terms of this Agreement and (ii) any and all information relating to the subject matter of this Agreement, including but not limited to know-how, technical information, research, marketing, strategic or other information that is disclosed in writing, visually, orally or in electronic medium by the Providing Party (as defined in Article 3) or its Affiliates to the Receiving Party (as defined in Article 3) or its Affiliates, whether prior to or after signature of this Agreement, in the course of the Parties' evaluation, negotiation of or performance under this Agreement.

1.2 "**Contract Services**" shall mean (i) contract manufacturing services for NAV gene therapy products with or without cell line and/or process development and scale-up activities, and (ii) testing services including but not limited to cell bank characterization, virus characterization, lot release testing, toxicology and biodistribution studies, molecular biology studies, analytical characterization, and custom testing studies.

Article 2 Object of the Agreement

2.1 Cooperative Partnership

Regulatory compliant manufacturing and testing services are required to enable the efficient advancement of REGENX-established NAV gene therapy programs, requiring the transfer ofspecific technical and process information to a contract development and manufacturing organization. Once transferred, efficiency is gained in the application of this information to subsequent products utilizing the REGENX technology for the development of REGENX products or those of REGENX licensees. Moreover, the ability to provide process development, manufacturing and testing facilities of adequate capacity and meeting compliance standards appropriate for supply of late stage clinical and commercial materials provides an incentive for adoption of the REGENX technology and can advance the programs of REGENX licensees.

WUXI APPTEC is currently providing specialized process development, testing and compliant manufacturing services for an array of biological and cellular therapy products and is expanding facilities and capabilities to provide services to support the clinical and commercial supply of gene therapy products. Establishing familiarity with the REGENX process and analytical technology, and receiving access to future technology enhancements may provide an advantage to WUXI APPTEC in providing commercial services for the programs of REGENX and its licensees.

REGENX and WUXI APPTEC acknowledge each other as cooperative partners with the goal of establishing WUXI APPTEC as the preferred process development, testing and manufacturing service provider for REGENX and REGENX licensees. Through this cooperative partnering relationship WUXI APPTEC will work with REGENX to establish standard processes applicable to the REGENX technology platform which may be applied for the development, testing and manufacture of REGENX products or those of REGENX licensees. In partnering, the parties intend to enhance the contract service business opportunities for WUXI APPTEC and to enhance the business opportunities of REGENX (1) in the efficient development of its gene therapy programs, (2) in the adoption of REGENX manufacturing technology, and (3) through the provision of a knowledgeable and compliant manufacturing and testing resource for its licensees.

2.2 Joint Steering Committee

REGENX and WUXI APPTEC will develop a Joint Steering Committee consisting of **** in order to discuss and address issues relating to the Cooperation Agreement. **** shall be selected by REGENX and **** shall be selected by WUXI APPTEC. The Joint Steering Committee shall meet **** or otherwise as agreed-to by the members, either in person or by phone.

2.3 Technology transfer and manufacturing resources

REGENX shall provide process development, analytical methods and information and training necessary for WUXI APPTEC to reasonably provide process development, testing and manufacturing services for the production of REGENX products. WUXI APPTEC will establish these services consistent with its manufacturing, testing and quality systems in order to provide regulatory compliant services to REGENX and REGENX licensees. For the avoidance of doubt, WUXI APPTEC shall provide services incorporating REGENX-provided information, methods and training only to REGENX and REGENX licensees.

2.4 Ongoing support and technology advancements

REGENX will provide reasonable direct support and the assistance of its technical, scientific staff and advisors if requested, to advise WUXI APPTEC on the effective application of the technology or to troubleshoot or resolve technical issues to the best of its abilities which may arise in the application of REGENX technology on behalf of WUXI APPTEC’s support of REGENX and its licensees. WUXI APPTEC and REGENX will work cooperatively to incorporate reasonable advancements in manufacturing processes, analytical testing or quality control paradigms which may provide for increased process efficiency, safety, quality or regulatory compliance.

2.5 Access to process development, testing and manufacturing

WUXI APPTEC shall provide access to process development, testing and manufacturing resources for REGENX and REGENX licensee's **** WUXI APPTEC will work with REGENX to provide preferred scheduling and performance of services supporting REGENX gene therapy programs and those of REGENX licensees. ****.

2.6 Recommendation and Use of WUXI APPTEC by REGENX for Contract Services

REGENX shall recommend WUXI APPTEC as its preferred provider of process development, testing and manufacturing services to Customers provided that WUXI APPTEC maintains acceptable quality standards and conformance with generally recognized industry standards. ****.

2.7 Ethical practices

The Parties agrees that they will not engage in any deceptive, misleading or unethical practices that are or might be detrimental to the other Party, any Customer or the general public.

2.8 Promotion of technology and services

WUXI APPTEC and REGENX shall jointly agree upon mechanisms to promote the availability of the specialized services relating to the REGENX technology, the potential advantages of the technology, and the availability of process development, testing and manufacturing services. The Parties agree that any promotional materials or reference to the other Parties' services will be with the prior review and expressed written approval of the other Party. During the term of the Agreement, WUXI APPTEC will not enter into a similar Cooperation Agreement for manufacturing services with other companies developing technology platforms for AAV-mediated gene therapies. ****.

Article 3 Confidentiality

3.1 General

Parties undertake to hold in strict confidence all Confidential Information which has been or will be made available to either Party (hereinafter referred to as the "Receiving Party") by the other Party (hereinafter referred to as the Providing Party") and accordingly (i) not to disclose such Confidential Information to third parties without the prior written consent of the Providing Party, except to such **** to disclose such Confidential Information for the purpose of this Agreement, and (ii) not to use such Confidential Information for any purpose other than the cooperation and the performance of this Cooperation Agreement as agreed herein.

3.2 Special Disclosure

If Confidential Information is disclosed by a Party or its Affiliates other than in written or electronic form, then the Receiving Party's obligations of confidentiality and non-use shall only apply if the Confidential Information is indicated upon disclosure as being confidential and is then summarized electronically or in writing and provided to the other Party within **** after initial disclosure.

3.3 Permitted Disclosure

Before any Confidential Information is passed on to **** or to third parties after written consent of the Providing Party in accordance with Article 3.1, the Receiving Party undertakes to impose all obligations on said **** or third parties as imposed on Receiving Party under this Agreement.

3.4 Exceptions

The obligations contained in this Article 3 shall not apply to such Confidential Information which (i) is in the possession of the Receiving Party prior to disclosure or which becomes known to the

Receiving Party without having been furnished, directly or indirectly, by the Providing Party or any of its Affiliated Companies, or (ii) was public knowledge at the time of disclosure or which becomes public knowledge after disclosure in circumstances which are not the fault or negligence of the Receiving Party, or (iii) can be shown by written documentation to have been made known to a Party or its Affiliates from another source free from any obligation of confidentiality, or (iv) was not obtained either directly or indirectly from the other Party or its Affiliates.

3.5 Disclosure of Confidential Information

In the event that a Party is required by law, regulation, rule, act or order of any governmental authority or agency to disclose Confidential Information of the other Party, it shall be entitled to do so provided that it shall first notify the other Party forthwith of any such required disclosure and limit such disclosure as far as is possible under applicable law. Such disclosure shall, however, not relieve either Party of its other obligations contained herein.

3.6 Return of Confidential Information

The Receiving Party shall upon expiry or termination of this Agreement return to the Providing Party all Confidential Information and shall not retain any copies thereof, except for one copy, which might be retained by either party for documentation purposes.

3.7 Term of Confidentiality

This Article 3 shall remain in force during the period of this Agreement and for a further period of **** after the termination or expiration thereof.

Article 4 Public Disclosure

Except to the extent allowed under Article 2 or required by law, neither Party will make any public announcement, statement or communication with respect to the existence and/or activities under this Cooperation Agreement without the prior written consent of the other Party.

Article 5 No financial obligation

Neither Party shall owe any payment to the other Party due to activities undertaken under this Cooperation Agreement. ****.

Article 6 Term and Termination

6.1 Term

This Agreement shall enter into force on the date of signature hereof by both Parties and shall continue until and unless terminated in accordance with this Agreement.

6.2 Termination

Either Party may terminate this Agreement:

a) if the other commits a material breach of the Agreement which in the case of a breach capable of remedy is not remedied to the reasonable satisfaction of the non-breaching Party within **** of the receipt by the other of written notice identifying the breach and requiring its remedy; or

b) if the other party files a petition of any type as to its bankruptcy, is declared bankrupt, becomes insolvent, makes an assignment for the benefit of creditors, goes into liquidation or receivership, otherwise loses legal control of its business or ceases to carry on its business.

6.3 Elective Termination

Either party shall be entitled to terminate this Agreement at any time by giving **** written notice.

Article 7 General Provisions

7.1 Force Majeure

Neither Party shall be liable for delay or failure to perform hereunder due to any contingency beyond its control, including, but not limited to acts of God, fires, floods, wars, civil wars or sabotage, provided, such Party promptly gives to the other Party hereto written notice claiming force majeure and uses its best efforts to eliminate the effect of such force majeure, insofar as is possible and with all reasonable dispatch. If the period of delay or failure with respect to either Party should extend for **** or more, consecutively or cumulatively, in any one-year period commencing on the effective date of this Agreement, as provided above, then either Party shall in its discretion have the right to terminate this Agreement forthwith upon written notice at any time after expiration of said **** period.

7.2 No Waiver

No failure or delay on the part of either WUXI APPTEC or REGENX to exercise or enforce any rights conferred on it by the Agreement shall be construed or operate as a waiver thereof nor shall any single or partial exercise of any right, power or privilege or further exercise thereof operate so as to bar the exercise or enforcement thereof at any time or times thereafter.

7.3 Independent Contractor

In the performance of this Agreement each Party shall be an independent contractor, and therefore, no Party shall be entitled to any benefits applicable to any employee of the other Party. No Party is authorized to act as an agent for the other Party for any purpose, and no Party shall enter into any contract, warranty or representation as to any matter on behalf of the other Party. In all cases unless otherwise modified by written agreement of both Parties this Agreement is not creating or inferring a Joint Venture or a legal Partnership between WUXI APPTEC and REGENX.

7.4 Written Form

No amendments, changes, modifications or alterations of the terms and conditions of this Cooperation Agreement shall be binding upon either Party, unless in writing and signed by both Parties. This applies also to the amendment, change etc. of the requirement of written form under this Section.

7.5 Entire Understanding

This Agreement contains the entire understanding between the Parties and supersedes any and all prior agreements, understandings and arrangements, whether written or oral between the Parties.

7.6 Severability

The illegality or invalidity of any provision (or any part thereof) of this Cooperation Agreement shall not affect the legality, validity or enforceability of the remainder of its provisions or the other parts of such provision as the case may be.

Article 8 Mediation, Arbitration, and Governing Law

8.1 Applicable Law

This Cooperation Agreement shall be governed by and construed under the laws of the Commonwealth of Pennsylvania without regard to the doctrine of conflict of laws.

8.2 Mediation

In the event of any dispute, controversy or claim arising out of or relating to this Agreement, or the breach, termination or invalidity thereof, each party shall by written notice to the other have the right to have such dispute referred to the senior management of WUXI APPTEC and REGENX for attempted resolution by good faith negotiations within **** after such notice is received. If such senior management are unable to resolve such dispute within such **** **** period, either party may invoke the provisions of Section 8.3. Any settlement reached by the parties under this Section 8.2 shall not be binding until reduced to writing and signed by the above-specified management of WUXI APPTEC and REGENX. When reduced to writing, such agreement shall supersede all other agreements, written or oral, to the extent such agreements specifically pertain to the matters so settled.

8.3 Arbitration

In the event of the failure to reach a resolution pursuant to Section 8.2, any dispute, controversy or claim arising out of or relating to this Agreement, or the breach, termination or invalidity thereof, shall be finally settled by binding arbitration in accordance with the complex rules of the Commercial Arbitration Rules of the American Arbitration Association in effect on the date of this Agreement by a single arbitrator who shall be experienced in the Biopharmaceutical industry and who shall be appointed in accordance with such rules. The place of arbitration shall be Philadelphia, Pennsylvania, and the parties shall **** filing fees, arbitrator fees or other costs of such proceedings, **** attorney's fees, and other out-of-pocket arbitration expenses, unless the arbitrator decides otherwise.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their respective duly authorized representatives as of the date of the last signature below.

WuXi AppTec Inc.		**REGENXBIO Inc.**	
By:	/s/ W. Alan Moore	By:	/s/ Kenneth Mills
Name :	W. Alan Moore	Name:	Kenneth Mills
Title:	Vice President, Cell Manufacturing	Title:	President & CEO
Date:	May 28, 2015	Date:	May 27, 2015

Exhibit 10.29

REGENXBIO INC.

MANAGEMENT CASH INCENTIVE PLAN

(AS ADOPTED EFFECTIVE SEPTEMBER , 2015)

TABLE OF CONTENTS

REGENXBIO INC.
MANAGEMENT CASH INCENTIVE PLAN

ARTICLE 1. BACKGROUND AND PURPOSE

1.1 Effective Date. The Plan was adopted by the Committee on the date set forth above, became effective immediately and is not subject to approval by the Company's stockholders.

1.2 Purpose of the Plan. The Plan is intended to motivate Participants to achieve excellent short- and long-term financial performance for the Company and its business units. The Plan provides Participants with the opportunity to earn cash incentive awards for the achievement of goals relating to the performance of the Company.

ARTICLE 2. DEFINITIONS

The following words and phrases shall have the following meanings, unless a different meaning is plainly required by the context:

2.1 "**Actual Award**" means, as to any Performance Period, the actual award (if any) payable to a Participant for the Performance Period. Each Actual Award is determined by the Payout Formula for the Performance Period, subject to the Committee's authority under Section 3.5 to increase, eliminate or reduce the award otherwise indicated by the Payout Formula.

2.2 "**Affiliate**" means any corporation or other entity (including, without limitation, partnerships and joint ventures) controlled by the Company.

2.3 "**Base Salary**" means, as to any Performance Period, the Participant's earned salary during the Performance Period. Base Salary shall be calculated before both (a) deductions for taxes or benefits and (b) deferrals of compensation pursuant to Company-sponsored plans or Affiliate-sponsored plans.

2.4 "**Board**" means the Company's Board of Directors.

2.5 "**Committee**" means the Compensation Committee of the Board.

2.6 "**Company**" means REGENXBIO Inc., a Delaware corporation, or any successor thereto.

2.7 "**Disability**" means a permanent disability, as determined for purposes of the principal long-term disability insurance plan maintained by the Company for the benefit of the Participant. If there is no such plan, Disability shall be determined in accordance with a policy established by the Committee.

2.8 “**Employee**” means any employee of the Company or of an Affiliate, whether such employee is so employed when the Plan is adopted or becomes so employed after the adoption of the Plan.

2.9 “**Fiscal Quarter**” means a fiscal quarter within a Fiscal Year of the Company.

2.10 “**Fiscal Year**” means the fiscal year of the Company.

2.11 “**Participant**” means, as to any Performance Period, an Employee who has been selected for participation in the Plan for that Performance Period pursuant to Section 3.1.

2.12 “**Payout Formula**” means, as to any Performance Period, the formula or payout matrix established by the Committee pursuant to Section 3.4 in order to determine the Actual Awards (if any) to be paid to Participants. The formula or matrix may differ from Participant to Participant.

2.13 “**Performance Period**” means a Fiscal Year, or any longer or shorter period determined by the Committee.

2.14 “**Performance Goals**” means the goal(s) determined by the Committee to be applicable to a Participant for a Target Award for a Performance Period. As determined by the Committee, the Performance Goal(s) may provide for a targeted level or levels of achievement using the performance criteria specified by the Committee. Such criteria shall be based on one or more of the performance metrics set forth in **Appendix A** attached to the Plan.

2.15 “**Plan**” means this REGENXBIO Inc. Management Cash Incentive Plan, as set forth in this instrument and as hereafter amended from time to time.

2.16 “**Progress Payment**” means a portion of the Target Award or Actual Award determined in accordance with Section 3.5 that has been earned by the Participant as of the end of the Progress Period, based on achievement of the applicable Performance Goals, and that may be paid to the Participant during the Performance Period.

2.17 “**Progress Period**” means a period shorter than and within the Performance Period for which a Progress Payment may be made.

2.18 “**Retirement**” means, with respect to any Participant, a Termination of Employment occurring in accordance with a policy or policies established by the Committee from time to time.

2.19 “**Target Award**” means the target award payable under the Plan to a Participant for the Performance Period or Progress Period, as applicable, expressed as a percentage of his or her Base Salary or a specific dollar amount, as determined by the Committee in accordance with Section 3.3.

2.20 "**Termination of Employment**" means a cessation of the employee-employer relationship between an Employee and the Company or an Affiliate for any reason, including (without limitation) a termination by resignation, discharge, death, Disability, Retirement or the disaffiliation of an Affiliate, but excluding a transfer from the Company to an Affiliate or between Affiliates.

ARTICLE 3. SELECTION OF PARTICIPANTS AND DETERMINATION OF AWARDS

3.1 Selection of Participants. The Committee shall select the Employees who shall be Participants for any Performance Period. The Committee also may designate as Participants one or more individuals (by name or position) who are expected to become Employees during a Performance Period. Participation in the Plan is in the sole discretion of the Committee and shall be determined Performance Period by Performance Period. Accordingly, an Employee who is a Participant for a given Performance Period is in no way assured of being selected for participation in any subsequent Performance Period.

3.2 Determination of Performance Goals. The Committee shall establish the Performance Goals for each Participant for the Performance Period. Such Performance Goals shall be set forth in writing and shall be based on one or more of the performance metrics set forth in Appendix A attached to the Plan. Any criteria used may be measured (a) in absolute terms, (b) in relative terms, including (without limitation) the passage of time and/or against other companies or metrics, (c) on a per-share basis, (d) against the performance of the Company as a whole or against particular segments or products of the Company and/or (e) on a pre-tax or after-tax basis. Any Performance Goal may be measured on a basis other than generally accepted accounting principles.

3.3 Determination of Target Awards. The Committee shall establish a Target Award for each Participant for each Performance Period. Such Target Award shall be set forth in writing.

3.4 Determination of Payout Formula or Formulae. The Committee shall establish a Payout Formula or Formulae for purposes of determining the Actual Award (if any) payable to each Participant. Each Payout Formula shall (a) be in writing, (b) be based on a comparison of actual performance to the Performance Goals, (c) provide for the payment of a Participant's Target Award if the Performance Goals for the Performance Period are achieved at the predetermined level and (d) provide for the payment of an Actual Award greater than or less than the Participant's Target Award, depending upon the extent to which actual performance exceeds or falls below the Performance Goals.

3.5 Determination of Actual Awards. After the end of each Performance Period or, to the extent that Progress Payments will be made, after the end of each Progress Period, the Committee shall certify the extent to which the Performance Goals applicable to each Participant for the Performance Period or Progress Period, as applicable, were achieved or exceeded, as determined by the Committee. The Actual Award for each Participant shall be determined by applying the Payout Formula to the level of actual performance that has been certified by the Committee. Any contrary provision of the Plan notwithstanding, the Committee

may (a) reduce or eliminate the Actual Award that otherwise would be payable under the Payout Formula or (b) determine whether or not any Participant will receive an Actual Award or Progress Payment in the event that the Participant incurs a Termination of Employment before such Actual Award or Progress Payment is to be paid pursuant to Section 4.2.

3.6 Adjustments. The Committee may adjust the results under any Performance Goal to exclude any of the following events that occurs during a Performance Period: (a) asset write-downs, (b) litigation, claims, judgments or settlements, (c) the effect of changes in tax law, accounting principles or other such laws or provisions affecting reported results, (d) accruals for reorganization and restructuring programs, (e) mergers or acquisitions and (f) any other extraordinary, unusual or non-recurring items.

ARTICLE 4. PAYMENT OF AWARDS

4.1 Right to Receive Payment. Each Actual Award or Progress Payment that may become payable under the Plan shall be paid solely from the general assets of the Company or the Affiliate that employs the Participant (as the case may be), as determined by the Company. No amounts awarded or accrued under the Plan shall be funded, set aside or otherwise segregated prior to payment. The obligation to pay Actual Awards or Progress Payments under the Plan shall at all times be an unfunded and unsecured obligation of the Company. Participants shall have the status of general creditors of the Company or the Affiliate that employs the Participant.

4.2 Timing of Payment. Subject to Section 3.5, payment of each Actual Award or Progress Payment shall be made as soon as administratively practicable, but in no event later than two and one-half months after the end of the applicable Performance Period or Progress Period, as the case may be.

4.3 Form of Payment. Each Actual Award or Progress Payment shall be paid in cash (or its equivalent) in a single lump sum.

4.4 Payment in the Event of Death. If a Participant dies before receiving an Actual Award or Progress Payment (determined under Section 3.5) that was scheduled to be paid before his or her death for a prior Performance Period or Progress Period, then the Actual Award or Progress Payment shall be paid to the Participant's designated beneficiary or, if no beneficiary has been designated, to the administrator or representative of his or her estate. Any beneficiary designation or revocation of a prior designation shall be effective only if it is in writing, signed by the Participant and received by the Company prior to the Participant's death.

ARTICLE 5. ADMINISTRATION

5.1 Committee Authority. The Plan shall be administered by the Committee, subject to Section 5.3. The Committee shall have all powers and discretion necessary or appropriate to administer the Plan and to control its operation, including (without limitation) the power to (a) determine which Employees shall be granted awards, (b) prescribe the terms and conditions of the awards, (c) interpret the Plan, (d) adopt such procedures and sub-plans as are necessary or appropriate to permit participation in the Plan by Employees who are foreign nationals or employed outside of the United States, (e) adopt rules for the administration, interpretation and application of the Plan and (f) interpret, amend or revoke any such rules.

5.2 Decisions Binding. All determinations and decisions made by the Committee, the Board or any delegate of the Committee pursuant to the provisions of the Plan shall be final, conclusive and binding on all persons and shall be given the maximum deference permitted by law.

5.3 Delegation by the Committee. The Committee, on such terms and conditions as it may provide, may delegate all or part of its authority and powers under the Plan to one or more directors and/or employees of the Company.

ARTICLE 6. GENERAL PROVISIONS

6.1 Tax Withholding. The Company or an Affiliate, as applicable, shall withhold all required taxes from an Actual Award or Progress Payment, including any federal, state, local or other taxes.

6.2 No Effect on Employment. Nothing in the Plan shall interfere with or limit in any way the right of the Company or an Affiliate, as applicable, to terminate any Participant's employment or service at any time, with or without cause. Employment with the Company and its Affiliates is on an at-will basis only. The Company expressly reserves the right, which may be exercised at any time and without regard to when during or after a Performance Period such exercise occurs, to terminate any individual's employment with or without cause, and to treat him or her without regard to the effect that such treatment might have upon him or her as a Participant.

6.3 No Effect on Other Benefits. Except as expressly set forth in a Participant's employment agreement with the Company, any Actual Awards or Progress Payments under the Plan shall not be considered for the purpose of calculating any other benefits to which such Participant may be entitled, including (a) any termination, severance, redundancy or end-of-service payments, (b) other bonuses or long-service awards, (c) overtime premiums, (d) pension or retirement benefits or (e) future Base Pay or any other payment to be made by the Company to such Participant.

6.4 Successors. All obligations of the Company and any Affiliate under the Plan, with respect to awards granted hereunder, shall be binding on any successor to the Company and/or such Affiliate, whether the existence of such successor is the result of a merger, consolidation, direct or indirect purchase of all or substantially all of the business or assets of the Company or such Affiliate, or any similar transaction.

6.5 Nontransferability of Awards. No award granted under the Plan shall be sold, transferred, pledged, assigned or otherwise alienated or hypothecated, other than by will, by the laws of descent and distribution or to the limited extent provided in Section 4.4. All rights with respect to an award granted to a Participant shall be available during his or her lifetime only to the Participant.

ARTICLE 7. DURATION, AMENDMENT AND TERMINATION

7.1 Duration of the Plan. The Plan shall commence on the date specified herein and shall remain in effect thereafter until terminated pursuant to Section 7.2.

7.2 Amendment, Suspension or Termination. The Board or the Committee may amend, suspend or terminate the Plan, or any part thereof, at any time and for any reason. No award may be granted during any period of suspension or after termination of the Plan.

ARTICLE 8. LEGAL CONSTRUCTION

8.1 Severability. In the event any provision of the Plan shall be held illegal or invalid for any reason, the illegality or invalidity shall not affect the remaining parts of the Plan, and the Plan shall be construed and enforced as if the illegal or invalid provision had not been included.

8.2 Requirements of Law. The granting of awards under the Plan shall be subject to all applicable laws, rules and regulations, and to such approvals by any governmental agencies or national securities markets as may be required.

8.3 Governing Law. The Plan and all awards shall be construed in accordance with and governed by the laws of the State of Delaware, without regard to their conflict-of-law provisions.

8.4 Captions. Captions are provided herein for convenience only and shall not serve as a basis for interpretation or construction of the Plan.

APPENDIX A

PERFORMANCE METRICS

The Committee may establish Performance Goals derived from the following metrics:

- Backlog
- Bookings (including annual or total contract value bookings)
- Cash
- Cash and short-term investments
- Cash flow return on investment
- Comparisons with various stock market indices
- Deferred revenue
- Earnings or earnings per share (including earnings before taxes, earnings before interest and taxes or earnings before interest, taxes, depreciation and amortization)
- Expenses or expense reductions
- Free cash flow or free cash flow per share
- Gross profits
- Headcount
- Implementation, completion or attainment of measurable objectives with respect to research, development, products, regulatory approvals, projects or recruiting and maintaining personnel
- Market share
- Net income (before or after taxes)
- Operating margin or cash margin
- Operating profit/loss (on a GAAP or non-GAAP basis)
- Pre- or after-tax income (before or after allocation of corporate overhead and bonus)
- Reductions in costs
- Return on equity
- Revenue
- Stock price
- Total expenses
- Total stockholder return
- Working capital
- Increases or growth in any of the foregoing

Exhibit 10.30

REGENX BIOSCIENCES, LLC
BOARD OF MANAGERS AGREEMENT

This BOARD OF MANAGERS AGREEMENT (this "Agreement") is made and entered into as of February 6, 2013 by and between ReGenX Biosciences, LLC, a Delaware limited liability company ("ReGenX"), and Mr. Don Hayden, an individual (the "Manager"). ReGenX and the Manager are referred to herein together as the "Parties."

RECITAL

ReGenX desires that the Manager become a member of its Board of Managers, and the Manager desires to become a member of the Board of Managers of ReGenX.

AGREEMENT

In consideration of the mutual covenants below, and intending to be legally bound, the Parties hereby agree as follows:

1. **Board of Managers**. ReGenX hereby retains the Manager, and the Manager hereby agrees to serve, as a member of the Board of Managers of ReGenX. As a member of the Board of Managers, the Manager shall devote his efforts to satisfying the responsibilities, and shall have the powers and be entitled to the rights, of a member of the Board of Managers as set forth in ReGenX's Fifth Amended and Restated Limited Liability Company Agreement (a copy of which has been provided to the Manager), as such agreement may be amended from time to time.

2. **Compensation and Expenses**.

(a) As compensation for the Manager's service on the Board of Managers, ReGenX will (i) pay the Manager an annual fee of $40,000, payable within ten days after the execution of this Agreement and thereafter on each anniversary date of the date of this Agreement so long as the Manager serves on the Board of Managers, and (ii) grant to the Manager an equity incentive consisting of 6,420,000 Class B Units of ReGenX. It is understood by the Manager that ReGenX is in the process of raising equity capital through the sale of Preferred Units and ReGenX wishes to compensate the Manager for the dilution caused by such sale. Accordingly in the event that (i) ReGenX closes a sale of Preferred Units on or before December 31, 2013 and (ii) the Manager remains a member of the Board of Managers of ReGenX at the time of such sale then, no later than promptly following such sale, ReGenX will grant the Manager such number of additional shares of Class B Units of ReGenX as is necessary to maintain Manager's 2.5% equity interest in ReGenX, on a fully diluted basis. The terms and conditions of the Class B Units and the grant are governed by the ReGenX Fifth Amended and Restated Limited Liability Company Agreement, the ReGenX 2009 Equity Incentive Plan and the Manager's Class B Equity Interest Award Agreement, a copy of each of which has been provided to the Manager.

(b) ReGenX will also reimburse or cause one or more of its affiliates to reimburse the Manager for all reasonable travel and other expenses preapproved by ReGenX that

the Manager incurs in connection with the services the Manager will provide to ReGenX. Reimbursements will be made in accordance with ReGenX's policies and procedures for reimbursement, including the delivery to ReGenX of appropriate expense vouchers or other documentation.

3. **Recognition of ReGenX's Rights; Nondisclosure**.

(a) At all times during the term of the Manager's association with ReGenX and thereafter, the Manager shall hold in strictest confidence and shall not disclose, use, lecture upon or publish any of ReGenX's Proprietary Information (defined below), except to the extent such disclosure, use or publication may be required in direct connection with the Manager's performance for ReGenX as a member of the Board of Managers or is expressly authorized in writing by an officer of ReGenX.

(b) The term "Proprietary Information" shall mean any and all trade secrets, confidential knowledge, know-how, data or other proprietary information or materials of ReGenX. By way of illustration but not limitation, Proprietary Information includes: (i) inventions, ideas, samples, media and/or viral vectors and procedures and formulations for producing any such samples, media and/or viral vectors, processes, formulas, data, know-how, improvements, discoveries, developments, designs and techniques; (ii) information regarding plans for research, development, new products, marketing and selling, business plans, budgets and unpublished financial statements, licenses, prices and costs, suppliers and customers; (iii) information regarding the skills and compensation of employees or other consultants of ReGenX; and (iv) the compensation of the Manager and the terms and conditions of this Agreement. Notwithstanding the foregoing "Proprietary Information" shall not include information that the Manager can demonstrate by competent evidence:

(i) is known by the Manager prior to receipt from ReGenX;

(ii) is hereafter disclosed to the Manager by a third party having no obligation of confidentiality to ReGenX;

(iii) is available to the public at the time of the Manager's receipt from ReGenX;

(iv) subsequently becomes available to the public through no fault of the Manager; or

(v) is developed by the Manager independently of the Proprietary Information provided by ReGenX.

(c) In addition, the Manager understands that ReGenX has received and in the future will receive from third parties confidential or proprietary information ("Third Party Information") subject to a duty on the part of ReGenX to maintain the confidentiality of such information and to use it only for certain limited purposes. During the term of the Manager's association with ReGenX and thereafter, the Manager shall hold all Third Party Information in the strictest confidence and shall not disclose or use Third Party Information, except as required in direct connection with the Manager's performance for ReGenX as a Board member or as expressly authorized in writing by an officer of ReGenX.

4. **Return of Documents**. At the written request of ReGenX, the Manager shall return to ReGenX any and all materials and physical documents, whether prepared by ReGenX or its affiliates or by the Manager, if such materials or documents include or incorporate in any way Proprietary Information. The term document is used in its broadest sense and includes electronic information in the form of discs, tapes and the like.

5. **No License**. No rights or licenses in or to Proprietary Information are granted to the Manager by virtue of this Agreement.

6. **No Improper Use of Materials**. The Manager agrees not to bring to ReGenX or to use in the performance of services for ReGenX under this Agreement any materials or documents of a present or former employer of the Manager, or any materials or documents that the Manager obtained from a third party under a binder of confidentiality, unless such materials or documents are generally available to the public or the Manager has authorization from such present or former employer or third party for the possession and unrestricted use of such materials. The Manager understands that the Manager is not to breach any obligation of confidentiality that the Manager has to present or former employers or clients, and agrees to fulfill all such obligations during the term of the Manager's service on the Board of Managers.

7. **Miscellaneous**. This Agreement constitutes the entire and exclusive agreement between the Manager and ReGenX with respect to the subject matter hereof and supersedes any prior or contemporaneous agreements, representations and understandings of the parties with respect thereto. No supplement, modification or amendment of the arrangement described in this Agreement shall be binding upon the Manager or ReGenX or unless set forth in a writing and executed by the Manager and ReGenX. The obligations described under Sections 3, 4 and 6 shall survive the termination of this Agreement. This Agreement and the obligation hereunder shall not be assignable, except that ReGenX may assign this Agreement and the obligations hereunder to any person or entity acquiring all or substantially all of the assets of ReGenX or the assets used in the Business. This Agreement shall be governed by the laws of the State of Delaware without regard to any otherwise applicable principles of conflicts of law.

[*signature page follows*]

IN WITNESS WHEREOF, the Parties hereto have executed this Agreement as of the date first written above.

REGENX BIOSCIENCES, LLC

By: /s/ Kenneth T. Mills
Name: Kenneth T. Mills
Title: President & CEO

/s/ Don Hayden
Mr. Don Hayden

Exhibit 16.1

Baker Tilly Virchow Krause, LLP
8219 Leesburg Pike, Suite 800
Tysons Corner, VA 22182-2625
tel 703 923 8300
fax 703 923 8330
bakertilly.com

July 1, 2015

Securities and Exchange Commission
100 F Street, N.E.
Washington, DC 20549

Commissioners:

We have read the change in accountants disclosure pursuant to Item 304 of Regulation S-K, captioned “Change in Independent Accountant” in the Registration Statement on Form S-1 of REGENXBIO Inc., dated July 1, 2015, as may be amended from time to time. We agree with the statements concerning our firm contained therein. We have no basis to agree or disagree with the statements seen elsewhere within the Form S-1.

Sincerely,

BAKER TILLY VIRCHOW KRAUSE, LLP

Exhibit 23.1

CONSENT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

We hereby consent to the use in this Registration Statement on Form S-1 of REGENXBIO Inc. of our report dated July 1, 2015 relating to the financial statements which appears in such Registration Statement. We also consent to the reference to us under the heading “Experts” in such Registration Statement.

/s/ PricewaterhouseCoopers LLP

McLean, VA
August 17, 2015