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

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**FORM 10-Q**

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(Mark One)

**QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

For the quarterly period ended September 30, 2015

OR

**TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

Commission File Number 001-37553

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**REGENXBIO Inc.**

(Exact Name of Registrant as Specified in its Charter)

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**Delaware**  
(State or other jurisdiction of  
incorporation or organization)

**47-1851754**  
(I.R.S. Employer  
Identification No.)

**9712 Medical Center Drive, Suite 100**  
**Rockville, MD**  
(Address of principal executive offices)

**20850**  
(Zip Code)

**(240) 552-8181**  
(Registrant's telephone number, including area code)

**Not Applicable**  
(Former name, former address and former fiscal year, if changed since last report)

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Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes  No

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

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

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

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes  No

As of November 5, 2015, there were 26,313,471 outstanding shares of the registrant's common stock, \$0.0001 par value per share.

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**REGENXBIO INC.  
QUARTERLY REPORT ON FORM 10-Q  
FOR THE QUARTERLY PERIOD ENDED SEPTEMBER 30, 2015**

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## INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains “forward-looking statements” that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q, including statements regarding our future results of operations and financial position, strategy and plans and our expectations for future operations, are forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. The words “anticipate,” “believe,” “contemplates,” “continue,” “could,” “design,” “estimate,” “expect,” “intend,” “likely,” “may,” “ongoing,” “plan,” “potential,” “predict,” “project,” “will,” “would,” “seek,” “should,” “target,” or the negative version of these words and similar expressions are intended to identify forward-looking statements. We have based these forward-looking statements on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, strategy, short- and long-term business operations and objectives and financial needs. These forward-looking statements are subject to a number of risks, uncertainties, assumptions and other important factors, including those described in the section titled “Risk Factors” and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in this Quarterly Report on Form 10-Q and our prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on September 17, 2015 (the Prospectus). In light of these risks, uncertainties, assumptions and other factors, the forward-looking events and circumstances discussed in this Quarterly Report on Form 10-Q or the Prospectus may not occur, and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. 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 plans for the use of our cash and cash equivalents.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, level of activity, performance or achievements. Any forward-looking statement made by us in this Quarterly Report on Form 10-Q speaks only as of the date of this report. Except as required by law, we disclaim any duty to update any of these forward-looking statements after the date such statements are made, or to update the reasons actual results could differ materially from those anticipated in these forward-looking statements, even if new information becomes available in the future.

## PART I – FINANCIAL INFORMATION

## Item 1. Financial Statements.

**REGENXBIO INC.**  
**BALANCE SHEETS**  
**(unaudited)**  
**(in thousands, except per share data)**

	December 31, 2014	September 30, 2015
<b>Assets</b>		
Current assets		
Cash and cash equivalents	\$ 1,121	\$ 205,324
Marketable securities	—	8,025
Accounts receivable		
Trade receivables	805	609
Related party receivables	750	—
Unbilled receivables	327	—
Prepaid expenses	28	1,400
Other current assets	—	127
Total current assets	3,031	215,485
Marketable securities	—	11,013
Property and equipment, net	—	385
Cost method investments	303	303
Deferred issuance costs	157	—
Other assets	—	128
Total assets	<u>\$ 3,491</u>	<u>\$ 227,314</u>
<b>Liabilities, Convertible Preferred Stock and Stockholders' Equity (Deficit)</b>		
Current liabilities		
Accounts payable	\$ 334	\$ 3,105
Accrued expenses	1,115	1,727
Due to related party under services agreement	1,423	—
Related party promissory notes	2,403	—
Other related party payables	3,761	349
Advance payments	153	127
Total current liabilities	9,189	5,308
Deferred rent	—	198
Total liabilities	9,189	5,506
Commitments and contingencies (Note 7)		
Convertible preferred stock		
Series A convertible preferred stock; \$0.0001 par value; 2,393 shares authorized, issued and outstanding at December 31, 2014; no shares authorized, issued and outstanding at September 30, 2015	3,963	—
Series B convertible preferred stock; \$0.0001 par value; 1,906 shares authorized, issued and outstanding at December 31, 2014; no shares authorized, issued and outstanding at September 30, 2015	8,630	—
Total convertible preferred stock	12,593	—
Stockholders' equity (deficit)		
Preferred stock; \$0.0001 par value; no shares authorized, issued and outstanding at December 31, 2014; 10,000 shares authorized, and no shares issued and outstanding at September 30, 2015	—	—
Common stock; \$0.0001 par value; 9,500 and 100,000 shares authorized at December 31, 2014 and September 30, 2015, respectively; 2,645 and 26,306 shares issued and outstanding at December 31, 2014 and September 30, 2015, respectively	—	3
Additional paid-in capital	10,518	268,275
Accumulated other comprehensive loss	—	(26)
Accumulated deficit	(28,809)	(46,444)
Total stockholders' equity (deficit)	(18,291)	221,808
Total liabilities, convertible preferred stock and stockholders' equity (deficit)	<u>\$ 3,491</u>	<u>\$ 227,314</u>

*See accompanying notes to unaudited financial statements.*

**REGENXBIO INC.**  
**STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS**  
(unaudited)  
(in thousands, except per share data)

	Three Months Ended		Nine Months Ended	
	September 30,		September 30,	
	2014	2015	2014	2015
<b>Revenues</b>				
License revenue	\$ —	\$ 65	\$ 3,705	\$ 635
License revenue from related party	150	1,000	150	2,000
Reagent sales	23	61	317	200
Grant revenue	310	14	800	305
Total revenues	483	1,140	4,972	3,140
<b>Expenses</b>				
Costs of revenues				
Licensing costs to related parties	—	213	741	527
Costs of reagent sales (including amounts to related parties)	12	44	115	94
Research and development (including amounts to related parties)	1,085	5,664	2,869	12,471
General and administrative (including amounts to related parties)	1,070	2,567	2,732	7,671
Foreign currency transaction losses (gains)	25	4	12	41
Other operating income	(8)	(5)	(32)	(26)
Total operating expenses	2,184	8,487	6,437	20,778
Loss from operations	(1,701)	(7,347)	(1,465)	(17,638)
<b>Other Income (Expense)</b>				
Investment income	—	15	—	23
Interest expense	(92)	—	(202)	(20)
Total other income (expense)	(92)	15	(202)	3
Net loss	<u>\$ (1,793)</u>	<u>\$ (7,332)</u>	<u>\$ (1,667)</u>	<u>\$ (17,635)</u>
<b>Other Comprehensive Loss</b>				
Unrealized loss on available-for-sale securities	—	(26)	—	(26)
Total other comprehensive loss	—	(26)	—	(26)
Comprehensive loss	<u>\$ (1,793)</u>	<u>\$ (7,358)</u>	<u>\$ (1,667)</u>	<u>\$ (17,661)</u>
<b>Reconciliation of net loss to net loss applicable to common stockholders</b>				
Net loss	\$ (1,793)	\$ (7,332)	\$ (1,667)	\$ (17,635)
Accretion and dividends on convertible preferred stock	(126)	—	(592)	(1,747)
Net gain on extinguishment of convertible preferred stock	—	—	—	759
Net loss applicable to common stockholders	<u>\$ (1,919)</u>	<u>\$ (7,332)</u>	<u>\$ (2,259)</u>	<u>\$ (18,623)</u>
Basic and diluted net loss per common share	<u>\$ (0.73)</u>	<u>\$ (1.52)</u>	<u>\$ (0.85)</u>	<u>\$ (5.48)</u>
Weighted-average basic and diluted common shares	<u>2,643</u>	<u>4,809</u>	<u>2,643</u>	<u>3,397</u>

*See accompanying notes to unaudited financial statements.*

**REGENXBIO INC.**  
**STATEMENTS OF CASH FLOWS**  
**(unaudited)**  
**(in thousands)**

	<b>Nine Months Ended</b>	
	<b>September 30,</b>	
	<b>2014</b>	<b>2015</b>
<b>Cash flows from operating activities</b>		
Net loss	\$(1,667)	\$ (17,635)
Adjustments to reconcile net loss to net cash used in operating activities		
Unrealized foreign currency transaction gains	(25)	(4)
Stock-based compensation expense	220	2,059
Imputed interest on related party promissory notes	45	13
Other non-cash adjustments	1	—
Depreciation and amortization	—	43
Amortization of premiums on marketable debt securities	—	1
Changes in operating assets and liabilities		
Trade receivables	(1,097)	200
Related party receivables	774	750
Unbilled receivables	30	327
Prepaid expenses	(131)	(1,372)
Other current assets	—	(127)
Other assets	—	(128)
Accounts payable	440	317
Accrued expenses	295	758
Due to related party under services agreement	(255)	(34)
Other related party payables	(1,335)	(3,412)
Advance payments	168	(26)
Deferred rent	—	198
Net cash used in operating activities	<u>(2,537)</u>	<u>(18,072)</u>
<b>Cash flows from investing activities</b>		
Purchases of marketable securities	—	(19,065)
Purchases of property and equipment	—	(394)
Net cash used in investing activities	<u>—</u>	<u>(19,459)</u>
<b>Cash flows from financing activities</b>		
Proceeds from related party promissory notes	2,400	—
Proceeds from exercise of stock options	—	100
Proceeds from issuance of Series C convertible preferred stock, net of transaction costs	—	26,021
Proceeds from issuance of Series D convertible preferred stock, net of transaction costs	—	67,998
Proceeds from initial public offering of common stock, net of underwriting discounts and commissions	—	148,233
Payments of initial public offering costs	—	(618)
Net cash provided by financing activities	<u>2,400</u>	<u>241,734</u>
Net increase (decrease) in cash and cash equivalents	(137)	204,203
<b>Cash and cash equivalents</b>		
Beginning of period	1,119	1,121
End of period	<u>\$ 982</u>	<u>\$205,324</u>
<b>Supplemental cash flow information</b>		
Cash paid for interest	\$ 164	\$ 7
<b>Supplemental disclosures of non-cash investing and financing activities</b>		
Conversion of accrued service fees into Series C convertible preferred stock	\$ —	\$ 2,403
Conversion of related party promissory notes into Series C convertible preferred stock	\$ —	\$ 1,389
Conversion of convertible preferred stock into common stock upon initial public offering	\$ —	\$111,392
Purchases of property and equipment in accounts payable	\$ —	\$ 34
Issuance costs for initial public offering in accounts payable and accrued expenses	\$ —	\$ 2,431

*See accompanying notes to unaudited financial statements.*

**REGENXBIO INC.**  
**NOTES TO FINANCIAL STATEMENTS**  
**(unaudited)**  
**(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.

***Initial Public Offering***

On September 22, 2015, the Company completed its initial public offering (IPO) whereby the Company sold 7,245 shares of common stock (inclusive of 945 shares of common stock sold by the Company pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) at a price of \$22.00 per share. The shares began trading on the Nasdaq Global Select Market on September 17, 2015. The aggregate net proceeds received by the Company from the offering were \$145,184, net of underwriting discounts and commissions and offering expenses payable by the Company. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,298 shares of common stock.

***Liquidity and Risks***

As of September 30, 2015, the Company has generated an accumulated deficit of \$46,444 since inception. 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.

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 trials, dependence on key personnel, protection of proprietary technology, compliance with government regulations and ability to transition from preclinical manufacturing to commercial production of products.

**2. Summary of Significant Accounting Policies**

***Basis of Presentation and Unaudited Interim Financial Information***

The accompanying financial statements are unaudited and 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). Certain information and footnote disclosures normally included in the Company's annual financial statements have been condensed or omitted. The interim unaudited financial statements have been prepared on the same basis as the annual audited financial statements as of and for the year ended December 31, 2014 included in the prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on September 17, 2015 (the Prospectus). In the opinion of management, the accompanying financial statements reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of September 30, 2015, and the results of its operations and its cash flows for the interim periods ended September 30, 2014 and 2015.

The results of operations for the interim periods are not necessarily indicative of the results of operations to be expected for the full year, any other interim periods, or any future year or period. These interim financial statements should be read in conjunction with the audited financial statements as of and for the year ended December 31, 2014, and the notes thereto, which are included in the Prospectus.

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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.

### *Marketable Securities*

The Company's marketable securities are classified as available-for-sale and are carried at fair value. Marketable securities with remaining maturity dates exceeding twelve months which are not intended to be sold prior to maturity for use in current operations are classified as non-current. Unrealized gains and losses, net of any related tax effects, are excluded from earnings and are included in other comprehensive loss and reported as a separate component of stockholders' equity (deficit) until realized. A decline in the fair value below cost of available-for-sale securities that is deemed other-than-temporary is charged to results of operations, resulting in the establishment of a new cost basis for the security. Premiums and discounts are amortized or accreted into the cost basis over the life of the related security as adjustments to the yield using the effective-interest method. Interest income is recognized when earned. Realized gains and losses from the sale of marketable securities are based on the specific identification method and are included in results of operations.

The Company regularly evaluates whether declines in the fair value of its investments below their cost are other-than-temporary. The evaluation includes consideration of the cause of the impairment, including the creditworthiness of the security issuers, the number of securities in an unrealized loss position, the severity and duration of the unrealized losses, whether the Company has the intent to sell the securities and whether it is more likely than not that the Company will be required to sell the securities before the recovery of their amortized cost basis. If the Company determines that the decline in fair value of an investment is below its accounting basis and this decline is other-than-temporary, the Company would reduce the carrying value of the security it holds and record a loss for the amount of such decline. The Company has not recorded any declines in value judged to be other-than-temporary on its investments.

### *Concentrations of Credit Risk and Off-balance Sheet Risk*

Cash and cash equivalents, marketable securities 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 multiple 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. The Company's marketable securities consist of fixed income corporate debt securities and may subject the Company to concentrations of credit risk. The Company has adopted an investment policy, under which its marketable securities are purchased, which limits potential concentrations of investments and establishes minimum acceptable credit ratings, thereby reducing credit risk exposure. 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.

### *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.



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- 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. Please refer to Note 4 for further information on the fair value measurement of the Company’s financial instruments.

### Net Loss Per Share

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 outstanding prior to the IPO to be participating securities. The holders of convertible preferred stock outstanding prior to the IPO were entitled to receive preferential dividends in the event that a dividend was to be paid to the holders of common stock. The holders of convertible preferred stock outstanding prior to the IPO did not have a contractual obligation to share in the losses of the Company. As such, the Company’s net losses for the three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, were not allocated to these participating securities. In connection with the IPO, all outstanding shares of convertible preferred stock were automatically converted into shares of common stock.

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 outstanding at the end of the period were excluded from the computations of diluted weighted-average shares outstanding for the periods indicated as they would be anti-dilutive:

	Three and Nine Months Ended September 30,	
	2014	2015
Series A convertible preferred stock	2,393	—
Series B convertible preferred stock	1,906	—
Stock options issued and outstanding	1,885	3,464
Debt with share settlement option (Note 6)	3,295	—
	<u>9,479</u>	<u>3,464</u>

Amounts in the table above reflect the common stock equivalents of the noted instruments.

### Comprehensive Loss

The Company’s comprehensive loss includes its net loss as well as net unrealized losses on available-for-sale securities.

### 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

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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. In August 2015, the FASB issued ASU No. 2015-14, *Revenue from Contracts with Customers (Topic 606)*, which deferred the effective date of the guidance under ASU No. 2014-09 for entities by one year. The ASU is now effective for annual and interim reporting periods beginning after December 15, 2017. Earlier application is permitted only as of annual reporting periods beginning after December 15, 2016, including interim reporting periods within that reporting period. 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.

### 3. Marketable Securities

The following table presents a summary of the Company's marketable securities, which consist solely of available-for-sale securities:

	<u>Amortized Cost</u>	<u>Unrealized Gains</u>	<u>Unrealized Losses</u>	<u>Fair Value</u>
<b>September 30, 2015</b>				
Corporate debt securities	\$ 19,064	\$ 2	\$ (28)	\$19,038
	<u>\$ 19,064</u>	<u>\$ 2</u>	<u>\$ (28)</u>	<u>\$19,038</u>

The Company did not hold any available-for-sale securities as of December 31, 2014. As of September 30, 2015, no available-for-sale securities had remaining maturities greater than three years.

The amortized cost of available-for-sale securities is adjusted for amortization of premiums and accretion of discounts to maturity. As of September 30, 2015, the balance in the Company's accumulated other comprehensive loss consisted solely of net unrealized gains and losses on available-for-sale securities. For the nine months ended September 30, 2015, the Company recognized net unrealized losses on available-for-sale securities of \$26, which is included in other comprehensive loss. There were no realized gains or losses recognized on the sale or maturity of available-for-sale securities during the nine months ended September 30, 2015, and as a result, the Company did not reclassify any amounts out of accumulated other comprehensive loss for the period.

As of September 30, 2015, the Company did not hold any available-for-sale securities in an unrealized loss position for more than twelve months. The aggregate fair value of securities held by the Company in an unrealized loss position for less than twelve months as of September 30, 2015 was \$18,012, which consisted of corporate debt securities with credit ratings of A-2/A or higher. The Company has the intent and ability to hold such securities until recovery. The Company determined that there have been no material changes in the credit risk of these investments since their initial purchases. As a result, the Company determined that none of these investments are other-than-temporarily impaired as of September 30, 2015.

### 4. Fair Value of Financial Instruments

Financial instruments reported at fair value on a recurring basis include cash equivalents and marketable securities. Cash equivalents consist solely of money market mutual funds. The following tables present the fair value of cash equivalents and marketable securities in accordance with the hierarchy discussed in Note 2:

	<u>Quoted prices in active markets (Level 1)</u>	<u>Significant other observable inputs (Level 2)</u>	<u>Significant unobservable inputs (Level 3)</u>	<u>Total</u>
<b>December 31, 2014</b>				
Money market mutual funds (cash equivalents)	\$ —	\$ 844	\$ —	\$844
	<u>\$ —</u>	<u>\$ 844</u>	<u>\$ —</u>	<u>\$844</u>

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	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
<b>September 30, 2015</b>				
Money market mutual funds (cash equivalents)	\$ —	\$205,324	\$ —	\$205,324
Corporate debt securities (marketable securities)	—	19,038	—	19,038
	<u>\$ —</u>	<u>\$224,362</u>	<u>\$ —</u>	<u>\$224,362</u>

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 6) outstanding at December 31, 2014 accrued 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.

### 5. Property and Equipment, Net

Property and equipment, net consists of the following as of September 30, 2015:

	September 30, 2015
Computer equipment and software	\$ 332
Furniture and fixtures	94
Leasehold improvements	2
Total property and equipment	428
Accumulated depreciation and amortization	(43)
Property and equipment, net	<u>\$ 385</u>

As of December 31, 2014, the Company had recorded no property and equipment because the Company's resources used in operations were provided by a related party (Note 12).

### 6. Related Party Debt Instruments

As of December 31, 2014, the Company had accrued \$1,423 payable to FoxKiser, LLP (FoxKiser) under a services agreement (Note 12), which may be settled in preferred or common shares. Additionally, the Company had \$2,403 of principal and interest due under related party promissory notes to FoxKiser, which also may be settled in preferred or common shares.

On January 13, 2015, in conjunction with the Company's issuance of Series C Preferred Stock (Note 8), 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 September 30, 2015.

### 7. Commitments and Contingencies

#### *Lease Agreements*

The Company recognizes rent expense on a straight-line basis over the term of its operating leases.

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 September 30, 2015 had an expiration date of December 31, 2015. Monthly rent under the lease agreement is \$4.

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Effective January 31, 2015, the services agreement with FoxKiser (Note 12) was terminated and the Company entered into an operating lease with FoxKiser for office space in Washington, D.C. The lease agreement, which had a month-to-month term, required monthly payments of \$20. The lease was terminated in April 2015.

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 were \$24 and escalate annually in accordance with the lease.

In September 2015, the Company amended its operating lease in Rockville, Maryland to include additional office and laboratory space, and extend the term of the existing lease to October 2020. The lease for the additional space is expected to commence in March 2016, and has a 5-year term. The Company has options to extend the lease for the additional space to be coterminous with the Company's existing lease at that facility. Initial monthly payments required under the lease for the additional space are \$41 and escalate annually in accordance with the lease.

As of September 30, 2015, future minimum lease payments under non-cancelable operating leases are as follows:

	<b>Operating Leases</b>
2015 (remainder of year)	\$ 73
2016	721
2017	825
2018	850
2019	875
2020	833
Thereafter	96
Total minimum lease payments	<u>\$ 4,273</u>

### ***License Agreements Granted to the Company***

License agreements granted to the Company may require the Company to make future payments relating to sublicense fees, milestone fees for milestones not met as of December 31, 2014 and September 30, 2015 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.

*The Trustees of the University of Pennsylvania.* The Company has licensed intellectual property from The Trustees of the University of Pennsylvania (Penn). Please refer to Note 12 for further information regarding this agreement.

*GlaxoSmithKline LLC.* The Company has licensed intellectual property from GlaxoSmithKline LLC (GSK). Please refer to Note 12 for further information regarding this agreement.

*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, 2014 and September 30, 2015, 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 September 30, 2015, 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.

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***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, 2014 and September 30, 2015, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded any related liabilities.

**8. Capitalization**

As of September 30, 2015, the authorized capital stock of the Company included 100,000 shares of common stock, par value \$0.0001 per share, and 10,000 shares of preferred stock, par value \$0.0001 per share. The Company's restated certificate of incorporation and bylaws, which were adopted in connection with the IPO, contain the rights, preferences and privileges of the Company's stockholders and their respective shares.

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.

***Issuance of Series C Preferred Stock***

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 of \$3,792 of debt as discussed in Notes 6 and 12. 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***

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 the Black-Scholes option-pricing model.

***Issuance of Series D Preferred Stock***

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.

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In connection with the issuance of the Series D Preferred Stock, the Company amended and restated its certificate of incorporation. 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. Under the amended and restated certificate of incorporation in effect prior to the IPO, the rights, preferences and privileges of Preferred Stock consisted of the following:

*Dividends.* The holders of Series D Preferred Stock were 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 Company's Board of Directors. After payment of dividends to holders of Series D Preferred Stock, holders of Series C Preferred Stock were 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 were entitled to receive dividends in preference to common stock. Dividends to holders of Preferred Stock were non-cumulative and had 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 were 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 had 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 had 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 had preference in liquidation proceeds over holders of common stock. Liquidation preferences of Preferred Stock were 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 included (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 was \$1.255, \$4.1399, \$6.477 and \$9.5699 per share, respectively. If proceeds from the liquidation event were insufficient to pay the entire liquidation preference to holders of any series of Preferred Stock, then the proceeds were 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 were 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 was entitled to receive with respect to a liquidation event, holders of Preferred Stock were 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 was deemed to have converted shares of 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 Stock that were not converted.

*Redemption.* All series of Preferred Stock were 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 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, Series B Preferred Stock, Series C Preferred Stock and Series D Preferred Stock was \$1.255, \$4.1399, \$6.477 and \$9.5699 per share, respectively.

*Conversion.* Each share of 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 Preferred Stock would be fully settled. Each share of 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, Series B Preferred Stock, Series C Preferred Stock and Series D Preferred Stock was \$1.255, \$4.1399, \$6.477 and \$9.5699 per share, respectively. Initially, 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.

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Each share of Preferred Stock was to automatically convert into shares of common stock at the applicable conversion rate upon (i) a qualified public offering, as defined in the Company's amended and restated 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 had the right to one vote for each share of common stock into which the Preferred Stock could then be converted. Holders of Preferred Stock had full voting rights and powers equal to those of common stockholders.

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. As long as shares of Series C Preferred Stock remained outstanding, the holders of Series C Preferred Stock, voting as a separate class, were entitled to elect one director to the Board of Directors. As long as shares of Series D Preferred Stock remained outstanding, the holders of Series D 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.

Prior to the closing of the IPO on September 22, 2015, all of the outstanding shares of the Company's Preferred Stock were converted into 16,298 shares of its common stock. As of September 30, 2015, the Company did not have any convertible preferred stock issued or outstanding.

### *Common stock*

Prior to the closing of the Company's IPO on September 22, 2015, the dividend, liquidation and voting rights of the Company's common stockholders were subject to, and qualified by, the rights, preferences and privileges of the holders of the outstanding Preferred Stock.

The Company's reserved shares of common stock for future issuance are as follows:

	<b>December 31, 2014</b>	<b>September 30, 2015</b>
Series A convertible preferred stock	2,393	—
Series B convertible preferred stock	1,906	—
Reserved for issuance under equity incentive plans	2,500	6,005
Reserved for issuance under employee stock purchase plan	—	254
Debt with share settlement option (Note 6)	3,715	—
	<u>10,514</u>	<u>6,259</u>

## **9. Significant Agreements**

See Note 12 for significant agreements with related parties and Note 7 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 September 30, 2015, the Company's 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, \$11,300 upon the commencement of various stages of clinical trials, \$20,000 upon the submission of regulatory approval filings, \$51,500 upon the approval of commercial products by regulatory agencies and \$2,000 upon the achievement of specified sales targets for licensed products.

License revenue, including amounts from related parties, recognized for the three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, is as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Up-front fees and option fees for commercial licenses	\$ —	\$ 1,000	\$3,600	\$2,000
Milestone fees for commercial licenses	—	—	—	250
Maintenance fees for commercial licenses	—	65	65	245
Research and other license revenue	150	—	190	140
	<u>\$ 150</u>	<u>\$ 1,065</u>	<u>\$3,855</u>	<u>\$2,635</u>

## 10. Stock-based Compensation

### Equity Incentive Plans

In June 2015, the Company's Board of Directors adopted the 2015 Equity Incentive Plan (2015 Plan). The 2015 Plan replaces the 2014 Stock Plan (2014 Plan). Any options or awards outstanding under the 2014 Plan at the effective date of the 2015 Plan remain outstanding and effective. As of September 30, 2015, the total number of shares of common stock authorized for issuance under the 2015 Plan and 2014 Plan is 6,005, of which 2,541 remain available for future grants.

The 2015 Plan provides for the issuance of stock options, stock appreciation rights, restricted and unrestricted stock awards and performance cash awards to employees, members of the Board of Directors and consultants of the Company. No stock appreciation rights, restricted or unrestricted stock awards or performance cash awards have been granted under the 2014 Plan or the 2015 Plan, since the inception of the plans. The initial amount of shares authorized for issuance under the 2015 Plan was 2,952. The number of authorized shares under the 2015 Plan automatically increases annually on January 1, beginning January 1, 2016, by the lesser of (i) 4% of the total number of shares of common stock outstanding on December 31 of the prior year, or (ii) a number of common shares determined by the Board of Directors. Stock options under the 2015 Plan generally expire ten years following the date of grant. Options typically vest over a four year period, 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. Stock options under the 2015 Plan carry an exercise price at least equal to the estimated fair value of the Company's common stock on the date of grant.

The following table summarizes stock option activity under the Company's equity incentive plans:

	Shares	Weighted- average Exercise Price	Weighted- average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2014	2,107	\$ 0.85	9.8	\$ 379
Granted	1,498	\$ 9.05		
Exercised	(118)	\$ 0.85		
Cancelled or forfeited	(23)	\$ 0.85		
Outstanding at September 30, 2015	<u>3,464</u>	<u>\$ 4.39</u>	<u>9.3</u>	<u>\$ 61,097</u>
Exercisable at September 30, 2015	<u>1,182</u>	<u>\$ 1.20</u>	<u>9.1</u>	<u>\$ 24,629</u>
Vested and expected to vest at September 30, 2015	<u>3,464</u>	<u>\$ 4.39</u>	<u>9.3</u>	<u>\$ 61,097</u>

- (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 September 30, 2015.



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The weighted-average grant date fair value of options granted during the nine months ended September 30, 2015 was \$5.88. During the nine months ended September 30, 2015, the total number of stock options exercised was 118, resulting in total proceeds of \$100. The total intrinsic value of options exercised during the nine months ended September 30, 2015 was \$354.

Stock-based compensation expense for the three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, relates solely to stock options granted under the 2014 Plan and the 2015 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 and comprehensive loss as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Research and development	\$ 16	\$ 1,101	\$ 16	\$ 1,413
General and administrative	204	248	204	646
	<u>\$ 220</u>	<u>\$ 1,349</u>	<u>\$ 220</u>	<u>\$ 2,059</u>

*Stock Options Granted to Employees.* 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:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Expected volatility	65%	75%	65%	67%
Expected term (in years)	5.9	6.2	5.9	6.1
Risk-free interest rate	2.0%	1.8%	2.0%	1.7%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

As of September 30, 2015, there was \$4,999 of unrecognized stock-based compensation expense related to employees' awards that is expected to be recognized over a weighted-average period of 3.2 years.

*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. The Company used the following weighted-average assumptions in estimating non-employees stock-based compensation expense:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Expected volatility	65%	81%	65%	71%
Expected term (in years)	10.0	9.3	10.0	9.7
Risk-free interest rate	2.6%	2.1%	2.6%	2.1%
Expected dividend yield	0.0%	0.0%	0.0%	0.0%

### ***Employee Stock Purchase Plan***

In June 2015, the Company's Board of Directors adopted the 2015 Employee Stock Purchase Plan (2015 ESPP), which became effective on September 16, 2015, the date on which the registration statement for the IPO was declared effective. The 2015 ESPP authorizes the initial issuance of up to a total of 254 shares of the Company's common stock to participating employees. The number of shares reserved for issuance under the 2015 ESPP automatically increases on the first business day of each fiscal year, commencing in 2016, by a number equal to the lesser of (i) 1% of the shares of common stock outstanding on the last business day of the prior fiscal year; or (ii) the number of shares determined by the Company's Board of Directors. Unless otherwise determined by the administrator of the 2015 ESPP, two offering periods of six months' duration will begin each year on January 1 and July 1. As of September 30, 2015 there was no activity under the 2015 ESPP.

### **11. Retirement Plan**

As of December 31, 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 three months and the nine months ended September 30, 2015, the Company contributed \$56 and \$120, respectively, in matching contributions to the 401(k) Plan.

## 12. Related Party Transactions

### *The Trustees of the University of Pennsylvania*

On February 20, 2009, the Company entered into a license agreement, as amended, with 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 the 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 three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, are as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Sublicense fees	\$ —	\$ 107	\$ 371	\$ 264
Royalties on sales of reagents	5	2	16	8
Maintenance of licensed patents	33	29	191	118
Manufacturing of reagents for sale	5	40	87	80
Research and development services	142	2,493	624	5,136
	<u>\$ 185</u>	<u>\$ 2,671</u>	<u>\$ 1,289</u>	<u>\$ 5,606</u>

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, 2014 and September 30, 2015, the Company had accrued \$1,732 and \$163, respectively, in expenses payable to Penn which are included in other related party payables on the balance sheets.

As a result of various factors, including the dilution to the Company's stockholders resulting from the sale and issuance of Series C Preferred Stock and Series D Preferred Stock, and the closing of the Company's IPO on September 22, 2015, the Company no longer considers Penn to be a related party subsequent to the period ended September 30, 2015.

### *GlaxoSmithKline LLC*

On March 6, 2009, the Company entered into a license agreement, as amended, with 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 to pay GSK up to \$1,650 upon the achievement of various milestones. As of September 30, 2015, 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 three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, are as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Sublicense fees	\$ —	\$ 107	\$ 371	\$ 264
Royalties on sales of reagents	3	1	10	5
Maintenance of licensed patents	108	57	324	474
	<u>\$ 111</u>	<u>\$ 165</u>	<u>\$ 705</u>	<u>\$ 743</u>

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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, 2014 and September 30, 2015, the Company had accrued \$2,028 and \$186, respectively, payable to GSK under the license agreement.

As a result of various factors, including the dilution to the Company's stockholders resulting from the sale and issuance of Series C Preferred Stock and Series D Preferred Stock, and the closing of the Company's IPO on September 22, 2015, the Company no longer considers GSK to be a related party subsequent to the period ended September 30, 2015.

### ***Dimension Therapeutics, Inc.***

On October 30, 2013, the Company granted an exclusive, sublicensable, worldwide commercial license to Dimension Therapeutics, Inc. (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. 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 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 date of the license. Management has evaluated consolidation guidance under FASB ASC Topic 810, *Consolidation* and determined that Dimension is considered a variable interest entity (VIE), 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, 2014 and September 30, 2015 was \$3 and the receivables due from Dimension as of December 31, 2014 and September 30, 2015 were \$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 three months and nine months ended September 30, 2014, the Company recorded research and development expense to Penn, and related reimbursements from Dimension of \$1,455 and \$3,115, respectively, for a net cost of \$0 for all periods. As of December 31, 2014, the Company had recorded a payable of \$750 to Penn and related party receivables of \$750 from Dimension under the arrangement. The arrangement expired on December 31, 2014 and as of September 30, 2015 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

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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 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 three months ended September 30, 2014 and 2015, the Company recognized \$150 and \$1,000, respectively, in license revenue from license agreements with Dimension. During the nine months ended September 30, 2014 and 2015 the Company recognized \$150 and \$2,000, 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 three months ended September 30, 2014 and 2015, the Company recognized gains of \$8 and \$5, respectively, related to the delivery of purchased material. For the nine months ended September 30, 2014 and 2015, the Company recognized gains of \$32 and \$26, respectively, related to the delivery of purchased material. Gains resulting from the delivery of the purchased material to Dimension is included in other operating income in the statements of operations. As of December 31, 2014 and September 30, 2015, the Company recorded an advance payment liability of \$153 and \$127, respectively, for proceeds received for undelivered material.

As of September 30, 2015, the Company does not have any overlapping board members with Dimension. Additionally, the Company's directors and management do not have significant influence over Dimension. Accordingly, the Company no longer considers Dimension to be a related party subsequent to the period ended September 30, 2015.

### *FoxKiser*

The Company was party to a services agreement, as amended from time to time, with FoxKiser, an affiliate of certain stockholders 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. The amounts outstanding to FoxKiser 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 allocates the service fees from FoxKiser between research and development and general and administrative expense. For the three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, costs incurred under the services agreement with FoxKiser were as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Research and development	\$ 300	\$ —	\$ 855	\$ 168
General and administrative	399	—	1,134	237
	<u>\$ 699</u>	<u>\$ —</u>	<u>\$ 1,989</u>	<u>\$ 405</u>

As of December 31, 2014, the Company had recorded \$1,423 payable to FoxKiser under the services agreement. As discussed in Note 6 and Note 8, amounts owed under the services agreement were converted into 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 6.

## **Item 2. 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 in conjunction with the financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q and with our audited financial statements included in our prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on September 17, 2015 (the Prospectus). Some of the information contained in this discussion and analysis or set forth elsewhere in this Quarterly Report on Form 10-Q, including information with respect to our plans and strategy for our business and related financing, includes forward-looking statements that involve risks, uncertainties and assumptions. You should read the "Risk Factors" and "Information Regarding Forward-Looking Statements" sections of this Quarterly Report on Form 10-Q for a discussion of important factors that could cause actual results to 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 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. 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 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 third-party licensees (NAV Technology Licensees). Our most advanced internally developed product candidates include programs for the treatment of two severe, rare genetic diseases: homozygous familial hypercholesterolemia (HoFH) and Mucopolysaccharidosis Type I (MPS I).

RGX-501 is our product candidate for the treatment of HoFH, which uses the AAV8 vector to deliver the human low-density lipoprotein receptor gene to liver cells. In October 2015, our development partners at the University of Pennsylvania filed an Investigational New Drug application (IND) with the United States Food and Drug Administration (FDA) to support the planned 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. RGX-111 is our product candidate for the treatment of MPS I, which uses the AAV9 vector to deliver the human  $\alpha$ -L-iduronidase gene to the central nervous system. We intend to file an IND in the first half of 2016 to support the planned initiation of a dose-escalation Phase I/II clinical trial of RGX-111 based gene delivery via CNS administration in subjects with MPS I. We received orphan drug product designation from the FDA for RGX-501 and RGX-111 in January 2012 and September 2015, respectively. In September 2015, we received endorsement from the United States National Institutes of Health Office of Biotechnology Activities' Recombinant DNA Advisory Committee, commonly referred to as the RAC, for our RGX-111 protocol.

We also have a program for wet age-related macular degeneration (wet AMD), which we refer to as RGX-314, that is in the preclinical stage and for which we plan to file an 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.

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. 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.

On September 22, 2015, we closed our initial public offering (IPO) whereby we sold 7,245 shares of common stock (inclusive of 945 shares of common stock sold pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) at a price of \$22.00 per share. The shares began trading on the Nasdaq Global Select Market on September 17, 2015. The aggregate net proceeds from the offering were \$145,183, net of underwriting discounts and commissions and offering expenses payable by us. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,298 shares of common stock.

### **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.

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### ***License Revenue***

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 by the licensee 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 highly dependent on the successful development and commercialization of products by our licensees, which is uncertain and may fluctuate significantly from period to period.

License revenue from a related party consists of license fees from licenses granted to Dimension Therapeutics, Inc. (Dimension). Since October 2013, we have granted Dimension a number of commercial licenses to our proprietary technology. In March 2015, we entered into a new option and license agreement with Dimension that grants Dimension options to exclusive commercial licenses for four new disease indications to be elected by Dimension. When elected, each option carries an option fee of \$1,000 payable to us upon exercise, and annual maintenance fees of \$50. Additionally, for each option exercised, Dimension is obligated to pay us up to \$9,000 upon their 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. In May and August 2015, Dimension exercised its first and second options, respectively, under the agreement for total aggregate license option fees of \$2,000. In accordance with our revenue recognition policy, the license fees were recognized in full upon the delivery of the license, as we have no further performance obligations under the agreements.

### ***Grant Revenue***

Grant revenue is generated through research and development grant programs offered by the United States federal government and the European Union. As of September 30, 2015, all grants from the United States federal government have been completed. Grant revenue is expected to decrease in 2015 and in future periods as we expect to incur significantly less costs under the MeuSIX grant funded by the European Union. 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.

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### ***Research and Development Expense***

Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits, travel 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.

Prior to 2015, 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 in January 2015.

We plan to increase our research and development expenses for the foreseeable future as we continue development of our product candidates. Our current and planned research and development activities include the following:

- initiation of Phase I/II clinical trials to evaluate the safety and efficacy of our RGX-501 and RGX-111 product candidates for HoFH and MPS I, respectively;
- clinical trials to evaluate the safety and efficacy of our RGX-314 product candidate for wet AMD;
- research and development for our RGX-121 and RGX-321 programs for Mucopolysaccharidosis Type II (MPS II) and X-linked retinitis pigmentosa (XLRP), respectively;
- research and development for additional product candidates addressing other diseases in the metabolic, neurodegenerative and retinal therapeutic areas;
- continued investment in manufacturing process development activities; and
- continued acquisition and manufacture of clinical trial materials in support of our anticipated clinical trials.

During the three months ended September 30, 2014 and 2015, and the nine months ended September 30, 2014 and 2015, we incurred the following external research and development expenses:

- \$528, \$1,343, \$1,516 and \$4,250, respectively, for external, preclinical research and development as well as grant activities related to our internal product candidates, and the advancement of our technology and other potential product candidates;
- \$152, \$1,157, \$152 and \$1,565, 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 and RGX-314; and
- \$29, \$647, \$202 and \$2,059, 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 three months ended September 30, 2014 and 2015, and the nine months ended September 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.

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***General and Administrative Expense***

General and administrative expense consists primarily of salaries and personnel-related costs, including employee travel, benefits and any stock-based compensation, for employees performing functions other than research and development. This includes personnel in executive, finance, legal 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, insurance costs, costs of our information systems and other general corporate activities.

Prior to 2015, 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 in January 2015.

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. In January 2015, FoxKiser exercised its share settlement options and converted the aggregate principal and interest due under both its promissory notes of \$2,403, as well as \$1,389 outstanding under the services agreement, into 585 shares of Series C convertible preferred stock (Series C Preferred Stock). The services agreement was terminated in January 2015. 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 September 30, 2015.

**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.

Our significant accounting policies are more fully described in Note 2 of the accompanying unaudited financial statements and in Note 2 to our audited financial statements which are included in our Prospectus.

During the nine months ended September 30, 2015, we began to invest in marketable securities, and as a result, have adopted a new marketable securities accounting policy as discussed in Note 2 of the accompanying unaudited financial statements. There have been no other changes in our critical accounting policies since December 31, 2014.



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**Results of Operations**

(in thousands)	Three Months Ended September 30,			Nine Months Ended September 30,		
	2014	2015	Change	2014	2015	Change
<b>Revenues</b>						
License revenue	\$ —	\$ 65	\$ 65	\$ 3,705	\$ 635	\$ (3,070)
License revenue from related party	150	1,000	850	150	2,000	1,850
Reagent sales	23	61	38	317	200	(117)
Grant revenue	310	14	(296)	800	305	(495)
Total revenues	483	1,140	657	4,972	3,140	(1,832)
<b>Expenses</b>						
Costs of revenues						
Licensing costs to related parties	—	213	213	741	527	(214)
Costs of reagent sales (including amounts to related parties)	12	44	32	115	94	(21)
Research and development (including amounts to related parties)	1,085	5,664	4,579	2,869	12,471	9,602
General and administrative (including amounts to related parties)	1,070	2,567	1,497	2,732	7,671	4,939
Foreign currency transaction losses (gains)	25	4	(21)	12	41	29
Other operating income	(8)	(5)	3	(32)	(26)	6
Total operating expenses	2,184	8,487	6,303	6,437	20,778	14,341
Loss from operations	(1,701)	(7,347)	(5,646)	(1,465)	(17,638)	(16,173)
<b>Other Income (Expense)</b>						
Investment income	—	15	15	—	23	23
Interest expense	(92)	—	92	(202)	(20)	182
Total other income (expense)	(92)	15	107	(202)	3	205
Net loss	<u>\$ (1,793)</u>	<u>\$ (7,332)</u>	<u>\$ (5,539)</u>	<u>\$ (1,667)</u>	<u>\$ (17,635)</u>	<u>\$ (15,968)</u>

**Comparison of the Three Months Ended September 30, 2014 and 2015**

*License Revenue and License Revenue from Related Party.* License revenue and license revenue from a related party increased by \$915, from \$150 for the three months ended September 30, 2014 to \$1,065 for the three months ended September 30, 2015. This increase is primarily attributable to \$1,000 of license revenue recognized upon the exercise of a commercial license option by Dimension in August 2015. We did not recognize any revenue from up-front fees or option fees on commercial licenses during the three months ended September 30, 2014.

*Grant Revenue.* Grant revenue decreased by \$296, from \$310 for the three months ended September 30, 2014 to \$14 for the three months ended September 30, 2015. This decrease is primarily due to significantly less research and development activities conducted under our grant programs. As of January 2015, all grants awards from agencies of the United States federal government were completed. Additionally, we incurred significantly less reimbursable costs under our grant award from the European Union. We expect grant revenue to decrease in future periods as we are not currently seeking any further grant awards.

*Licensing Costs to Related Parties.* Licensing costs to related parties increased by \$213, from \$0 for the three months ended September 30, 2014 to \$213 for the three months ended September 30, 2015. This increase is primarily attributable to an increase in sublicense fees payable to The Trustees of the University of Pennsylvania (Penn) and GlaxoSmithKline LLC (GSK) corresponding to \$1,000 in license revenue recognized from a commercial option exercised by Dimension in August 2015. We did not recognize any revenue from up-front fees or option fees on commercial licenses during the three months ended September 30, 2014.

*Research and Development Expense.* Research and development expenses increased by \$4,579, from \$1,085 for the three months ended September 30, 2014 to \$5,664 for the three months ended September 30, 2015. This increase is primarily attributable to the following:

- an increase of \$2,643 for externally sourced research and development, process development and manufacturing of material for clinical trials related primarily to RGX-111, RGX-121 and RGX-314; and
- an increase of \$1,839 for additional personnel costs as a result of increased headcount and stock-based compensation expense, as well as recruiting costs associated with the hiring of key research and development personnel.

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The increase was partially offset by a decrease of \$217 for research and development costs incurred under our grant programs.

*General and Administrative Expense.* General and administrative expenses increased by \$1,497, from \$1,070 for the three months ended September 30, 2014 to \$2,567 for the three months ended September 30, 2015. This increase is primarily attributable to the following:

- an increase of \$719 for additional personnel costs as a result of increased headcount and stock-based compensation expense, as well as recruiting costs associated with the hiring of general corporate personnel;
- an increase of \$477 for professional fees related to legal, accounting and consulting services; and
- an increase of \$203 for additional facilities, information technology and travel expenses supporting general corporate activities.

The increase was partially offset by a decrease of \$53 for costs associated with the maintenance of licensed patents.

*Interest Expense.* Interest expense decreased by \$92, from \$92 for the three months ended September 30, 2014 to \$0 for the three months ended September 30, 2015. This decrease is attributable to the conversion of debt instruments outstanding with FoxKiser into Series C Preferred Stock in January 2015, and the termination of the services agreement with FoxKiser in January 2015.

### **Comparison of the Nine Months Ended September 30, 2014 and 2015**

*License Revenue and License Revenue from Related Party.* License revenue and license revenue from a related party decreased by \$1,220, from \$3,855 for the nine months ended September 30, 2014 to \$2,635 for the nine months ended September 30, 2015. This decrease is primarily attributable to a \$1,600 decrease in up-front fees and option fees recognized upon commercial licenses granted by us. During the nine months ended September 30, 2014 we granted four commercial licenses for total up-front and option fees of \$3,600. During the nine months ended September 30, 2015, we granted two commercial licenses for total up-front and option fees of \$2,000. The decrease in license revenue is partially offset by a \$250 increase in milestone fees, and a \$180 increase in maintenance fees, recognized from commercial licenses we granted prior to 2015.

*Reagent Sales.* Reagent sales decreased by \$117, from \$317 for the nine months ended September 30, 2014 to \$200 for the nine months ended September 30, 2015. This decrease is due to a general decline in sales volume during the nine months ended September 30, 2015. The number of customer orders for reagents is low and may fluctuate significantly from period to period based on the research objectives of our current and prospective customers. 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 \$495, from \$800 for the nine months ended September 30, 2014 to \$305 for the nine months ended September 30, 2015. This decrease is primarily due to significantly less research and development activities conducted under our grant programs. As of January 2015, all grants awards from agencies of the United States federal government were completed. Additionally, we incurred significantly less reimbursable costs under our grant award from the European Union. We expect grant revenue to decrease in future periods as we are not currently seeking any further grant awards.

*Licensing Costs to Related Parties.* Licensing costs to related parties decreased by \$214, from \$741 for the nine months ended September 30, 2014 to \$527 for the nine months ended September 30, 2015. This decrease is primarily attributable to a decrease in sublicense fees payable to Penn and GSK, as a result of a \$1,600 decrease in up-front fees and option fees recognized upon commercial licenses granted by us.

*Research and Development Expense.* Research and development expenses increased by \$9,602, from \$2,869 for the nine months ended September 30, 2014 to \$12,471 for the nine months ended September 30, 2015. This increase is primarily attributable to the following:

- an increase of \$6,146 for externally sourced research and development, process development and manufacturing of material for clinical trials related primarily to RGX-111, RGX-121 and RGX-314; and
- an increase of \$2,805 for additional personnel costs as a result of increased headcount and stock-based compensation expense, as well as recruiting costs associated with the hiring of key research and development personnel.

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*General and Administrative Expense.* General and administrative expenses increased by \$4,939, from \$2,732 for the nine months ended September 30, 2014 to \$7,671 for the nine months ended September 30, 2015. This increase is primarily attributable to the following:

- an increase of \$1,878 for additional personnel costs as a result of increased headcount and stock-based compensation expense, as well as recruiting costs associated with the hiring of general corporate personnel;
- an increase of \$2,252 for professional fees related to legal, accounting and consulting services; and
- an increase of \$355 for information technology and travel expenses supporting general corporate activities.

*Interest Expense.* Interest expense decreased by \$182, from \$202 for the nine months ended September 30, 2014 to \$20 for the nine months ended September 30, 2015. This decrease is attributable to the conversion of debt instruments outstanding with FoxKiser into Series C Preferred Stock in January 2015, and the termination of the services agreement with FoxKiser in January 2015.

## **Liquidity and Capital Resources**

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

In January 2015, we completed the sale and issuance 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 \$26,208 of cash proceeds and the conversion \$3,792 of debt by FoxKiser. In May 2015, we completed the sale and issuance of 7,367 shares of Series D convertible preferred stock (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.

In September 2015, we completed our IPO whereby we sold 7,245 shares of common stock (inclusive of 945 shares of common stock sold pursuant to the full exercise of an option to purchase additional shares granted to the underwriters in connection with the offering) at a price of \$22.00 per share. The aggregate net proceeds from the offering were \$145,183, net of underwriting discounts and commissions and offering expenses payable by us. Upon the closing of the IPO, all outstanding shares of convertible preferred stock converted into 16,298 shares of common stock. As of September 30, 2015, we had cash, cash equivalents and marketable securities of \$224,362. We believe that our cash, cash equivalents and marketable securities as of September 30, 2015 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.

We have incurred losses since our inception and, as of September 30, 2015, had an accumulated deficit of \$46,444. 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 for the foreseeable future. As a result, we may 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**

(in thousands)	Nine Months Ended	
	September 30,	
	2014	2015
Net cash used in operating activities	\$(2,537)	\$(18,072)
Net cash used in investing activities	—	(19,459)
Net cash provided by financing activities	2,400	241,734
	<u>\$ (137)</u>	<u>\$204,203</u>

## **Operating Activities**

The significant increase in cash used in operating activities for the nine months ended September 30, 2015, compared to the nine months ended September 30, 2014, is primarily attributable to increases in research and development and general and administrative expenses as a result of increased headcount, spending on the advancement of our lead product candidates, professional fees and corporate overhead. We expect operating expenses to continue to increase for the foreseeable future.

For the nine months ended September 30, 2015, our net cash used in operating activities of \$18,072 consisted of a net loss of \$17,635 and changes in working capital of \$2,549, offset by \$2,112 in adjustments for non-cash items. The change in working capital is primarily attributable to an increase in prepaid expenses of \$1,372 and a decrease in related party payables of \$3,446, and was partially offset by a decrease in accounts receivable of \$1,277 and an increase in accounts payable and accrued expenses of \$1,075. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of \$2,059.

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For the nine months ended September 30, 2014, our net cash used in operating activities of \$2,537 consisted of a net loss of \$1,667 and changes in working capital of \$1,111, offset by \$241 in adjustments for non-cash items. The change in working capital was primarily attributable to a decrease in related party payables of \$1,590 and an increase in accounts receivable of \$293, and was partially offset by increases in accounts payable and accrued expenses of \$735. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of \$220.

### ***Investing Activities***

For the nine months ended September 30, 2015, net cash used in investing activities consisted of \$19,065 to purchase marketable securities and \$394 to purchase property and equipment.

We had no cash flows from investing activities for the nine months ended September 30, 2014.

### ***Financing Activities***

For the nine months ended September 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, \$67,998 in net proceeds from the sale and issuance of Series D Preferred Stock and \$159,390 in gross proceeds from the sale and issuance of common stock in our IPO, which is partially offset by \$11,775 of issuance costs paid by us as of September 30, 2015 as a result of the offering.

For the nine months ended September 30, 2014, net cash provided by financing activities consisted of \$2,400 in proceeds from promissory notes issued to FoxKiser.

### ***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. As a result of our IPO, 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 our cash and cash equivalents and marketable securities as of September 30, 2015, 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. We intend to devote the majority of our current capital to 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 internal 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;

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- 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 to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

### **Contractual Obligations, Commitments and Contingencies**

As of September 30, 2015, there have been no material changes to our contractual obligations as of December 31, 2014 from those described in the Prospectus, except as noted below:

In March 2015, we entered into a lease agreement for our corporate headquarters in Rockville, Maryland. In September 2015, we amended the lease to include additional office and laboratory space at the same facility. Additionally, we have entered into a short-term lease agreement for laboratory space in Philadelphia, Pennsylvania. Future minimum lease payments are as follows:

(in thousands)	Total	Less Than 1 Year	Years 1-3	Years 4-5	More Than 5 Years
Future minimum lease payments	\$4,273	\$ 73	\$2,396	\$1,708	\$ 96

### **Off-Balance Sheet Arrangements**

We did not have any off-balance sheet arrangements during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

### **Item 3. Quantitative and Qualitative Disclosures about Market Risk.**

For additional information regarding market risk, refer to the *Quantitative and Qualitative Disclosures About Market Risk* section of the Prospectus. There have been no material changes to our exposure to market risk during the nine months ended September 30, 2015, except as noted below:

#### ***Interest Rate Sensitivity***

Our primary exposure to interest income sensitivity results from the cash equivalents and marketable securities in our investment portfolio. Our primary objectives in managing our investment portfolio are to preserve principal, maintain proper liquidity to meet operating needs and maximize yields. The securities we hold in our investment portfolio are subject to interest rate risk. At

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any time, significant changes in interest rates can affect the fair value of the investment portfolio and its interest earnings. After a review of the investment portfolio, we do not believe a sudden change in the interest rates would have a material impact on our financial condition or results of operations. Currently, we do not hedge these interest rate exposures. We have established policies and procedures to manage exposure to fluctuations in interest rates. We place our investments with high quality issuers and limit the amount of credit exposure to any one issuer and do not use derivative financial instruments in our investment portfolio. We invest in highly liquid, investment-grade securities and money market funds of various issues, types and maturities. Our investments in marketable securities are classified as available-for-sale and are carried at fair value. Unrealized gains and losses, net of any related tax effects, are excluded from earnings and are included in other comprehensive loss and reported as a separate component of stockholders' equity until realized. A decline in the fair value below cost of available-for-sale securities that is deemed other-than-temporary is charged to results of operations, resulting in the establishment of a new cost basis for the security.

### **Item 4. Controls and Procedures.**

#### ***Evaluation of Disclosure Controls and Procedures***

We have carried out an evaluation, under the supervision of our Chief Executive Officer and our Chief Financial Officer, in which we have evaluated the effectiveness of our disclosure controls and procedures as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, as of the end of the period covered by this report. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer concluded that our disclosure controls and procedures were not effective as of September 30, 2015 to ensure that information required to be disclosed by us in the reports that we file or submit under the Exchange Act is recorded, processed, summarized and reported, within the time periods specified in the SEC's rules and forms, and that such information is accumulated and communicated to our management, including our principal executive and principal financial officers, as appropriate, to allow timely decisions regarding required disclosure.

Our disclosure controls and procedures were not effective as of September 30, 2015 because of material weaknesses in internal control over financial reporting. We did not maintain (i) effective controls in our contract review process to ensure the completeness of contracts reviewed and to appropriately identify and account for provisions within our contracts; and (ii) a sufficient complement of resources to ensure adequate review and segregation of duties within our financial reporting processes.

For further information regarding material weaknesses in internal control over financial reporting, please refer to Item 1A of this Quarterly Report on Form 10-Q in "Risk Factors—Risks Related to Our Financial Position."

#### ***Plan of Remediation of Material Weakness***

In order to remediate these material weaknesses, we have hired additional qualified accounting and finance staff members to augment our current staff, and we are formalizing the documentation of our accounting policies and internal controls, and strengthening supervisory reviews by our management. Additionally, we will be utilizing various information systems to help facilitate contract management, and the documentation and review of contracts by appropriate accounting personnel. While we have implemented a plan to remediate this weakness, our remediation efforts are still ongoing and we cannot assure you of when we will be able to remediate this material weakness.

#### ***Changes in Internal Control Over Financial Reporting***

Other than the remedial efforts described above, there were no changes in our internal control over financial reporting (as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act) that occurred during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## **PART II – OTHER INFORMATION**

### **Item 1. Legal Proceedings.**

From time to time, we are subject to claims in legal proceedings arising in the normal course of its business. We do not believe that we are currently party to any pending legal actions that could reasonably be expected to have a material adverse effect on our business, financial condition, results of operations or cash flows.

### **Item 1A. Risk Factors.**

*You should carefully consider the risk factors set forth below as well as the other information contained in this Quarterly Report and in our other public filings in evaluating our business, including our prospectus dated September 16, 2015, filed pursuant to Rule 424(b)(4) under the Securities Act of 1933, as amended, with the Securities and Exchange Commission on September 17, 2015 (the Prospectus). Any of the following risks could materially and adversely affect our business, financial condition or results of operations. The risks described below are not the only risks facing us. Additional risks and uncertainties not currently known to us or that we currently view to be immaterial may also materially adversely affect our business, financial condition or results of operations. In these circumstances, the market price of our common stock would likely decline and you could lose all or part 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

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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 Mucopolysaccharidosis Type I (MPS I), Mucopolysaccharidosis Type II (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.



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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.

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***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 studies 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 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;

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- 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;

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- 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 planned 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.

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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 Food, Drug and Cosmetic 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.

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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,

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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.

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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 nine months ended September 30, 2015 were \$5.4 million, \$4.0 million and \$17.6 million, respectively. As of September 30, 2015, we had an accumulated deficit of \$46.4 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



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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 studies 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.

***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 September 30, 2015, our cash, cash equivalents and marketable securities were \$224.4 million. We expect that our existing cash, cash equivalents and marketable securities 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.

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;

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- 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;

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- 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 studies 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.

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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 are 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 our initial public offering (completed in September 2015), 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 Securities and Exchange Commission (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 our initial public 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

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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 Penn 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;

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- 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

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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 biologics 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

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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 processes 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.



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***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.

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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

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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.

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***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 studies 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.

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### ***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.

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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;

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- 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.



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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.

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***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 infrastructure 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.

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

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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. 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.

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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.

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### ***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. 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

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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

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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.

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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.



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***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**

***The trading price of the shares of our common stock has been and will likely continue to be highly volatile, and purchasers of our common stock may not be able to resell the shares of our common stock at or above the purchase price and could incur substantial losses.***

Our stock price has been and is likely to continue to be volatile. 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;

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- 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;
- 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; or
- 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.

***As a newly public company, our stock price has been and may continue to 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.

***Our quarterly operating results may fluctuate significantly.***

We expect our operating results to be subject to quarterly fluctuations. Our net loss and other operating results will be affected by numerous factors, including:

- variations in the level of expenses related to our NAV Technology Platform and Lead Product Candidates;
- addition or termination of clinical trials;
- regulatory developments affecting our Lead Product Candidates or future product candidates;
- our execution of any collaborative, licensing or similar arrangements, including our NAV Technology Licensees, and the timing of payments we may make or receive under these arrangements; and
- nature and terms of stock-based compensation grants and any intellectual property infringement lawsuit in which we may become involved.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially.

***If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.***

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. If few analysts commence coverage of us, the trading price of our stock would likely decrease. Although we have obtained analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

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### ***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.

### ***We may use our cash and cash equivalents in ways that you and other stockholders may not approve.***

Our management has broad discretion in the application of our cash and cash equivalents. Because of the number and variability of factors that will determine our use of our cash and cash equivalents, their ultimate use may vary substantially from their currently intended use. Our management might not apply our cash and cash equivalents in ways that ultimately increase the value of your investment. The failure by our management to apply our cash and cash equivalents effectively could harm our business. Pending their use, we may invest our cash and cash equivalents in a variety of capital preservation investments, including short-term, interest-bearing, investment-grade instruments and U.S. government securities. These investments may not yield a favorable return to our stockholders.

### ***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 September 30, 2015, our executive officers, directors, holders of more than five percent of our capital stock and their respective affiliates beneficially owned 33.5% of our outstanding capital stock. Therefore, these stockholders may have the ability to influence us through their ownership position. 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. We have 26,313,471 outstanding shares of common stock as of November 5, 2015. Of these shares, only the shares of common stock sold in our initial 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 of our initial public offering 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 Prospectus. However, we and the lead underwriters in our initial public offering may permit these holders to sell shares prior to the expiration of the lock-up agreements with the underwriters.

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Based on 26,313,471 shares outstanding as of November 5, 2015, after the contractual lock-up agreements pertaining to our initial public offering expire 180 days from the date of the 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.

In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock 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.

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 will continue to 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 continue to 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 of the Sarbanes-Oxley Act of 2002 (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”;

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- 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.

***Our restated certificate of incorporation designates 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, 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.

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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 our initial public offering.

**Item 2. Unregistered Sales of Equity Securities and Use of Proceeds.**

*Sales of Unregistered Securities*

In July 2015, an employee exercised an option to purchase 10,150 shares of our common stock. The issuance of the securities described in the preceding sentence was deemed to be exempt from registration under the Securities Act in reliance on Rule 701.

*Use of Proceeds*

On September 22, 2015, we closed our initial public offering (IPO) whereby 7,245,000 shares of common stock were sold at a public offering price of \$22.00 per share for an aggregate offering price of \$159.4 million, which included 945,000 shares of common stock sold at the IPO price of \$22.00 pursuant to the underwriters' option to purchase additional shares of common stock (Underwriters' Option). The offer and sale of all of the shares in the IPO and pursuant to the Underwriters' Option were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333-206430), which was declared effective by the SEC on September 16, 2015. The offering commenced as of September 16, 2015 and did not terminate before all of the securities registered in the registration statement were sold. The syndicate of underwriters was led by Morgan Stanley & Co. LLC, Merrill Lynch, Pierce, Fenner & Smith Incorporated and Piper Jaffray & Co. as joint book-running managers, and Chardan Capital Markets, LLC, as co-manager. We raised approximately \$145.2 million in net proceeds after deducting underwriting discounts and commissions and other offering expenses. No payments were made by us to directors, officers or persons owning ten percent or more of our common stock or to their associates, or to our affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board committee service.

There has been no material change in the planned use of proceeds from our IPO as described in our prospectus dated September 16, 2015, filed with the SEC pursuant to Rule 424(b)(4) under the Securities Act.

**Item 3. Defaults Upon Senior Securities.**

None.

**Item 4. Mine Safety Disclosures.**

Not Applicable.

**Item 5. Other Information.**

None.

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**Item 6. Exhibits.**

<u>Exhibit</u>	<u>Description</u>
31.1	Certification of the Principal Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of the Principal Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Extension Label Linkbase Document
101.PRE	XBRL Taxonomy Extension Presentation Linkbase Document

The certification attached as Exhibit 32.1 that accompanies this Quarterly Report on Form 10-Q is not deemed filed with the SEC and is not to be incorporated by reference into any filing of REGENXBIO Inc. under the Securities Act or the Exchange Act, whether made before or after the date of this Quarterly Report on Form 10-Q, irrespective of any general incorporation language contained in such filing.

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**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, as amended, the registrant has duly caused this report to be signed on its behalf by the undersigned thereunto duly authorized.

REGENXBIO Inc.

Dated: November 5, 2015

/s/ Kenneth T. Mills

Kenneth T. Mills  
President and Chief Executive Officer  
(Principal Executive Officer)

Dated: November 5, 2015

/s/ Vittal Vasista

Vittal Vasista  
Chief Financial Officer (Principal Financial Officer  
and Principal Accounting Officer)



**EXHIBIT INDEX**

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## CERTIFICATION

I, Kenneth T. Mills, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. [Paragraph omitted in accordance with SEC Release Nos. 33-8238; 34-47986; IC-26068; June 5, 2003];
  - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 5, 2015

/s/ Kenneth T. Mills

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**Kenneth T. Mills**  
**President and Chief Executive Officer**  
**(Principal Executive Officer)**

## CERTIFICATION

I, Vittal Vasista, certify that:

1. I have reviewed this Quarterly Report on Form 10-Q of REGENXBIO Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a. designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. [Paragraph omitted in accordance with SEC Release Nos. 33-8238; 34-47986; IC-26068; June 5, 2003];
  - c. evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. all significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: November 5, 2015

/s/ Vittal Vasista

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**Vittal Vasista**  
**Chief Financial Officer**  
**(Principal Financial and Accounting Officer)**

**Certification****Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002****(Subsections (a) and (b) of Section 1350, Chapter 63 of Title 18, United States Code)**

Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (subsections (a) and (b) of section 1350, chapter 63 of title 18, United States Code), each of the undersigned officers of REGENXBIO Inc. (the Company), does hereby certify, to the best of such officer's knowledge, that:

The Quarterly Report on Form 10-Q for the quarter ended September 30, 2015 (the Form 10-Q) of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and the information contained in the Form 10-Q fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: November 5, 2015

/s/ Kenneth T. Mills

**Kenneth T. Mills**  
**President and Chief Executive Officer**  
**(Principal Executive Officer)**

Date: November 5, 2015

/s/ Vittal Vasista

**Vittal Vasista**  
**Chief Financial Officer**  
**(Principal Financial and Accounting Officer)**

A signed original of this written statement required by Section 906 has been provided to the Company and will be retained by the Company and furnished to the Securities and Exchange Commission or its staff upon request. This certification "accompanies" the Form 10-Q to which it relates, is not deemed filed with the SEC and is not to be incorporated by reference into any filing of the Company under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended (whether made before or after the date of the Form 10-Q), irrespective of any general incorporation language contained in such filing.

