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Registration No. 333-206430
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PROSPECTUS

6,300,000 Shares

Common Stock

REGENXBIO Inc. is offering 6,300,000 shares of common stock. This is our initial public offering, and no public market currently exists for our common stock. The initial public offering price is $22.00 per share.

Our common stock has been approved for listing on the Nasdaq Global Select 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	*$22.00*	*$138,600,000.00*
Underwriting discount and commissions(1)	*$1.54*	*$9,702,000.00*
Proceeds, before expenses, to us	*$20.46*	*$128,898,000.00*

(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 945,000 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 September 22, 2015.

MORGAN STANLEY *BofA MERRILL LYNCH* *PIPER JAFFRAY*

CHARDAN CAPITAL MARKETS, LLC

The date of this prospectus is September 16, 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, including an estimated 11,000 individuals who we believe may be primary candidates for gene therapy based on disease severity and molecular characteristics. 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	6,300,000 Shares
Common stock to be outstanding after this offering	25,350,708 Shares
Option to purchase additional common stock offered by us	945,000 Shares
Use of proceeds	We estimate that we will receive net proceeds from this offering of approximately $125.6 million, based on the initial public offering price of $22.00 per share, and after deducting the underwriting discount 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 $144.9 million. As of June 30, 2015, we had cash and cash equivalents of $85.2 million. We intend to use the 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.
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
- 2,025,000 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 254,000 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)
Balance sheet data:			
Cash and cash equivalents	$ 85,215	$ 85,215	$ 210,813
Working capital	81,051	81,051	206,649
Total assets	88,800	88,800	214,398
Accrued expenses	3,062	3,062	3,062
Other related party payables	1,919	1,919	1,919
Total liabilities	6,172	6,172	6,172
Convertible preferred stock	111,392	—	—
Total stockholders' equity (deficit)	(28,764)	82,628	208,226

(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 6,300,000 shares of our common stock offered in this offering, based on the initial public offering price of $22.00 per share, and after deducting underwriting discounts and commissions and 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 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 $125.6 million, based on the initial public offering price of $22.00 per share, and 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 as adjusted 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 $13.83 per share, based on the initial public offering price of $22.00 per share. 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 47.9% 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 25,350,708 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 6,269,300 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 (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District 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. Additionally, if the subject matter of any action within the scope of the preceding sentence is filed in a court other than a court located with the State of Delaware (a Foreign Action) in the name of any stockholder, such stockholder shall be deemed to have consented to (i) the personal jurisdiction of the state and federal courts located within the State of Delaware in connection with any action brought in any such court to enforce the preceding sentence and (ii) having service of process made upon such stockholder in any such action by service upon such stockholder's counsel in the Foreign Action as agent for such stockholder. 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 $125.6 million, based on the initial public offering price of $22.00 per share, and after deducting 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 $144.9 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, as follows:

- approximately $15.0 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 $13.0 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 $10.0 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 $23.0 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 6,300,000 shares of our common stock in this offering, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, at the initial public offering price of $22.00 per share.

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	$ 210,813
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; 100,000,000 shares authorized and 25,350,708 shares issued and outstanding pro forma as adjusted	—	2	3
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	247,334
Accumulated deficit	(39,110)	(39,110)	(39,110)
Total stockholders' (deficit) equity	(28,764)	82,628	208,227
Total capitalization	$ 82,628	$ 82,628	$ 208,227

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
- 2,025,000 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 254,000 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 underwriting discounts and commissions and estimated offering expenses payable by us, at the initial public offering price of $22.00 per share, our pro forma as adjusted net tangible book value as of June 30, 2015 would have been approximately $207.2 million, or approximately $8.17 per share of common stock. This represents an immediate increase in pro forma as adjusted net tangible book value of $3.89 per share to our existing stockholders and an immediate dilution of $13.83 per share to investors participating in this offering. The following table illustrates this per share dilution:

Initial public offering price per share		$22.00
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	3.89	
Pro forma as adjusted net tangible book value per share after this offering		8.17
Pro forma as adjusted dilution per share to purchasers of common stock in this offering		$13.83

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 $8.61 per share, the

increase in pro forma net tangible book value per share to existing stockholders would be $4.33 per share and the dilution to purchasers of common stock in this offering would be $13.39 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 underwriting discounts and commissions and estimated offering expenses, at the initial public offering price of $22.00 per share.

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	19,050,708	75.1%	$116,639,618	45.7%	$ 6.12
Purchasers of common stock in this offering	6,300,000	24.9%	138,600,000	54.3%	$ 22.00
Totals	25,350,708	100.0%	$255,239,618	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
- 2,025,000 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 254,000 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 the initial public offering price of $22.00 per share, the aggregate intrinsic value of outstanding options to purchase shares of our common stock as of June 30, 2015 was $61.7 million, of which $10.3 million related to vested options and $51.4 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

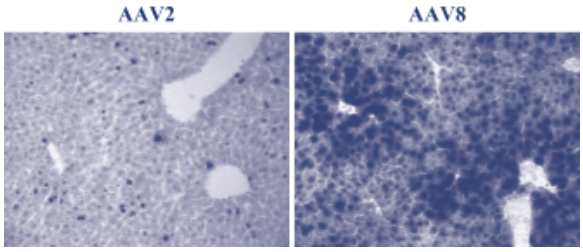
Key Potential Benefits of NAV Technology

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.

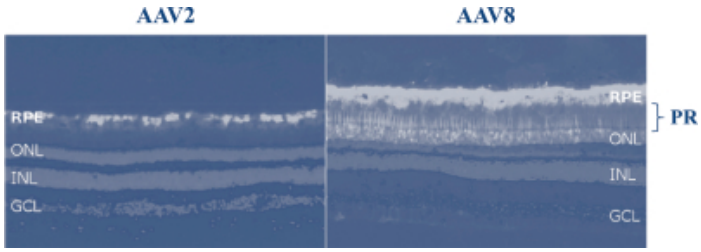
Broad and Novel Tissue Selectivity

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. Based on disease severity and molecular characteristics, we estimate there are approximately 11,000 individuals globally who are primary candidates for gene therapy treatment of HoFH. 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 and August 2015, respectively, the European Commission and the FDA approved Repatha (Amgen) for the treatment of high cholesterol and HoFH, among other indications. In July 2015, the FDA also approved Praluent (Sanofi-Aventis) for the treatment of high cholesterol. Repatha and Praluent represent the first drug

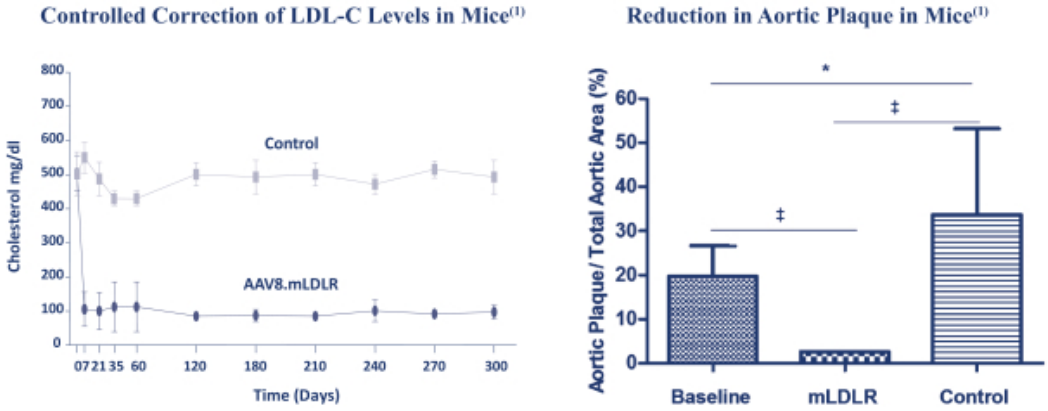
approvals in a new class of drug called PCSK9 inhibitors. PCSK9 inhibitors are designed to bind to a protein called PCSK9 and inhibit PCSK9 from binding to LDLR on the liver surface. In the absence of PCSK9, there is more LDLR on the surface of the liver to remove LDL-C from the blood. We believe that 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.

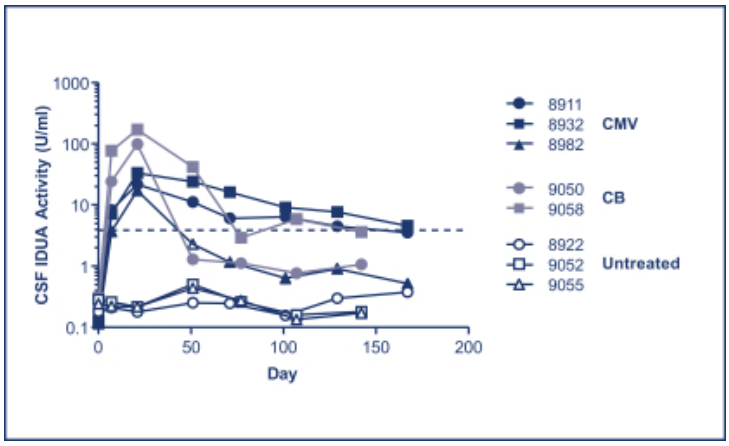
RGX-111

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 the United States and 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
Curran Simpson (1)	53	Senior Vice President, Technical Operations
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. (2),(3)	59	Chairman of the Board of Directors
Luke M. Beshar (2),(3)	57	Director
Edgar G. Engleman, M.D. (4)	68	Director
Allan M. Fox	67	Director
A.N. “Jerry” Karabelas, Ph.D. (4)	62	Director
Camille Samuels (2),(3)	44	Director

(1) Mr. Simpson commenced his employment with us in August 2015.
(2) Member of Audit Committee.
(3) Member of Compensation Committee.
(4) 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.

Curran Simpson has been our Senior Vice President, Technical Operations since August 2015. Prior to joining us, Mr. Simpson was the Head, North American Supply Chain and also served as Interim Chief Operating Officer and Integration Lead with GlaxoSmithKline and Human Genome Sciences division of GlaxoSmithKline (HGS), respectively, from December 2012 until August 2015. From July 2006 to December 2012, Mr. Simpson was the Senior Vice President, Operations at HGS, as well as the Vice President, Manufacturing Operations at HGS from January 2003 to June 2006. Prior to HGS, Mr. Simpson held various positions with Biogen, Inc., Covance Biotechnology Services Inc., Novo-Nordisk Biochem Inc., Genentech, Inc. and Genencor, Inc. Mr. Simpson received an M.S. in Surface and Colloid Science (Physical Chemistry) from Clarkson University and a B.S. in Chemistry/Chemical Engineering from Clarkson College of Technology.

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 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 January 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 June 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. 6,015,300 shares of our common stock were reserved for issuance under our 2015 Plan, which amount equals the sum of 2,025,000 shares plus up to 3,990,300 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:

- four 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.

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 1,500,000 shares in any calendar year, except that a new employee may receive stock options or stock appreciation rights covering up to 500,000 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 1,500,000 shares during any calendar year, except that a new employee may receive restricted shares or stock units covering up to 500,000 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, 2015 and May 14, 2015 to increase the number of shares available for issuance under the 2014 Plan. 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 2015, 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 254,000 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 1,600 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 September 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 September 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 September 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
- 2,025,000 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 254,000 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 25,360,858 shares of common stock outstanding upon completion of this offering, assuming no exercise of the underwriters' option to purchase up to an aggregate of 945,000 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 September 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%	3,221,048	12.7%
Entities Affiliated with John Daniel Kiser(2) 750 17th St., NW, Suite 1100 Washington, DC 20006	2,280,110	12.0%	2,280,110	9.0%
Entities Affiliated with Venrock Partners(3) 3340 Hillview Avenue Palo Alto, CA 94304	1,816,907	9.5%	1,816,907	7.2%
Brookside Capital Partners Fund, L.P.(4) John Hancock Tower 200 Clarendon Street Boston, MA 02116	1,759,961	9.2%	1,759,961	6.9%
Deerfield Private Design Fund III, L.P.(5) 780 Third Avenue 37th floor New York, NY 10017	1,169,042	6.1%	1,169,042	4.6%
Beacon Bioventures Fund III Limited Partnership(6) 82 Devonshire Street, MZ EPC 13A Boston, MA 02109	1,085,816	5.7%	1,085,816	4.3%
GlaxoSmithKline LLC(7) 2301 Renaissance Blvd. Mail Code RN0220 King of Prussia, PA 19406	1,085,824	5.7%	1,085,824	4.3%
Directors and Named Executive Officers				
Kenneth T. Mills(8)	337,634	1.8%	337,634	1.3%
Vittal Vasista(9)	292,517	1.5%	307,400	1.2%
Stephen Yoo, M.D.(10)	88,978	*	88,978	*
Donald J. Hayden, Jr.(11)	245,191	1.3%	245,191	1.0%
Luke Beshar(12)	11,667	*	11,667	*
Edgar G. Engleman, M.D.(13)	940,449	4.9%	940,449	3.7%
Allan M. Fox(1)	3,221,048	16.9%	3,221,048	12.7%
A.N. “Jerry” Karabelas, Ph.D.(14)	16,250	*	16,250	*
Camille Samuels(15)	—	*	—	*
All current directors and executive officers as a group (10 persons)(16)	5,153,734	27.0%	5,168,617	20.4%

* 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 VII) is the sole general partner of VA VII and VP VII. VHCP Management II, LLC (VHCPM II) is the sole general partner of VHCP II and the manager of VHCP Co. II. VM 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 Bong Y. Koh are members of VHCPM II and disclaim beneficial ownership over all shares held by VHCP II and VHCP Co. II, except to the extent of their indirect pecuniary interests therein. The percentage of shares beneficially owned after this offering would be 7.9%, assuming the purchase of 175,000 shares that Venrock Partners or its affiliates have agreed to purchase in this offering at the initial public offering price.

(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. The percentage of shares beneficially owned after this offering would be 7.6%, assuming the purchase of 175,000 shares that Brookside Capital Partners Fund, L.P. or its affiliates have agreed to purchase in this offering at the initial public offering price.

(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. The percentage of shares beneficially owned after this offering would be 5.3%, assuming the purchase of 175,000 shares that Deerfield Private Design Fund III, L.P. or its affiliates have agreed to purchase in this offering at the initial public offering price.

(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. Assuming the purchase of 325,000 shares that entities that may be deemed

to be affiliated with Beacon Fund have agreed to purchase in this offering at the initial public offering price, the percentage of shares beneficially owned after this offering would be 5.6%.

(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 322,194 shares of common stock that may be exercised within 60 days of September 1, 2015.

(9) Consists of 115,440 shares of common stock and common stock issuable upon conversion of preferred stock and includes (i) options to purchase 177,077 shares of common stock that may be exercised within 60 days of September 1, 2015 prior to the offering and (ii) options to purchase 191,960 shares of common stock that may be exercised within 60 days of September 1, 2015 after the offering, including 14,883 shares which vest upon the consummation of the offering.

(10) Includes options to purchase 88,978 shares of common stock that may be exercised within 60 days of September 1, 2015.

(11) Consists of 38,599 shares of common stock issuable upon conversion of preferred stock and includes options to purchase 206,592 shares of common stock that may be exercised within 60 days of September 1, 2015.

(12) Consists of options to purchase 11,667 shares of common stock that may be exercised within 60 days of September 1, 2015.

(13) 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.

(14) Consists of options to purchase 16,250 shares of common stock that may be exercised within 60 days of September 1, 2015.

(15) Ms. Samuels is affiliated with Venrock Partners. Ms. Samuels does not have voting or dispositive control over the shares held by the entities affiliated with Venrock Partners referenced in footnote 3 above.

(16) Consists of 5,168,617 shares of common stock and common stock issuable upon conversion of preferred stock and includes options to purchase 837,641 shares of common stock that may be exercised within 60 days of September 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 514,762 were vested and exercisable as of that date.

In September 2015, our board of directors approved the grant of stock options to purchase an aggregate of 331,000 shares of common stock under the 2015 Plan, effective as of the date of this prospectus, at a per share exercise price equal to the initial public offering price, subject to vesting based upon the optionee's continued service with us.

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 (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District 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. Additionally, if the subject matter of any action within the scope of the preceding sentence is filed in a court other than a court located with the State of Delaware (a Foreign Action) in the name of any stockholder, such stockholder shall be deemed to have consented to (i) the personal jurisdiction of the state and federal courts located within the State of Delaware in connection with any action brought in any such court to enforce the preceding sentence and (ii) having service of process made upon such stockholder in any such action by service upon such stockholder's counsel in the Foreign Action as agent for such stockholder. 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 Select Market

Our common stock has been approved for listing on the NASDAQ Global Select 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 25,350,708 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 25,350,708 shares, the 6,300,000 shares (7,245,000 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 September 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 253,507 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	2,583,000
Merrill Lynch, Pierce, Fenner & Smith Incorporated	2,205,000
Piper Jaffray & Co.	1,008,000
Chardan Capital Markets, LLC	504,000
Total:	6,300,000

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 945,000 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 945,000 shares of common stock.

	Per Share	Total	
		No Exercise	Full Exercise
Public offering price	$ 22.00	$ 138,600,000.00	$ 159,390,000.00
Underwriting discounts and commissions to be paid by us	$ 1.54	$ 9,702,000.00	$ 11,157,300.00
Proceeds, before expenses, to us	$ 20.46	$ 128,898,000.00	$ 148,232,700.00

The estimated offering expenses payable by us, exclusive of the underwriting discounts and commissions, are approximately $3.3 million. We have agreed to reimburse the underwriters for expense relating to clearance of this offering with the Financial Industry Regulatory Authority up to $50,000.

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.

Our common stock has been approved for quotation on the NASDAQ Global Select 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.

Canada

The shares may be sold only to purchasers purchasing, or deemed to be purchasing, as principal that are accredited investors, as defined in National Instrument 45-106 Prospectus Exemptions or subsection 73.3(1) of the Securities Act (Ontario), and are permitted clients, as defined in National Instrument 31-103 Registration Requirements, Exemptions and Ongoing Registrant Obligations. Any resale of the shares must be made in accordance with an exemption from, or in a transaction not subject to, the prospectus requirements of applicable securities laws.

Securities legislation in certain provinces or territories of Canada may provide a purchaser with remedies for rescission or damages if this prospectus (including any amendment thereto) contains a misrepresentation, provided that the remedies for rescission or damages are exercised by the purchaser within the time limit prescribed by the securities legislation of the purchaser's province or territory. The purchaser should refer to any applicable provisions of the securities legislation of the purchaser's province or territory for particulars of these rights or consult with a legal advisor.

Pursuant to section 3A.3 (or, in the case of securities issued or guaranteed by the government of a non-Canadian jurisdiction, section 3A.4) of National Instrument 33-105 Underwriting Conflicts (NI 33-105), the underwriters are not required to comply with the disclosure requirements of NI 33-105 regarding underwriter conflicts of interest in connection with this offering.

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)
Notes to Financial Statements
(In thousands, except per share data)

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.

REGENXBIO Inc. (formerly ReGenX Biosciences, LLC)
Notes to Financial Statements
(In thousands, except per share data)

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

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

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

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

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,

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

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

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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6,300,000 Shares

REGENXBIO Inc.

Common Stock

PROSPECTUS

MORGAN STANLEY *BofA MERRILL LYNCH* *PIPER JAFFRAY*

CHARDAN CAPITAL MARKETS, LLC

September 16, 2015

Until October 11, 2015, 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.