REGENXBIO Inc.
Form 10-Q
May 08, 2018
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-Q

(Mark One)

QUARTERLY REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the quarterly period ended March 31, 2018

OR

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the transition period from ___ to ___

Commission File Number 001-37553

REGENXBIO Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	47-1851754
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
9600 Blackwell Road, Suite 210	20850

Rockville, MD
(Address of principal executive offices) (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)

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, a smaller reporting company, or an emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth 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
		Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

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 May 4, 2018, there were 31,936,013 outstanding shares of the registrant's common stock, par value $0.0001 per share.

Table of Contents

REGENXBIO INC.

QUARTERLY REPORT ON FORM 10-Q

FOR THE QUARTERLY PERIOD ENDED MARCH 31, 2018

TABLE OF CONTENTS

	PART I—FINANCIAL INFORMATION	
Item 1.	Financial Statements (Unaudited)	3
	Consolidated Balance Sheets as of March 31, 2018 and December 31, 2017	3
	Consolidated Statements of Operations and Comprehensive Income (Loss) for the Three Months Ended March 31, 2018 and 2017	4
	Consolidated Statements of Cash Flows for the Three Months Ended March 31, 2018 and 2017	5
	Notes to the Consolidated Financial Statements	6
Item 2.	Management's Discussion and Analysis of Financial Condition and Results of Operations	24
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	33
Item 4.	Controls and Procedures	33
	PART II—OTHER INFORMATION	
Item 1.	Legal Proceedings	34
Item 1A.	Risk Factors	34
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	77
Item 3.	Defaults Upon Senior Securities	77
Item 4.	Mine Safety Disclosures	77
Item 5.	Other Information	77
Item 6.	Exhibits	78
Signatures		79

Table of Contents

INFORMATION REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains "forward-looking statements" within the meaning of Section 27A of the Securities Act of 1933, as amended (the Securities Act), and Section 21E of the Securities Exchange Act of 1934, as amended (the Exchange Act). These statements express a belief, expectation or intention and are generally accompanied by words that convey projected future events or outcomes such as "believe," "may," "will," "estimate," "continue "anticipate," "design," "intend," "expect," "could," "plan," "potential," "predict," "seek," "should," "would" or by variations o by similar expressions. We have based these forward-looking statements on our current expectations and assumptions and analyses made by us in light of our experience and our perception of historical trends, current conditions and expected future developments, as well as other factors we believe are appropriate under the circumstances. However, whether actual results and developments will conform with our expectations and predictions is subject to a number of risks, uncertainties, assumptions and other important factors, including, but not limited to:

- the timing of enrollment, commencement and completion and the success of clinical trials conducted by us, our licensees and our partners;
- the timing of commencement and completion and the success of preclinical studies conducted by us and our development partners;
- the timely development and launch of new products;
- 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 and technology;
- 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 our expenses and revenue;
- our expectations regarding regulatory developments in the United States and foreign countries; and
- the use or sufficiency of our cash and cash equivalents and needs for additional financing.

You should carefully read the factors discussed in the sections titled "Risk Factors," "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Quarterly Report on Form 10-Q, our Annual Report on Form 10-K for the year ended December 31, 2017 and in our other filings with the U.S. Securities and Exchange Commission (the SEC) for additional discussion of the risks, uncertainties, assumptions and other important factors that could cause our actual results or developments to differ materially and adversely from those projected in the forward-looking statements. The actual results or developments anticipated may not be realized or, even if substantially realized, they may not have the expected consequences to or effects on us or our businesses or operations. Such statements are not guarantees of future performance and actual results or developments may differ materially and adversely from those projected in the forward-looking statements. These forward-looking statements speak only as of the date of this report. Except as required by law and the rules of the SEC, we do not undertake any obligation, and specifically decline any obligation, to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

Table of Contents

Available Information

We file annual, quarterly, and current reports, proxy statements, and other documents with the SEC under the Exchange Act. The public may read and copy any materials that we file with the SEC at the SEC's Public Reference Room at 100 F Street, NE, Washington, DC 20549. The public may obtain information on the operation of the Public Reference Room by calling the SEC at 1 800 SEC 0330. Also, the SEC maintains a website that contains reports, proxy and information statements, and other information that we file electronically with the SEC. The public can obtain any documents that we file with the SEC at www.sec.gov.

The public also may view and download copies of our SEC filings free of charge at our website, www.regenxbio.com. The information contained on, or that can be accessed through, our website will not be deemed to be incorporated by reference in, and are not considered part of, this Quarterly Report on Form 10-Q. Investors should also note that we use our website, as well as SEC filings, press releases, public conference calls and webcasts, to announce financial information and other material developments regarding our business. We use these channels, as well as any social media channels listed on our website, to communicate with investors and members of the public about our company. It is possible that the information that we post on our social media channels could be deemed material information. Therefore, we encourage investors, the media and others interested in our company to review the information that we post on our social media channels.

As used in this Quarterly Report on Form 10-Q, the terms "REGENXBIO," "we," "us," "our" or the "Company" mean REGENXBIO Inc. and its subsidiaries, on a consolidated basis, unless the context indicates otherwise.

NAV, REGENXBIO and the REGENXBIO logos are our registered trademarks. Any other trademarks appearing in this Quarterly Report on Form 10-Q are the property of their respective holders.

Table of Contents

PART I – FINANCIAL INFORMATION

Item 1. Financial Statements.

REGENXBIO INC.

CONSOLIDATED BALANCE SHEETS

(unaudited)

(in thousands, except per share data)

	March 31, 2018	December 31, 2017
Assets		
Current assets		
Cash and cash equivalents	$71,870	$46,656
Marketable securities	157,997	114,122
Accounts receivable	25,976	473
Prepaid expenses	4,667	5,334
Other current assets	2,208	1,412
Total current assets	262,718	167,997
Marketable securities	5,917	15,616
Accounts receivable	32,645	—
Property and equipment, net	14,829	13,977
Restricted cash	225	225
Other assets	883	862
Total assets	$317,217	$198,677
Liabilities and Stockholders' Equity		
Current liabilities		
Accounts payable	$5,007	$4,832
Accrued expenses and other current liabilities	9,869	9,605
Total current liabilities	14,876	14,437
Deferred rent, net of current portion	1,225	1,211
Other liabilities	1,776	—
Total liabilities	17,877	15,648
Commitments and contingencies (Note 6)		
Stockholders' equity		
Preferred stock; $0.0001 par value; 10,000 shares authorized, and no shares issued and outstanding at March 31, 2018 and December 31, 2017	—	—
Common stock; $0.0001 par value; 100,000 shares authorized at March 31, 2018 and December 31, 2017; 31,900 and 31,295 shares issued and outstanding at	3	3

March 31, 2018 and December 31, 2017, respectively		
Additional paid-in capital	378,954	371,497
Accumulated other comprehensive loss	(903)	(715)
Accumulated deficit	(78,714)	(187,756)
Total stockholders' equity	299,340	183,029
Total liabilities and stockholders' equity	$317,217	$198,677

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Table of Contents

REGENXBIO INC.

CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE INCOME (LOSS)

(unaudited)

(in thousands, except per share data)

	Three Months Ended March 31,	
	2018	2017
Revenues		
License revenue	$132,391	$455
Total revenues	132,391	455
Expenses		
Costs of revenues		
Licensing costs	2,408	91
Research and development	19,550	16,619
General and administrative	8,380	6,622
Other operating expenses	28	45
Total operating expenses	30,366	23,377
Income (loss) from operations	102,025	(22,922)
Other Income		
Interest income from licensing	1,355	—
Investment income	859	929
Total other income	2,214	929
Net income (loss)	$104,239	$(21,993)
Other Comprehensive Loss		
Unrealized loss on available-for-sale securities, net of reclassifications and income tax expense	(188)	(539)
Total other comprehensive loss	(188)	(539)
Comprehensive income (loss)	$104,051	$(22,532)
Net income (loss) applicable to common stockholders	$104,239	$(21,993)
Net income (loss) per share:		
Basic	$3.30	$(0.82)
Diluted	$3.04	$(0.82)
Weighted-average common shares outstanding:		
Basic	31,632	26,673
Diluted	34,275	26,673

The accompanying notes are an integral part of these unaudited consolidated financial statements.

4

Table of Contents

REGENXBIO INC.

CONSOLIDATED STATEMENTS OF CASH FLOWS

(unaudited)

(in thousands)

	Three Months Ended March 31,	
	2018	2017
Cash flows from operating activities		
Net income (loss)	$104,239	$(21,993)
Adjustments to reconcile net income (loss) to net cash provided by (used in) operating activities		
Stock-based compensation expense	3,291	2,591
Net amortization of premiums and accretion of discounts on marketable debt securities	378	469
Depreciation and amortization	834	554
Net realized gains on sales of marketable securities	—	(480)
Imputed interest income from licensing	(1,355)	—
Other non-cash adjustments	10	41
Changes in operating assets and liabilities		
Accounts receivable	(51,414)	803
Prepaid expenses	667	(68)
Other current assets	(446)	88
Other assets	(21)	10
Accounts payable	477	2,468
Accrued expenses and other current liabilities	490	(1,920)
Deferred rent	27	(44)
Other liabilities	957	—
Net cash provided by (used in) operating activities	58,134	(17,481)
Cash flows from investing activities		
Purchases of marketable securities	(54,267)	(5,188)
Maturities of marketable securities	19,525	10,500
Sales of marketable securities	—	780
Purchases of property and equipment	(2,344)	(2,929)
Net cash provided by (used in) investing activities	(37,086)	3,163
Cash flows from financing activities		
Proceeds from exercise of stock options	3,824	161
Proceeds from issuance of common stock under employee stock purchase plan	342	147
Proceeds from public offering of common stock, net of underwriting discounts and commissions	—	71,299
Issuance costs for public offering of common stock	—	(84)

Net cash provided by financing activities	4,166	71,523
Net increase in cash and cash equivalents and restricted cash	25,214	57,205
Cash and cash equivalents and restricted cash		
Beginning of period	46,881	25,065
End of period	$72,095	$82,270
Supplemental disclosures of non-cash investing and financing activities		
Issuance costs for public offering of common stock in accounts payable and accrued expenses	$—	$328

The accompanying notes are an integral part of these unaudited consolidated financial statements.

Table of Contents

REGENXBIO INC.

NOTES TO CONSOLIDATED FINANCIAL STATEMENTS

(unaudited)

1. Nature of Business

REGENXBIO Inc. (the Company) is a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. The Company's proprietary adeno-associated virus (AAV) gene delivery platform (NAV Technology Platform) consists of exclusive rights to over 100 novel AAV vectors, including AAV7, AAV8, AAV9 and AAVrh10. The Company's NAV® Technology Platform is being applied by the Company, as well as by third-party licensees (NAV Technology Licensees), in the development of product candidates for a variety of diseases with unmet needs. The Company was formed in 2008 in the State of Delaware and is headquartered in Rockville, Maryland.

Liquidity and Risks

As of March 31, 2018, the Company had generated an accumulated deficit of $78.7 million since inception. As the Company has incurred cumulative losses since inception, transition to recurring 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 recurring profitability, and unless and until it does, the Company will continue to need to raise additional capital. As of March 31, 2018, the Company had cash, cash equivalents and marketable securities of $235.8 million, which management believes is sufficient to fund operations for at least the next 12 months from the date these consolidated financial statements were issued.

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 clinical manufacturing to the commercial production of products.

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying consolidated 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 GAAP as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB). The interim

unaudited consolidated financial statements have been prepared on the same basis as the annual audited consolidated financial statements as of and for the year ended December 31, 2017 included in the Company's Annual Report on Form 10-K for the year ended December 31, 2017, which was filed with the SEC on March 6, 2018. Certain information and footnote disclosures required by GAAP which are normally included in the Company's annual consolidated financial statements have been omitted pursuant to SEC rules and regulations for interim reporting. In the opinion of management, the accompanying consolidated financial statements reflect all adjustments, which include all normal and recurring adjustments necessary for the fair statement of the Company's financial position as of March 31, 2018, and the results of its operations and its cash flows for the interim periods ended March 31, 2018 and 2017.

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 consolidated financial statements should be read in conjunction with the audited consolidated financial statements as of and for the year ended December 31, 2017, and the notes thereto, which are included in the Company's Annual Report on Form 10-K.

The accompanying consolidated financial statements include the accounts of the Company and its wholly owned subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

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 differ materially 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 consolidated financial statements. Management must apply significant judgment in this process. In addition, other factors may affect estimates, including: expected business and operational

Table of Contents

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 consolidated financial statements. Estimates are used in the following areas, among others: revenue, stock-based compensation expense, accrued research and development expenses and other accrued expenses, income taxes and the fair value of financial instruments.

Accounts Receivable

Accounts receivable primarily consists of consideration due to the Company resulting from its license agreements with NAV Technology Licensees. Accounts receivable includes amounts invoiced to licensees as well as rights to consideration which have not yet been invoiced to licensees and for which payment is conditional solely upon the passage of time. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to the Company and any accounts receivable from the licensee which is not contractually payable to the Company is charged off as a reduction of license revenue in the period of the termination. Accounts receivable from licensees which are not expected to be received by the Company within 12 months from the reporting date are recorded as non-current assets on the consolidated balance sheets.

Receivables are stated net of an allowance for doubtful accounts, if deemed necessary based on the Company's evaluation of collectability using specific identification of account balances, the credit profile of its customers and historical information regarding write-offs. Account balances are charged off against the allowance when the potential for recovery is considered remote. The Company did not record an allowance for doubtful accounts as of March 31, 2018 or December 31, 2017.

Non-marketable Equity Securities

The Company's non-marketable equity securities do not have readily determinable fair values and consist of equity investments in other entities in which the Company's ownership interest is below 20% and the Company does not have significant influence over the operations of the entity. Prior to January 1, 2018, non-marketable equity securities were accounted for using the cost method and measured at cost less impairment. Beginning January 1, 2018, upon the Company's adoption of ASU 2016-01, Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities, non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. Please refer to Note 4 for further information on non-marketable equity securities.

Fair Value of Financial Instruments

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC Topic 820, Fair Value Measurements and Disclosures (ASC 820), establishes a hierarchy of inputs used in measuring fair value that maximizes the use of observable inputs and minimizes the use of unobservable inputs by requiring that the observable inputs be used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability, and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the

investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1—Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2—Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3—Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair values of the Company's Level 2 instruments are based on quoted market prices or broker or dealer quotations for similar assets. These investments are initially valued at the transaction price and subsequently valued utilizing third party pricing providers or other market observable data. Please refer to Note 4 for further information on the fair value measurement of the Company's financial instruments.

Table of Contents

Revenue Recognition

Effective January 1, 2018, the Company adopted ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition (Topic 605). Please refer to Recent Accounting Pronouncements below for additional information on the adoption of Topic 606 and the impact upon adoption to the Company's financial position and results of operations.

Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

The Company applies the five-step model to contracts that are within the scope of Topic 606 only when it is probable that the Company will collect the consideration it is entitled to in exchange for the goods or services it transfers to the customer. At contract inception, for contracts within the scope of Topic 606, the Company assesses the goods or services promised within each contract and determined those that are performance obligations and whether each promised good or service is distinct. The Company then recognizes as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

The Company evaluates its contracts for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, the Company evaluates the credit profile of the customer and prevailing market interest rates and selects an interest rate in which it believes would be charged to the customer in a separate financing arrangement over a similar financing term.

License revenue

The Company licenses its NAV Technology Platform 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 using the Company's NAV Technology Platform. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration to the Company under its license agreements may include: (i) up-front fees, (ii) option fees to obtain additional licenses, (iii) annual maintenance fees, (iv) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (v) sublicense fees and (vi) royalties on sales of licensed products.

The Company has determined that all of its license agreements are contracts with customers within the scope of Topic 606. Although licenses are terminable at the option of licensee, the Company has determined that there is a substantive termination penalty associated with the termination of each license. Due to the substantive termination penalty, the contract term for purposes of applying Topic 606 is equal to the stated term of the license agreement, which is the life of the underlying licensed patents. The only identified performance obligations in the Company's license agreements are for the delivery of intellectual property licenses to licensees. The transaction price for each

license agreement is fully allocated to the delivery of the license(s) and is recognized as revenue upon the delivery of the license(s) to the licensee, which generally occurs upon the execution of the license agreement.

For license agreements which contain options for the licensee to purchase additional licenses in the future, the Company evaluates the options at the inception of the agreement to determine if they provide a material right to the licensee. In making this determination, the Company considers whether the optional licenses are priced at a discount to the standalone selling price for the licenses. The Company has determined that none of the options provided to licensees in its license agreements represent material rights to licensees, and therefore do not represent performance obligations of the Company until exercised by the licensee. Consideration contingent upon the exercise of options by licensees is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised. Upon the exercise of an option by a licensee, the additional consideration related to the option exercise is added to the transaction price and recognized as revenue upon the delivery of the newly purchased license.

Table of Contents

The Company evaluates the transaction price for its license agreements at each reporting date and recognizes changes to the transaction price as revenue during the period, provided that the associated license has been delivered to the licensee. The transaction price for each license includes all fixed consideration, as well as variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under the Company's license agreements includes up-front fees and annual maintenance fees. Variable consideration under the Company's license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products.

Up-front license fees are included in the transaction price and recognized as revenue upon the delivery of the license. If up-front license fees are payable to Company in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist and the Company adjusts the transaction price to include only the present value of the license fees. The discounted portion of the license fees is recognized as interest income in the consolidated statements of operations over the term of the financing period.

Annual maintenance fees are generally payable to the Company on each anniversary date over the term of the license agreement. The Company has determined that the payment of annual maintenance fees by licensees in future periods represents a significant financing component to the license since the delivery of the license occurs at the inception of the agreement. The present value of aggregate annual maintenance fees payable to the Company over the term of the license is included in the transaction price and recognized as revenue upon the delivery of the license. The discounted portion of the annual maintenance fees is recognized as interest income in the consolidated statements of operations over the term of the license.

Development milestone payments are payable to the Company upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, the Company evaluates whether the milestones are considered probable of achievement and estimates the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within the control of the Company or the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, the Company re-evaluates the probability of achievement of outstanding development milestones and, if necessary, adjusts the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recorded as license revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price for each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee. To date the Company has not recognized any revenue from royalties on sales of licensed products, sales-based milestone payments or sublicense fees.

The Company receives payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees are recorded as accounts receivable when the Company's rights to the consideration is conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when the Company's rights to the consideration is not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property

is returned to the Company and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination.

Costs of Revenues

Licensing costs consist of sublicense fees incurred by the Company to its licensors as a result of license revenues generated by the Company. Sublicense fees are based on a percentage of license fees received by the Company from licensees as specified in the Company's agreements with its licensors. The Company recognizes sublicense fees in the period that the underlying license revenue is recognized. Sublicense fees payable by the Company to licensors in periods beyond 12 months from the reporting are recorded as non-current liabilities on the consolidated balance sheets.

Net Income (Loss) Per Share

Basic net income (loss) per share is calculated by dividing net income (loss) applicable to common stockholders by the weighted-average common shares outstanding during the period, without consideration for common stock equivalents. Diluted net income (loss) per share is calculated by adjusting weighted-average common shares outstanding for the dilutive effect of common

Table of Contents

stock equivalents outstanding for the period, determined using the treasury-stock method. Contingently convertible shares in which conversion is based on non-market-priced contingencies are excluded from the calculations of both basic and diluted net income (loss) per share until the contingency has been fully met. For purposes of the diluted net income (loss) per share calculation, common stock equivalents are excluded from the calculation of diluted net income (loss) per share if their effect would be anti-dilutive.

Recent Accounting Pronouncements

Adoption of ASU 2014-09, Revenue from Contracts with Customers

In May 2014, the FASB issued ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition (Topic 605). Effective January 1, 2018, the Company adopted Topic 606 using the modified retrospective transition method. Under this method, the Company applied Topic 606 to all contracts with customers which were not completed as of January 1, 2018 and recorded the cumulative impact of the adoption as an adjustment to its accumulated deficit on January 1, 2018. The Company's financial results for periods ending after January 1, 2018 are presented in accordance with the requirements of Topic 606, while prior period amounts are not adjusted and continue to be reported in accordance with Topic 605. Please refer to Revenue Recognition above for additional information on Topic 606, including a description of the Company's revenue recognition policies upon adoption.

The Company recorded a net reduction in opening accumulated deficit of $4.8 million as of January 1, 2018 for the cumulative impact of adoption of Topic 606, which was primarily the result of accelerated recognition of license revenue due to annual maintenance fees under Topic 606. Under Topic 605, annual maintenance fees payable to the Company by licensees were recognized as license revenue annually when the amounts became fixed or determinable. Under Topic 606, the present value of aggregate annual maintenance fees over the term of the license agreement are recognized as revenue upon the delivery of the license to the licensee. The impact of the accelerated recognition of license revenue upon adoption was partially offset by the accelerated recognition of licensing costs to the Company's licensors. The Company recognizes sublicense fees to its licensors in the period the underlying license revenue is recognized.

The cumulative adjustment for the adoption of Topic 606 had the following effects on the Company's consolidated balance sheet as of January 1, 2018 (in thousands):

	Balance at December 31, 2017	Cumulative Adjustment for Adoption of Topic 606	Balance at January 1, 2018
Consolidated Balance Sheet			
Assets:			
Accounts receivable, current	$ 473	$ 527	$ 1,000
Other current assets	$ 1,412	$ 350	$ 1,762
Accounts receivable, non-current	$ —	$ 4,850	$ 4,850

Liabilities:			
Accrued expenses and other current liabilities	$ 9,605	$ 105	$ 9,710
Other liabilities	$ —	$ 819	$ 819
Stockholders Equity:			
Accumulated deficit	$ (187,756)	$ 4,803	$ (182,953)

Table of Contents

The following tables present the effects of the adoption of Topic 606 on each financial statement line item of the Company's financial statements for the period ended March 31, 2018 (in thousands):

	As of March 31, 2018		
	As Reported	Impact of Adoption of Topic 606	Results Without Adoption of Topic 606
Consolidated Balance Sheet			
Assets:			
Accounts receivable, current	$25,976	$25,903	$73
Accounts receivable, non-current	$32,645	$32,645	$—
Other current assets	$2,208	$250	$1,958
Liabilities:			
Accrued expenses and other current liabilities	$9,869	$131	$9,738
Other liabilities	$1,776	$1,776	$—
Stockholders Equity:			
Accumulated deficit	$(78,714)	$56,891	$(135,605)

	Three Months Ended March 31, 2018		
	As Reported	Impact of Adoption of Topic 606	Results Without Adoption of Topic 606
Consolidated Statement of Operations			
Revenues:			
License revenue	$132,391	$51,716	$80,675
Expenses:			
Licensing costs	$2,408	$983	$1,425
Other Income:			
Interest income from licensing	$1,355	$1,355	$—
Net Income	$104,239	$52,088	$52,151
Net Income Per Share:			

Basic	$3.30	$1.65	$1.65
Diluted	$3.04	$1.52	$1.52

	Three Months Ended March 31, 2018		
	As Reported	Impact of Adoption of Topic 606	Results Without Adoption of Topic 606
Consolidated Statement of Cash Flows			
Cash Flows from Operating Activities:			
Net income	$104,239	$52,088	$52,151
Imputed interest income from licensing	$(1,355)	$(1,355)	$—
Changes in accounts receivable	$(51,414)	$(51,816)	$402
Changes in other current assets	$(446)	$100	$(546)
Changes in accrued expenses and other current liabilities	$490	$26	$464
Changes in other liabilities	$957	$957	$—

The most significant effect that the adoption of Topic 606 had on the results of operations for the three months ended March 31, 2018, as compared to what results would have been under Topic 605, is related to the amount of revenue recognized under the Company's January 2018 amendment to its license agreement with AveXis, Inc. (AveXis) for the development and commercialization of treatments for spinal muscular atrophy (SMA). Under Topic 606, the Company recognized the present value of all fixed consideration due to the Company under the amendment as revenue during the period, including the present value of the two $30.0

Table of Contents

million payments due to the Company from AveXis in January 2019 and January 2020. Under Topic 605, the Company would not have recognized such revenue until it became fixed and determinable and collectability was reasonably assured. Please refer to Note 7 for further information on license revenue and the Company's accounting analysis for the amended license with AveXis.

Other recently adopted accounting pronouncements

In May 2017, the FASB issued ASU 2017-09, Compensation—Stock Compensation (Topic 718): Scope of Modification Accounting, which clarifies when modification accounting should be applied for changes to terms or conditions of a share-based award. The standard is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted, and is to be applied prospectively upon adoption. The Company adopted this standard effective January 1, 2018. The adoption of this standard did not have a material impact on the Company's financial position or results of operations.

In November 2016, the FASB issued ASU 2016-18, Statement of Cash Flows (Topic 230): Restricted Cash. The standard requires that the statement of cash flows explain the change during the period in the total of cash, cash equivalents and restricted cash. As a result, amounts generally described as restricted cash should be included with cash and cash equivalents when reconciling the beginning-of-period and end-of-period total amounts shown on the statement of cash flows. The standard is effective for annual and interim periods beginning after December 15, 2017, with early adoption permitted, and is to be applied retrospectively to each period presented. The Company adopted this standard effective January 1, 2018. The adoption of this standard did not have a material impact on the Company's consolidated statements of cash flows.

The Company's restricted cash includes money market mutual funds used to collateralize an irrevocable letter of credit as required by the Company's lease agreement for its office space in New York, New York. The following table provides a reconciliation of cash and cash equivalents and restricted cash as reported on the consolidated balance sheets to the total of these amounts as reported at the end of the period in the consolidated statements of cash flows (in thousands):

	March 31, 2018	March 31, 2017
Cash and cash equivalents	$71,870	$82,045
Restricted cash	225	225
Total cash and cash equivalents and restricted cash	$72,095	$82,270

In January 2016, the FASB issued ASU 2016-01, Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities, which modifies the current guidance on the recognition, measurement, presentation and disclosure of financial instruments. In February 2018, the FASB issued ASU 2018-03, Technical Corrections and Improvements to Financial Instruments—Overall (Subtopic 825-10): Recognition and Measurement of Financial Assets and Financial Liabilities, which clarifies the guidance in ASU 2016-01. The Company adopted these standards effective January 1, 2018. Upon the adoption of these standards, the Company elected to measure it non-marketable equity securities without readily available fair values at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. Prior to the adoption of these standards, the Company measured these investments at cost less impairment. The adoption of these standards did not have a material impact on the Company's financial position or results of operations.

Recent accounting pronouncements not yet adopted

In February 2018, the FASB issued ASU 2018-02, Income Statement—Reporting Comprehensive Income (Topic 220): Reclassification of Certain Tax Effects from Accumulated Other Comprehensive Income which amends the current guidance on comprehensive income to provide an option for an entity to reclassify the stranded tax effects of the Tax Cuts and Jobs Act of 2017 (the TCJA) that was signed into law in December 2017 from accumulated other comprehensive income directly to retained earnings. The stranded tax effects result from the remeasurement of deferred tax assets and liabilities which were originally recorded in comprehensive income but whose remeasurement is reflected in the income statement. The standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted upon issuance. The Company is evaluating the application of this standard but has not yet determined the potential effects it may have on the Company’s consolidated financial statements.

In April 2017, the FASB issued ASU 2017-08, Receivables—Nonrefundable Fees and Other Costs (Subtopic 310-20), which amends the required amortization period for certain purchased callable debt securities held at a premium by shortening the amortization period for the premium to the earliest call date. The standard is effective for annual and interim periods beginning after December 15, 2019, with early adoption permitted upon issuance, and is to be applied on a modified retrospective basis through a cumulative-effect adjustment directly to retained earnings as of the beginning of the period of adoption. The Company does not believe the application of this standard will have a material impact on the Company’s financial position or results of operations.

Table of Contents

In June 2016, the FASB issued ASU 2016-13, Financial Instruments—Credit Losses (Topic 326): Measurement of Credit Losses on Financial Instruments, which amends the accounting for credit losses for most financial assets and certain other instruments. The standard requires that entities holding financial assets and net investment in leases that are not accounted for at fair value through net income be presented at the net amount expected to be collected. An allowance for credit losses will be a valuation account that will be deducted from the amortized cost basis of the financial asset to present the net carrying value at the amount expected to be collected on the financial asset. The standard is effective for annual and interim periods beginning after December 15, 2019, with early adoption permitted for annual and interim periods beginning after December 15, 2018. The Company does not believe the application of this standard will have a material impact on the Company's financial position or results of operations.

In February 2016, the FASB issued ASU 2016-02, Leases (Topic 842) which supersedes FASB ASC Topic 840, Leases (Topic 840) and provides principles for the recognition, measurement, presentation and disclosure of leases for both lessees and lessors. The new standard requires lessees to apply a dual approach, classifying leases as either finance or operating leases based on the principle of whether or not the lease is effectively a financed purchase by the lessee. This classification will determine whether lease expense is recognized based on an effective interest method or on a straight-line basis over the term of the lease, respectively. A lessee is also required to record a right-of-use asset and a lease liability for all leases with a term of greater than 12 months regardless of classification. Leases with a term of 12 months or less will be accounted for similar to existing guidance for operating leases. The standard is effective for annual and interim periods beginning after December 15, 2018, with early adoption permitted upon issuance. The Company is evaluating the application of this standard but has not yet determined the potential effects it may have on the Company's consolidated financial statements.

3. Marketable Securities

The following tables present a summary of the Company's marketable securities, which consist solely of available-for-sale debt securities (in thousands):

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
March 31, 2018				
U.S. government and federal agency securities	$ 6,969	$ —	$ (5)	$ 6,964
Commercial paper	3,484	—	—	$ 3,484
Corporate bonds	153,929	—	(463)	$ 153,466
	$ 164,382	$ —	$ (468)	$ 163,914

	Amortized Cost	Unrealized Gains	Unrealized Losses	Fair Value
December 31, 2017				

Corporate bonds	$ 130,018	$ 2	$ (282)	$ 129,738
	$ 130,018	$ 2	$ (282)	$ 129,738

As of March 31, 2018 and December 31, 2017, 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 March 31, 2018 and December 31, 2017, the balance in the Company's accumulated other comprehensive loss consisted solely of net unrealized gains and losses on available-for-sale securities, net of income tax effects and reclassification adjustments for realized gains and losses. During the three months ended March 31, 2018, the Company recognized net unrealized losses on available-for-sale securities of $0.2 million and income tax expense of $0 in other comprehensive loss for the period. The Company did not recognize any realized gains or losses on the sale or maturity of available-for-sale securities during the three months ended March 31, 2018. During the three months ended March 31, 2017, the Company recognized net unrealized losses on available-for-sale securities of $0.1 million and income tax expense of $0 in other comprehensive loss for the period. The Company recognized net realized gains of $0.5 million on the sale or maturity of marketable securities during the three months ended March 31, 2017, which were reclassified out of accumulated other comprehensive loss during the period and are included in investment income in the consolidated statements of operations and comprehensive income (loss).

Table of Contents

The following tables present the fair values and unrealized losses of marketable securities held by the Company in an unrealized loss position for less than and greater 12 months (in thousands):

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
March 31, 2018						
U.S. government and federal agency securities	$6,964	$ (5)	$—	$ —	$6,964	$ (5)
Corporate bonds	120,338	(319)	28,073	(144)	148,411	(463)
	$127,302	$ (324)	$28,073	$ (144)	$155,375	$ (468)

	Less than 12 Months		12 Months or Greater		Total	
	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses	Fair Value	Unrealized Losses
December 31, 2017						
Corporate bonds	$109,238	$ (180)	$17,124	$ (102)	$126,362	$ (282)
	$109,238	$ (180)	$17,124	$ (102)	$126,362	$ (282)

As of March 31, 2018, securities held by the Company which were in an unrealized loss position consisted of 54 investment grade fixed income security positions. The Company has the intent and ability to hold such securities until recovery and has determined that none of its investments were other-than-temporarily impaired as of March 31, 2018 or December 31, 2017.

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 of money market mutual funds. Marketable securities consist of fixed income debt securities. The following tables present the fair value of cash equivalents and marketable securities in accordance with the hierarchy discussed in Note 2 (in thousands):

	Quoted prices in active markets	Significant other observable inputs	Significant unobservable inputs

	(Level 1)	(Level 2)	(Level 3)	Total
March 31, 2018				
Money market mutual funds (cash equivalents)	$ —	$ 71,847	$ —	$ 71,847
U.S. government and federal agency securities (marketable securities)	—	6,964	—	6,964
Commercial paper (marketable securities)	—	3,484	—	3,484
Corporate bonds (marketable securities)	—	153,466	—	153,466
	$ —	$ 235,761	$ —	$ 235,761

	Quoted prices in active markets (Level 1)	Significant other observable inputs (Level 2)	Significant unobservable inputs (Level 3)	Total
December 31, 2017				
Money market mutual funds (cash equivalents)	$ —	$ 46,646	$ —	$ 46,646
Corporate bonds (marketable securities)	—	129,738	—	129,738
	$ —	$ 176,384	$ —	$ 176,384

There were no transfers of financial instruments between levels of the fair value hierarchy during the three months ended March 31, 2018.

Table of Contents

Management estimates that the carrying amounts of its current accounts receivable, accounts payable and accrued expenses and other current liabilities approximate fair value due to the short-term nature of those instruments. Non-current accounts receivable are recorded at their present values using a discount rate that is based on prevailing market rates and the credit profile of the licensee on the date the amounts are initially recorded. Management does not believe there have been any significant changes in market conditions that would cause the discount rates used to be significantly different from those that would be used as of March 31, 2018 to determine the present value of the receivables. Accordingly, management estimates that the carrying value of its non-current accounts receivable approximates the fair value of those instruments. As of March 31, 2018, accounts receivable from AveXis accounted for 93% of the Company's total accounts receivable, current and non-current.

The Company's non-marketable equity securities are measured at cost less impairment, adjusted for observable price changes for identical or similar investments of the same issuer. As of March 31, 2018 and December 31, 2017, non-marketable equity securities had carrying values of $0.4 million and are included in other assets on the consolidated balance sheets. Since the acquisition of the securities, the Company has not identified any observable price changes or changes in circumstances that would have an adverse effect on the fair value of its non-marketable equity securities as of March 31, 2018. No impairment losses on non-marketable equity securities were recorded during the three months ended March 31, 2018 and 2017.

5. Property and Equipment, Net

Property and equipment, net consists of the following (in thousands):

	March 31, 2018	December 31, 2017
Computer equipment and software	$1,638	$ 1,481
Lab equipment	8,945	8,561
Furniture and fixtures	1,495	1,384
Leasehold improvements	6,841	5,828
Total property and equipment	18,919	17,254
Accumulated depreciation and amortization	(4,090)	(3,277)
Property and equipment, net	$14,829	$ 13,977

6. Commitments and Contingencies

Lease Agreements

The Company recognizes rent expense on a straight-line basis over the term of its operating leases commencing on the date the Company takes possession of the leased property. Tenant improvement allowances that are considered to be lease incentives from the lessor are recorded as deferred rent and amortized as a reduction of rent expense over the term of the lease from the possession date.

In March 2015, the Company entered into a non-cancelable operating lease for office space at 9712 Medical Center Drive in Rockville, Maryland (the Medical Center Drive Lease). The lease term commenced in April 2015. Monthly payments under the lease began in October 2015 and escalate annually in accordance with the lease agreement.

In September 2015, November 2015, July 2017 and April 2018, the Company amended the Medical Center Drive Lease to include additional office and laboratory space at 9714 Medical Center Drive, and ultimately extend the term of the lease to September 2021. The Company has options to extend the term of the Medical Center Drive Lease for up to six additional years. Under the amended lease, the Company has received a $0.4 million tenant improvement allowance from the landlord which will be deferred and amortized on a straight-line basis as a reduction of rent expense over the term of the lease.

In January 2016, the Company entered into a 7.5-year, non-cancelable operating lease for its corporate headquarters at 9600 Blackwell Road in Rockville, Maryland (the Blackwell Road Lease). The lease commenced in February 2016, and expires in September 2023. The Company has an option to extend the term of the lease for an additional five years. In November 2017, the Blackwell Road Lease was amended to include additional office space for the remainder of the lease term. Monthly payments under the lease began in September 2016 and escalate annually in accordance with the lease agreement. The Company received a $0.8 million tenant improvement allowance from the landlord which will be deferred and amortized on a straight-line basis as a reduction of rent expense over the term of lease.

Table of Contents

In May 2016, the Company entered into a 51-month, non-cancelable operating lease for additional office space at 400 Madison Avenue in New York, New York. The lease commenced in July 2016, and expires in October 2020. Monthly payments under the lease began in October 2016 and escalate annually in accordance with the lease agreement. Under the terms of the lease agreement, the Company has provided the landlord with an irrevocable letter of credit of $0.2 million which the landlord may draw upon in the event of any uncured default by the Company under the terms of the lease. As of March 31, 2018, the Company has recorded restricted cash of $0.2 million as collateral to the financial institution which issued the letter of credit.

As of March 31, 2018, future minimum lease payments under non-cancelable operating leases are as follows (in thousands):

	Operating Leases
2018 (remainder of year)	$ 1,714
2019	2,296
2020	2,309
2021	1,719
2022	621
Thereafter	479
Total minimum lease payments	$ 9,138

Licenses Granted to the Company

Licenses granted to the Company may require the Company to make future payments relating to sublicense fees, milestone fees for milestones achieved in the future 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. Up-front fees to obtain licensed technology are included in research and development expenses and patent maintenance costs are included in general and administrative expenses in the consolidated statements of operations and comprehensive income (loss). Sublicense fees are based on a specified percentage of license fees earned by the Company and are included in licensing costs in the consolidated statements of operations and comprehensive income (loss). Milestone fees are included in licensing costs in the consolidated statements of operations and comprehensive loss if the underlying milestone is achieved by a licensee, or in research and development expense if the underlying milestone is achieved by the Company as a result of the development of its product candidates. Royalties on sales of licensed reagents for use in research and development are included in costs of reagent sales in the consolidated statements of operations and comprehensive income (loss). The Company has not commercialized any product candidates or paid any royalties under these agreements other than for the sales of licensed reagents.

The Trustees of the University of Pennsylvania. In February 2009, the Company entered into a license agreement, which has been amended from time to time, with The Trustees of the University of Pennsylvania (together with the University of Pennsylvania, Penn) for exclusive, worldwide rights to certain patents owned by Penn underlying the Company’s NAV Technology Platform. Under the terms of the agreement, in consideration for the license, the Company issued to Penn a 24.5% equity interest in the Company 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.

In April 2016, the Company entered into an agreement with Penn whereby the Company will fund clinical trial activities performed by Penn for RGX-501, the Company's product candidate for the treatment of homozygous familial hypercholesterolemia (HoFH). In connection with the agreement, the Company amended its license from Penn to include exclusive license rights to data, results and other information generated in connection with the RGX-501 clinical trial.

Expenses incurred by the Company related to its license from Penn were as follows (in thousands):

	Three Months Ended March 31,	
	2018	2017
Sublicense fees	$—	$46
Maintenance of licensed patents	18	84
	$18	$130

As of March 31, 2018 and December 31, 2017, the Company had accrued $0.1 million and less than $0.1 million, respectively, in expenses payable to Penn under the license agreement, which are included in accounts payable, accrued expenses and other current liabilities and other liabilities on the Company's consolidated balance sheets.

Table of Contents

GlaxoSmithKline LLC. In March 2009, the Company entered into a license agreement, which was amended in April 2009, with GlaxoSmithKline LLC (GSK) for exclusive, worldwide rights to certain patents underlying the Company's NAV Technology Platform which are owned by Penn and exclusively licensed to GSK. Under the terms of the agreement, in consideration for the license, the Company issued to GSK a 19.9% equity interest in the Company 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 also obligated to pay GSK up to $1.5 million upon the achievement of various milestones. From the inception of the agreement through March 31, 2018, the Company has incurred $0.5 million for milestones that have been achieved or are deemed probable of achievement.

Expenses incurred by the Company related to its license from GSK were as follows (in thousands):

	Three Months Ended March 31,	
	2018	2017
Sublicense fees	$2,033	$46
Maintenance of licensed patents	393	145
	$2,426	$191

As of March 31, 2018 and December 31, 2017, the Company had accrued $3.8 million and $0.3 million, respectively, in expenses payable to GSK under the license agreement, which are included in accounts payable, accrued expenses and other current liabilities and other liabilities on the Company's consolidated balance sheets.

Regents of the University of Minnesota. In November 2014, the Company entered into a license agreement, which was amended in November 2016, 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, and reimbursed Minnesota for patent maintenance expenses, for a total of less than $0.1 million. Under the terms of the agreement, the Company is obligated to pay Minnesota annual maintenance fees of less than $0.1 million 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 $0.1 million per licensed product upon the achievement of various milestones. In November 2016, the license with Minnesota was amended to include additional patent rights. In consideration for the additional patent rights, the Company paid an up-front fee of less than $0.1 million. From the inception of the agreement through March 31, 2018, the Company has incurred less than $0.1 million for milestones that have been achieved or are deemed probable of achievement.

Expenses incurred by the Company related to its license from Minnesota were as follows (in thousands):

	Three Months Ended

	March 31,	
	2018	2017
Sublicense fees	$375	$ —
Maintenance of licensed patents	12	6
	$387	$ 6

As of March 31, 2018 and December 31, 2017, the Company had accrued $0.4 million and $0.1 million, respectively, in expenses payable to Minnesota under the license agreement, which are included in accounts payable and accrued expenses on the Company's consolidated balance sheets.

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 potential 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 March 31, 2018 and December 31, 2017, the Company did not have any material indemnification claims that were probable or reasonably possible and consequently has not recorded any related liabilities.

European Patent Office Proceeding

In June 2017, a third party filed an opposition with the European Patent Office (EPO) challenging the validity of a European patent owned by Penn for the AAV8 vector, which the Company has exclusively licensed. This matter is in its early stages and the Company is unable to estimate the outcome of this matter but intends to assist Penn in vigorously defending this patent. The EPO has scheduled oral proceedings to begin on October 26, 2018. As of March 31, 2018, the Company has not recorded any liabilities related to this matter.

Table of Contents

7. License Revenue

Effective January 1, 2018, the Company adopted Topic 606 using the modified retrospective transition method and has applied the new standard to all of its license agreements in effect as of January 1, 2018. Please refer to Note 2 for additional information regarding the adoption of Topic 606. License revenue for periods ending after January 1, 2018 is presented in accordance with the requirements of Topic 606, while prior period amounts are not adjusted and continue to be reported in accordance with Topic 605.

Consideration to the Company under its license agreements may include: (i) up-front fees, (ii) option fees to obtain additional licenses, (iii) annual maintenance fees, (iv) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (v) sublicense fees and (vi) royalties on sales of licensed 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 double-digit percentage of net sales by licensees. To date the Company has not recognized any revenue from sales-based milestone payments, royalties on sales of licensed products or sublicense fees.

Development milestone payments are only included in the transaction price of each license and recognized as revenue to the extent they are considered probable of achievement. Sales-based milestones are excluded from the transaction price of each license agreement and recognized as revenue in the period of achievement. As of March 31, 2018, 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 $26.1 million upon the commencement of various stages of clinical trials, $46.0 million upon the submission of regulatory approval filings, $105.5 million upon the approval of commercial products by regulatory agencies and $212.0 million upon the achievement of specified sales targets for licensed products. The achievement of milestones by licensees is highly dependent on the successful development and commercialization of licensed products and it is at least reasonably possible that some or all of the milestone fees will not be realized by the Company.

During the three months ended March 31, 2018, the Company recognized $0.3 million of license revenue for licenses delivered to licensees in prior periods as a result of increases in the transaction prices of its license agreements for development milestones that were previously not considered probable of achievement. Additionally, the Company recognized $0.2 million of interest income from licensing during the three months ended March 31, 2018 from licenses delivered in prior periods which contained significant financing components. As of March 31, 2018, the Company had recorded current accounts receivable of $26.0 million and non-current accounts receivable of $32.6 million under its license agreements which represent fixed license fees and annual maintenance fees payable to the Company over the term of the agreements, net of discounts for significant financing components. As of March 31, 2018, the Company had recorded contract assets of $0.3 million which represent development milestones that are considered probable of achievement but have not yet been achieved and are included in other current assets on the consolidated balance sheet. As of March 31, 2018, the Company had not recorded any contract liabilities related to contracts with customers and has no material undelivered performance obligations.

As of March 31, 2018, the Company had not recognized any impairment losses on its receivables or contract assets from contracts with customers.

AveXis Inc. March 2014 License and January 2018 Amendment

In March 2014, the Company entered into an exclusive license agreement (the March 2014 License) with AveXis. Under the license, the Company granted AveXis an exclusive, worldwide commercial license, with rights to sublicense, to the NAV AAV9 vector for the treatment of SMA in humans by in vivo gene therapy. In consideration for the license, AveXis paid the Company an up-front fee of $2.0 million, and is required to pay annual maintenance fees, development milestone payments of up to $12.3 million, mid-single to low double-digit royalties on net sales of licensed products, subject to reduction in specified circumstances, and a lower mid-double digit percentage of any sublicense fees AveXis receives from sublicensees for the licensed intellectual property rights.

In January 2018, the Company entered into an amendment (the January 2018 Amendment) to the March 2014 License with AveXis. Under the January 2018 Amendment, the licensed intellectual property was expanded to include, in addition to the NAV AAV9 vector previously licensed, any recombinant AAV vector in the Company’s intellectual property portfolio, during a period of 14 years from the effective date of the January 2018 Amendment, for the treatment of SMA in humans by in vivo gene therapy. The Company may also, in its sole discretion, provide specified collaborative services to AveXis as specified in the January 2018 Amendment.

Table of Contents

The January 2018 Amendment also modified the terms and conditions of the March 2014 License relating to assignment. Under the amended assignment provision, AveXis is permitted to transfer the March 2014 License, as amended, without the Company's consent in connection with a change of control of AveXis, subject to the transferee or successor agreeing in writing to be bound by the terms of the March 2014 License, as amended, and the payment to the Company of certain fees due upon such change of control, as described below. Under the original March 2014 License, any assignment by AveXis without the Company's prior written consent had been prohibited.

Pursuant to the January 2018 Amendment, in consideration for the additional rights granted thereunder and in addition to any consideration owed under the original March 2014 License, AveXis paid to the Company a fee of $80.0 million upon entry into the January 2018 Amendment. In addition, AveXis is obligated to pay the Company (i) $30.0 million on the first anniversary of the effective date of the January 2018 Amendment, (ii) $30.0 million on the second anniversary of the effective date of the January 2018 Amendment and (iii) potential sales-based milestone payments of up to $120.0 million. In the event of a change of control of AveXis, to the extent that any fee described in (i) or (ii) above, or the first $40.0 million of milestone payments described in (iii) above, has not yet been paid to the Company, AveXis will be obliged to pay any such unpaid fee to the Company upon the change of control. For any product developed for the treatment of SMA using the NAV AAV9 vector, AveXis will continue to be obligated to pay to the Company mid-single to low double-digit royalties on net sales as defined in the March 2014 License, and for any product developed for the treatment of SMA using a licensed vector other than NAV AAV9, the Company will receive a low double-digit royalty on net sales.

In April 2018, AveXis announced that it had entered into an agreement and plan of merger pursuant to which it will be acquired by Novartis AG. AveXis announced that it expects to complete the transaction in mid-2018, pending the successful completion of a tender offer and the satisfaction of all other closing conditions. The Company expects that the transaction, if completed, will qualify as a change of control of AveXis under the January 2018 Amendment.

Accounting Analysis

The January 2018 Amendment has been accounted for under Topic 606 as a modification of the license agreement resulting in a new and separate contract from the original March 2014 License for revenue recognition purposes. The only material performance obligation of the Company under the January 2018 Amendment is for the delivery of the modified license, which occurred upon the execution of the amendment in January 2018.

As of March 31, 2018, the transaction price of the original March 2014 License was $3.5 million. The transaction price of $3.5 million includes (i) the up-front payment in March 2014 of $2.0 million, (ii) the present value of aggregate annual maintenance fees payable to the Company over the term of the license and (iii) the development milestones that had been achieved to date. The discounted portion of the annual maintenance fees represents the financing benefit provided to AveXis and is recognized as interest income from licensing over the term of the license. Variable consideration under the original March 2014 License which has been excluded from the transaction price includes payments for remaining development milestones that have not yet been achieved and are not considered probable of achievement, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses, if any.

Upon its execution and as of March 31, 2018, the transaction price of the January 2018 Amendment was $132.1 million, which was fully recognized as license revenue upon the delivery of the modified license in January 2018. The transaction price of $132.1 million includes the following fixed consideration: (i) the $80.0 million payment in January 2018, (ii) the present value of the $30.0 million payment due in January 2019 and (iii) the present value of the $30.0 million payment due in January 2020. The discounted portion of the license fees represents the financing benefit

provided to AveXis and is recognized as interest income from licensing over the financing term of two years from the execution of the amendment. Variable consideration under the January 2018 Amendment which has been excluded from the transaction price includes sales-based milestone payments of $120.0 million, as well as any potential sublicense fees or royalties on sales of licensed products, which will be recognized in the period of the underlying sales or sublicenses, if any. The acceleration of payments under the January 2018 Amendment which would occur upon a change of control of AveXis has not been considered in determining the transaction price as of March 31, 2018 and will not be accounted for until such event occurs and has been completed in full.

During the three months ended March 31, 2018, the Company recognized license revenue of $132.1 million and interest income from licensing of $1.2 million from the March 2014 License, as amended, with AveXis, which includes the amounts from both the original March 2014 License and the January 2018 Amendment. As of March 31, 2018, the Company had recorded $53.5 million of accounts receivable from AveXis under the March 2014 License, as amended, of which $25.4 million are included in current assets and $28.1 million are included in non-current assets on the consolidated balance sheets.

Table of Contents

During the three months ended March 31, 2017, the Company recognized license revenue of $0.1 million from the March 2014 License which was recognized under the requirements of Topic 605. As of December 31, 2017, the Company had no amounts receivable from the March 2014 License under the requirements of Topic 605.

8. Stock-based Compensation

In January 2018, an additional 1,251,810 shares became available for issuance under the 2015 Equity Incentive Plan (the 2015 Plan). As of March 31, 2018, the total number of shares of common stock authorized for issuance under the 2015 Plan and 2014 Stock Plan (the 2014 Plan) was 9,488,413, of which 2,429,185 remain available for future grants under the 2015 Plan.

Stock-based Compensation Expense

The Company's stock-based compensation expense by award type is as follows (in thousands):

	Three Months Ended March 31,	
	2018	2017
Stock options	$3,122	$2,439
Restricted stock units	68	69
Employee stock purchase plan	101	83
	$3,291	$2,591

As of March 31, 2018, the Company had $37.6 million of unrecognized stock-based compensation expense related to stock options, restricted stock units and the 2015 Employee Stock Purchase Plan (the 2015 ESPP), which is expected to be recognized over a weighted-average period of 3.0 years.

The Company has recorded aggregate stock-based compensation expense in the consolidated statements of operations and comprehensive income (loss) as follows (in thousands):

	Three Months Ended March 31,	
	2018	2017
Research and development	$1,530	$1,238
General and administrative	1,761	1,353
	$3,291	$2,591

Stock Options

The following table summarizes stock option activity under the 2014 Plan and 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Exercise Price	Weighted-average Remaining Contractual Life (Years)	Aggregate Intrinsic Value (a)
Outstanding at December 31, 2017	5,468	$ 10.25	7.9	$ 125,738
Granted	914	$ 32.07		
Exercised	(587)	$ 6.57		
Cancelled or forfeited	(169)	$ 15.35		
Outstanding at March 31, 2018	5,626	$ 14.03	8.0	$ 91,837
Exercisable at March 31, 2018	2,825	$ 7.42	7.3	$ 63,385
Vested and expected to vest at March 31, 2018	5,626	$ 14.03	8.0	$ 91,837

(a) The aggregate intrinsic value is calculated as the difference between the exercise price of the underlying options and the fair value of the common stock for the options that were in the money at the dates reported.

Table of Contents

The weighted-average grant date fair value per share of options granted during the three months ended March 31, 2018 was $21.42. During the three months ended March 31, 2018, the total number of stock options exercised was 586,694, resulting in total proceeds of $3.8 million. The total intrinsic value of options exercised during the three months ended March 31, 2018 was $13.7 million.

Restricted Stock Units

The following table summarizes restricted stock unit activity under the 2015 Plan (in thousands, except per share data):

	Shares	Weighted-average Grant Date Fair Value
Unvested balance at December 31, 2017	40	$ 20.90
Granted	—	$ —
Vested	—	$ —
Forfeited	—	$ —
Unvested balance at March 31, 2018	40	$ 20.90

Employee Stock Purchase Plan

As of March 31, 2018, the total number of shares of common stock authorized for issuance under the 2015 ESPP was 254,000, of which 186,752 remain available for future issuance. During the three months ended March 31, 2018, 19,528 shares of common stock were issued under the 2015 ESPP.

9. Income Taxes

The TCJA was signed into law in December 2017, and has resulted in significant changes to the U.S. corporate income tax system. In December 2017, the SEC staff issued Staff Accounting Bulletin No. 118 (SAB 118), which allows the Company to record provisional amounts for the effects of the TCJA in the period it was enacted for a measurement period not extend beyond one year from the enactment date. In accordance with SAB 118, the Company has determined that the impact of the TCJA to its deferred tax assets and liabilities and valuation allowance as of March 31, 2018 and December 31, 2017 is a reasonable estimate and provisional amount. The final impact of the TCJA may differ from this provisional amount due to changes in the Company’s estimates and the issuance of additional regulatory or other guidance. The Company expects to complete its assessment of the final impact of the TCJA within the required measurement period under SAB 118.

The Company's net deferred tax assets decreased during the three months ended March 31, 2018, primarily as a result of the expected utilization of federal and state net operating loss carryforwards. The decrease in deferred tax assets was offset by a corresponding decrease in the Company's valuation allowance resulting in no impact on the Company's tax provision for the period.

The Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets. Based on the Company's history of operating losses, the Company has concluded that it is more likely than not that the benefit of its deferred tax assets will not be realized. Accordingly, the Company has provided a full valuation allowance for its net deferred tax assets as of March 31, 2018 and December 31, 2017.

10. Related Party Transactions

FOXKISER LLP

Effective January 2017, the Company entered into a Professional Services Agreement with FOXKISER LLP (FOXKISER), an affiliate of certain stockholders of the Company and an affiliate of a member of the Company's Board of Directors, pursuant to which the Company pays a fixed monthly fee in consideration for certain strategic planning, development and regulatory services provided by FOXKISER. The agreement expired in December 2017, and effective January 2018 the Company entered into a new Professional Services Agreement with FOXKISER, which has a term of one year and is terminable by either party, at any time, upon 60 days' prior written notice to the other party. Costs incurred under the agreements with FOXKISER for the three months ended March 31, 2018 and 2017 were $0.5 million and $0.4 million, respectively. Costs incurred under the agreements with FOXKISER are recorded as research and development expenses in the consolidated statements of operations and comprehensive income (loss).

Table of Contents

Scientific Founder and Special Advisor

In September 2014, the Company entered into an advisory agreement, as amended, with James M. Wilson, M.D., Ph.D., the Company's Scientific Founder and Special Advisor (formerly the Company's Chief Scientific Advisor), who is also the Chairman of the Company's Scientific Advisory Board. The agreement required a fixed monthly payment in consideration for scientific advisory services and expired in March 2017. Dr. Wilson will continue to provide services at no cost to the Company pursuant to a new advisory agreement entered into in March 2017. During the three months ended March 31, 2017, the Company incurred advisory fees of $0.1 million under the agreements. Additionally, in 2014 and 2015 the Company granted options to purchase a total of 211,600 shares of common stock to Dr. Wilson as compensation for the advisory services to be provided to the Company, which vest partially upon the completion of service conditions and partially upon the achievement of specified performance conditions as set forth in the award agreements.

11. Net Income (Loss) Per Share

The computations of basic and diluted net income (loss) per share are as follows (in thousands, except per share data):

	Three Months Ended March 31,	
	2018	2017
Basic net income (loss) per share:		
Net income (loss) applicable to common stockholders	$104,239	$(21,993)
Shares used in computation:		
Weighted-average common shares outstanding	31,632	26,673
Basic net income (loss) per share	$3.30	$(0.82)
Diluted net income (loss) per share:		
Net income (loss) applicable to common stockholders	$104,239	$(21,993)
Shares used in computation:		
Weighted-average common shares outstanding	31,632	26,673
Stock options	2,620	—
Restricted stock units	22	—
Employee stock purchase plan	1	—
Weighted-average diluted common shares	34,275	26,673
Diluted net income (loss) per share	$3.04	$(0.82)

For periods in which the Company incurred net losses applicable to common stockholders, common stock equivalents are excluded from the calculation of diluted net loss per share as their effect would be anti-dilutive, and accordingly, basic and diluted net loss per share are the same for such periods. Outstanding stock options with exercise prices greater than the average market price of common stock are excluded from the calculation of diluted net income (loss) per share as their effect would be anti-dilutive. The following potentially dilutive common stock equivalents

outstanding at the end of the period were excluded from the computations of weighted-average diluted common shares for the periods indicated as their effects would be anti-dilutive (in thousands):

	Three Months Ended March 31,	
	2018	2017
Stock options issued and outstanding	1,582	5,724
Unvested restricted stock units outstanding	—	40
Employee stock purchase plan	—	16
	1,582	5,780

Table of Contents

12. Supplemental Disclosures

Accrued expenses and other current liabilities consists of the following (in thousands):

	March 31, 2018	December 31, 2017
Accrued personnel costs	$3,771	$ 5,789
Accrued external research and development expenses	2,851	2,072
Accrued licensing costs	1,555	—
Accrued external general and administrative expenses	1,184	1,078
Accrued purchases of property and equipment	86	430
Other accrued expenses and current liabilities	422	236
	$9,869	$ 9,605

Table of Contents

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 and the notes thereto included in our Annual Report on Form 10-K for the year ended December 31, 2017, which we filed with the SEC on March 6, 2018. In addition, 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.

Overview

We are a leading clinical-stage biotechnology company seeking to improve lives through the curative potential of gene therapy. Our gene therapy product candidates are designed to deliver genes to cells to address genetic defects or to enable cells in the body to produce therapeutic proteins that are intended to impact disease. Through a single administration, our gene therapy product candidates are designed to provide long-lasting effects, potentially significantly altering the course of disease and delivering improved patient outcomes.

Overview of Product Candidates

We have developed an internal pipeline of product candidates across the therapeutic areas of retinal, metabolic and neurodegenerative diseases.

- RGX-314: Our lead product candidate RGX-314 is for the treatment of wet age-related macular degeneration (wet AMD), a leading cause of total and partial vision loss in the United States, Europe and Japan. We began enrollment in the Phase I clinical trial for RGX-314 for the treatment of wet AMD in May 2017 and have completed dosing of three cohorts of six patients each, a total of 18 patients. We expect to present topline data from the Phase I clinical trial in late 2018.
- RGX-501: We are developing RGX-501 for the treatment of homozygous familial hypercholesterolemia (HoFH), a severe genetic disease characterized by premature and aggressive plaque buildup, life threatening coronary artery disease and aortic valve disease predominantly due to abnormalities in the function or expression of the low-density lipoprotein receptor. Enrollment in the Phase I/II clinical trial for RGX-501 began in March 2017. We have completed dosing of the first cohort of three patients and have dosed three patients in the second cohort, a total of six patients. We expect to present topline data from the Phase I/II clinical trial in late 2018.
- RGX-111: We are developing RGX-111 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type I (MPS I), a severe genetic lysosomal storage disease caused by deficiency of -l-iduronidase (IDUA), an enzyme required for breakdown of cellular waste products. The investigational new drug (IND) application filed with the U.S. Food and Drug Administration (the FDA) for RGX-111 for the treatment of MPS I is active and we expect to begin enrollment in a Phase I clinical trial in mid-2018.
- RGX-121: We are developing RGX-121 for the treatment of the neurological symptoms of Mucopolysaccharidosis Type II (MPS II), a severe genetic lysosomal storage disease with a similar phenotype to MPS I. MPS II is caused by deficiency of iduronate-2-sulfatase (IDS), an enzyme that is also responsible for breakdown of cellular waste products. The IND application filed with the FDA for RGX-121 for the treatment of MPS II is active and we expect to begin enrollment in a Phase I/II clinical trial in mid-2018.

In addition to our lead product candidates, we have also funded, and plan to continue to fund, preclinical research on potential product candidate programs that may become part of our internal product development pipeline. We have partnered with a number of leading academic institutions and will continue to seek partnerships with innovative

institutions to develop novel NAV gene therapy product candidates. We expect to announce an additional lead product candidate in the second half of 2018.

Overview of Our NAV Technology Platform

In addition to our internal product development efforts, we also selectively sublicense our proprietary adeno-associated virus gene therapy delivery platform (NAV Technology Platform) to other leading biotechnology companies, which we refer to as NAV Technology Licensees. As of March 31, 2018, our NAV Technology Platform was being applied in the development of more than 20 partnered product candidates by our NAV Technology Licensees. Sublicensing allows us to maintain our internal product development focus on our core disease indications and therapeutic areas while still expanding the NAV gene therapy pipeline, developing a greater breadth of treatments for patients, providing additional technological and potential clinical proof-of-concept for our NAV Technology Platform, and creating potential additional revenue.

Table of Contents

Financial Overview

Revenues

To date, we have primarily generated revenue through our licensing agreements with our NAV Technology Licensees for research, development and commercialization of product candidates using our proprietary technology. 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 materially compromised.

We license our NAV Technology Platform 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 using the NAV Technology Platform. License agreements generally have a term at least equal to the life of the underlying patents, but are terminable at the option of the licensee. Consideration from licensees under our license agreements may include: (i) up-front fees, (ii) option fees to obtain additional licenses, (iii) annual maintenance fees, (iv) milestone payments based on the achievement of certain development and sales-based milestones by licensees, (v) sublicense fees and (vi) royalties on sales of licensed products. To date we have not recognized any revenue from sales-based milestone payments, royalties on sales of licensed products or sublicense fees.

Future license revenue is highly dependent on the successful development and commercialization of products by our licensees, which is uncertain, and revenue may fluctuate significantly from period to period. Additionally, we may never receive consideration in our license agreements that is contemplated on option fees, development and sales-based milestone payments, royalties on sales of licensed products or sublicense fees, given the contingent nature of these payments.

Effective January 1, 2018, we adopted ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition (Topic 605). We adopted Topic 606 using the modified retrospective transition method and have applied the new standard to all of our license agreements in effect as of January 1, 2018. License revenue for periods ending after January 1, 2018 is presented in accordance with the requirements of Topic 606, while prior period amounts are not adjusted and continue to be reported in accordance with Topic 605.

Operating Expenses

We classify our expenses into three primary 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 indirect 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 consist of sublicense fees to licensors related to the generation of revenue from the licensing of our NAV Technology Platform. Future costs of revenue are uncertain due to the nature of our license agreements and

significant fluctuations in costs of revenue may occur from period to period.

Research and Development Expense

Our research and development expense primarily consists of:

- salaries and personnel-related costs, including benefits, stock-based compensation and travel, 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 product candidate development;
- other costs in seeking regulatory approval of our product candidates; and
- allocated facility-related costs, depreciation expense and other overhead.

Table of Contents

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 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:

- a Phase I clinical trial to evaluate the safety and efficacy of our RGX-314 program for the treatment of wet AMD;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-501 program for the treatment of HoFH;
- a Phase I clinical trial to evaluate the safety and efficacy of our RGX-111 program for the treatment of MPS I;
- a Phase I/II clinical trial to evaluate the safety and efficacy of our RGX-121 program for the treatment of MPS II;
- preclinical research and development for additional product candidates addressing other diseases in the retinal, metabolic and neurodegenerative therapeutic areas;
- continued investment in advanced manufacturing analytics and process development activities; and
- continued acquisition and manufacture of clinical trial materials in support of our anticipated clinical trials.

The following table summarizes our research and development expenses incurred during the three months ended March 31, 2018 and 2017 (in thousands):

	Three Months Ended March 31,	
	2018	2017
Direct Expenses		
RGX-314	$1,620	$1,367
RGX-501	4,149	2,425
RGX-111	1,233	1,195
RGX-121	958	2,863
Total direct expenses:	7,960	7,850
Unallocated Expenses		
Unallocated external expenses	2,616	2,388
Personnel-related	7,371	5,416
Facilities and depreciation expense	1,221	704
Other unallocated	382	261
Total unallocated expenses	11,590	8,769
Total research and development	$19,550	$16,619

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

General and Administrative Expense

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

insurance costs, costs of our information systems and other commercial and general corporate activities. We expect that our general and administrative expense will continue to increase as we continue to develop, and potentially commercialize, our product candidates.

Other Income

Interest Income from Licensing

In accordance with our revenue recognition policies described below and in Note 2 to the accompanying unaudited financial statements, interest income from licensing consists of imputed interest recognized from significant financing components identified in our license agreements with NAV Technology Licensees.

Table of Contents

Investment Income

Investment income consists of interest income earned and gains and losses realized from our cash equivalents and marketable securities. Cash equivalents are comprised of money market mutual funds and marketable securities are comprised of fixed income debt securities.

Critical Accounting Policies and Significant Judgments and Estimates

This Management's Discussion and Analysis of 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 U.S. 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 and recently announced accounting pronouncements, including the expected impact of such pronouncements, are 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 Annual Report on Form 10-K for the year ended December 31, 2017. Other than the critical accounting policies discussed below, there have been no significant changes in our critical accounting policies since December 31, 2017.

Revenue Recognition

Effective January 1, 2018, we adopted ASU 2014-09, Revenue from Contracts with Customers (Topic 606), which supersedes the revenue recognition requirements in ASC 605, Revenue Recognition (Topic 605). Topic 606 requires entities to recognize revenue when control of the promised goods or services is transferred to customers at an amount that reflects the consideration to which the entity expects to be entitled to in exchange for those goods or services. The following five steps are performed to determine the appropriate revenue recognition for arrangements within the scope of Topic 606: (i) identify the contract(s) with a customer, (ii) identify the performance obligations in the contract, (iii) determine the transaction price, (iv) allocate the transaction price to the performance obligations in the contract and (v) recognize revenue when (or as) the entity satisfies the performance obligations.

We apply the five-step model to contracts that are within the scope of Topic 606 only when it is probable that we will collect the consideration we are entitled to in exchange for the goods or services we transfer to the customer. At contract inception, for contracts within the scope of Topic 606, we assess the goods or services promised within each contract and determined those that are performance obligations and whether each promised good or service is distinct. We then recognize as revenue the amount of the transaction price that is allocated to respective performance obligations when (or as) the respective performance obligations are satisfied.

We evaluate our contracts for the presence of significant financing components. If a significant financing component is identified in a contract and provides a financing benefit to the customer, the transaction price for the contract is adjusted to account for the financing portion of the arrangement, which is recognized as interest income over the financing term using the effective interest method. In determining the appropriate interest rates for significant financing components, we evaluate the credit profile of the customer and prevailing market interest rates and select an

interest rate which we believe would be charged to the customer in a separate financing arrangement over a similar financing term.

License revenue

We have determined that all of our license agreements are contracts with customers within the scope of Topic 606. Although licenses are terminable at the option of licensee, we have determined that there is a substantive termination penalty associated with the termination of each license. Due to the substantive termination penalty, the contract term for purposes of applying Topic 606 is equal to the stated term of the license agreement, which is the life of the underlying licensed patents. The only identified performance obligations in our license agreements are for the delivery of intellectual property licenses to licensees. The transaction price for each license agreement is fully allocated to the delivery of the license(s) and is recognized as revenue upon the delivery of the license(s) to the licensee, which generally occurs upon the execution of the license agreement.

For license agreements which contain options for the licensee to purchase additional licenses in the future, we evaluate the options at the inception of the agreement to determine if they provide a material right to the licensee. In making this determination, we consider whether the optional licenses are priced at a discount to the standalone selling price for the licenses. We have determined that none of the options provided to licensees in its license agreements represent material rights to licensees, and therefore do not represent

Table of Contents

performance obligations until exercised by the licensee. Consideration contingent upon the exercise of options by licensees is excluded from the transaction price and not accounted for as part of the license agreement until the option is exercised. Upon the exercise of an option by a licensee, the additional consideration related to the option exercise is added to the transaction price and recognized as revenue upon the delivery of the newly purchased license.

We evaluate the transaction price for our license agreements at each reporting date and recognize changes to the transaction price as revenue during the period, provided that the associated license has been delivered to the licensee. The transaction price for each license includes all fixed consideration, as well as variable consideration to the extent that it is probable that a significant reversal of revenue will not occur in the future. Fixed consideration under our license agreements includes up-front fees and annual maintenance fees. Variable consideration under our license agreements includes development and sales-based milestone payments, sublicense fees and royalties on sales of licensed products.

Up-front license fees are included in the transaction price and recognized as revenue upon the delivery of the license. If up-front license fees are payable to us in periods beyond 12 months from the delivery of the license, a significant financing component is deemed to exist and we adjust the transaction price to include only the present value of the license fees. The discounted portion of the license fees is recognized as interest income in the consolidated statements of operations over the term of the financing period.

Annual maintenance fees are generally payable to us on each anniversary date over the term of the license agreement. We have determined that the payment of annual maintenance fees by licensees in future periods represents a significant financing component to the license since the delivery of the license occurs at the inception of the agreement. The present value of aggregate annual maintenance fees payable to us over the term of the license is included in the transaction price and recognized as revenue upon the delivery of the license. The discounted portion of the annual maintenance fees is recognized as interest income in the consolidated statements of operations over the term of the license.

Development milestone payments are payable to us upon the achievement of specified development milestones by licensees. At the inception of each license agreement that contains development milestone payments, we evaluate whether the milestones are considered probable of achievement and estimate the amount to be included in the transaction price using the most likely amount method. If it is probable that a significant revenue reversal will not occur in the future, milestone payments are included in the transaction price and recognized as revenue upon the delivery of the license. Milestone payments contingent on the achievement of development milestones that are not within our control or the control of the licensee, such as regulatory approvals, are not considered probable of being achieved and are excluded from the transaction price until the milestone is achieved. At each reporting date, we re-evaluate the probability of achievement of outstanding development milestones and, if necessary, adjust the transaction price for any milestones for which the probability of achievement has changed due to current facts and circumstances. Any such adjustments are recorded on a cumulative catch-up basis and recorded as license revenue in the period of the adjustment.

Royalties on sales of licensed products, sales-based milestone payments and sublicense fees based on the receipt of certain fees by licensees from any sublicensees are excluded from the transaction price for each license and recognized as revenue in the period that the related sales or sublicenses occur, provided that the associated license has been delivered to the licensee. To date we have not recognized any revenue from royalties on sales of licensed products, sales-based milestone payments or sublicense fees.

We receive payments from licensees based on the billing schedules established in each license agreement. Amounts recognized as revenue which have not yet been received from licensees are recorded as accounts receivable when our rights to the consideration is conditional solely upon the passage of time. Amounts recognized as revenue which have not yet been received from licensees are recorded as contract assets when our rights to the consideration is not unconditional. Contract assets are recorded as other current assets on the consolidated balance sheets. If a licensee elects to terminate a license prior to the end of the license term, the licensed intellectual property is returned to us and any consideration recorded as accounts receivable or contract assets which is not contractually payable by the licensee is charged off as a reduction of license revenue in the period of the termination.

Impact of Adoption of Topic 606

We recorded a net reduction in opening accumulated deficit of $4.8 million as of January 1, 2018 for the cumulative impact of adoption of Topic 606, which was primarily the result of accelerated recognition of license revenue due to annual maintenance fees under Topic 606. Under Topic 605, annual maintenance fees payable to us by licensees were recognized as license revenue annually when the amounts became fixed or determinable. Under Topic 606, the present value of aggregate annual maintenance fees over the term of the license agreement are recognized as revenue upon the delivery of the license to the licensee. The impact of the accelerated recognition of license revenue upon adoption was partially offset by the accelerated recognition of licensing costs to our licensors. We recognize sublicense fees in the period the underlying license revenue is recognized.

Table of Contents

The most significant effect that the adoption of Topic 606 had on our results of operations for the three months ended March 31, 2018, as compared to what results would have been under Topic 605, is related to the amount of revenue recognized under the January 2018 amendment to our license agreement with AveXis, Inc. (AveXis) for the development and commercialization of treatments for spinal muscular atrophy. Under Topic 606, we recognized the present value of all fixed consideration under the amendment as revenue during the period, including the present value of the two $30.0 million payments due from AveXis in January 2019 and January 2020. Under Topic 605, we would not have recognized such revenue until it became fixed and determinable and collectability was reasonably assured. Please refer to Note 2 to the accompanying unaudited consolidated financial statements for further information regarding the adoption of Topic 606 and the impact to our financial position and results of operations.

Results of Operations

	Three Months Ended March 31,		
	2018	2017	Change
	(in thousands)		
Revenues			
License revenue	$132,391	$455	$131,936
Total revenues	132,391	455	131,936
Expenses			
Costs of revenues			
Licensing costs	2,408	91	2,317
Research and development	19,550	16,619	2,931
General and administrative	8,380	6,622	1,758
Other operating expenses	28	45	(17)
Total operating expenses	30,366	23,377	6,989
Income (loss) from operations	102,025	(22,922)	124,947
Other Income			
Interest income from licensing	1,355	—	1,355
Investment income	859	929	(70)
Total other income	2,214	929	1,285
Net income (loss)	$104,239	$(21,993)	$126,232

Comparison of the Three Months Ended March 31, 2018 and 2017

License Revenue. License revenue increased by $131.9 million, from $0.5 million for the three months ended March 31, 2017 to $132.4 million for the three months ended March 31, 2018. This increase is primarily attributable to the amendment of our March 2014 license with AveXis in January 2018, which resulted in $132.1 million of license revenue recognized during the three months ended March 31, 2018. The $132.1 million of license revenue recognized from the amendment of the AveXis license consists of the present value of the $140.0 million in fixed consideration under the amendment to the license, of which $80.0 million was received in January 2018 and $60.0 million of which is payable by AveXis in two $30.0 million installments in January 2019 and January 2020. The license payments from AveXis that were recognized as revenue during the three months ended March 31, 2018 are non-recurring. Accordingly, we expect license revenue to be substantially lower for the remainder of 2018 and for the foreseeable future. The increase in license revenue during the three months ended March 31, 2018 also resulted in a $2.3 million increase in licensing costs incurred during the period related to the sublicense fees we are obligated to pay to our

licensors.

Research and Development Expense. Research and development expenses increased by $2.9 million, from $16.6 million for the three months ended March 31, 2017 to $19.6 million for the three months ended March 31, 2018. This increase is primarily attributable to the following:

- an increase of $2.0 million for personnel costs as a result of increased employee headcount of research and development personnel, including a $0.3 million increase in stock-based compensation expense;
- an increase of $1.1 million for laboratory costs and facilities and equipment used by research and development personnel, including a $0.3 million increase in depreciation expense allocated to research and development functions; and
- an increase of $0.9 million for costs associated with clinical trial activities for our lead product candidates.

Table of Contents

The increase in research and development expenses was partially offset by a decrease of $1.3 million for externally sourced preclinical research and development, process development and manufacturing-related services.

General and Administrative Expense. General and administrative expenses increased by $1.8 million, from $6.6 million for the three months ended March 31, 2017 to $8.4 million for the three months ended March 31, 2018. This increase is primarily attributable to the following:

- an increase of $0.7 million for professional services, including legal, accounting, commercial and other advisory services; and
- an increase of $0.6 million for personnel costs as a result of increased employee headcount of general and administrative personnel, including a $0.4 million increase in stock-based compensation expense.

Liquidity and Capital Resources

As of March 31, 2018, we had cash, cash equivalents and marketable securities of $235.8 million, which were primarily derived from the sale of preferred stock and common stock. Additionally, we have supplemented our cash flows with fees received from granting commercial licenses to our proprietary technology to other biotechnology and pharmaceutical companies, including $80.0 million received from AveXis during the three months ended March 31, 2018. We expect that our cash, cash equivalents and marketable securities as of March 31, 2018, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan.

In January 2018, we amended our March 2014 license agreement with AveXis which modified its terms and conditions and provided additional intellectual property rights to AveXis. In consideration for the additional rights granted under the amended license agreement, AveXis paid the Company $80.0 million upon the effective date of the amendment. In addition, AveXis is obligated to pay us (i) $30.0 million on the first anniversary of the effective date of the January 2018 Amendment, (ii) $30.0 million on the second anniversary of the effective date of the January 2018 Amendment and (iii) potential sales-based milestone payments of up to $120.0 million. In the event of a change of control of AveXis, to the extent that any fee described in (i) or (ii) above, or the first $40.0 million of milestone payments described in (iii) above, has not yet been paid to us, AveXis will be obligated to pay any such unpaid fee to us upon the change of control. In April 2018, AveXis announced that it had entered into an agreement and plan of merger pursuant to which it will be acquired by Novartis AG. AveXis announced that it expects to complete the transaction in mid-2018, pending the successful completion of a tender offer and the satisfaction of all other closing conditions. We expect that the transaction, if completed, will qualify as a change of control of AveXis under the January 2018 Amendment.

We have incurred cumulative losses since our inception and as of March 31, 2018, had an accumulated deficit of $78.7 million. Our transition to recurring profitability is dependent upon the successful development, approval and commercialization of our product candidates and achieving a level of revenues adequate to support our cost structure. We do not expect to achieve such revenues, and expect to 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 will need significant 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

	Three Months Ended March 31,	
	2018	2017
	(in thousands)	
Net cash provided by (used in) operating activities	$58,134	$(17,481)
Net cash provided by (used in) investing activities	(37,086)	3,163
Net cash provided by financing activities	4,166	71,523
Net increase in cash and cash equivalents and restricted cash	$25,214	$57,205

Operating Activities

Our net cash provided by operating activities for the three months ended March 31, 2018 increased by $75.6 million from the three months ended March 31, 2017. This increase is primarily attributable to the $80.0 million payment we received in January 2018 in connection with the amendment of the March 2014 license agreement with AveXis and is partially offset by an increase in operating expenses during the period.

Table of Contents

For the three months ended March 31, 2018, our net cash provided by operating activities of $58.1 million consisted of net income of $104.2 million and $3.2 million in adjustments for non-cash items, offset by changes in working capital of $49.3 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $3.3 million, depreciation and amortization expense of $0.8 million and net amortization of premiums on marketable debt securities of $0.4 million and were partially offset by imputed interest earned from our license agreements of $1.4 million. The change in working capital was primarily attributable to an increase in accounts receivable of $51.4 million, which is largely driven by accounts receivable recorded in connection with the amendment of the March 2014 license agreement with AveXis in January 2018.

For the three months ended March 31, 2017, our net cash used in operating activities of $17.5 million consisted of a net loss of $22.0 million, offset by $3.2 million in adjustments for non-cash items and changes in working capital of $1.3 million. Adjustments for non-cash items primarily consisted of stock-based compensation expenses of $2.6 million, depreciation and amortization expense of $0.6 million and net amortization of premiums on marketable debt securities of $0.5 million. The change in working capital was primarily attributable to an increase in accounts payable of $2.5 million and a decrease in accounts receivable of $0.8 million, partially offset by a decrease in accrued expenses and other current liabilities of $1.9 million.

Investing Activities

For the three months ended March 31, 2018, net cash used in investing activities consisted of $54.3 million to purchase marketable securities and $2.3 million to purchase property and equipment, offset by $19.5 million in sales and maturities of marketable securities.

For the three months ended March 31, 2017, net cash provided by investing activities consisted of $11.3 million in sales and maturities of marketable securities, partially offset by $5.2 million to purchase marketable securities and $2.9 million to purchase property and equipment.

Financing Activities

For the three months ended March 31, 2018, net cash provided by financing activities consisted of $4.2 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

For the three months ended March 31, 2017, net cash provided by financing activities primarily consisted of $71.2 million in aggregate net proceeds from a follow-on public offering of common stock, net of underwriting discounts and commissions and offering expenses paid by us during the period, as well as $0.3 million in proceeds received from the exercise of stock options and issuance of common stock under our employee stock purchase plan.

Future Funding Requirements

To date, we have primarily generated revenue through license agreements with strategic partners for research, development and commercialization of product candidates using our proprietary technology. 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 product candidates. In addition, subject to obtaining regulatory approval for our 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.

We expect that our cash, cash equivalents and marketable securities as of March 31, 2018 will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan. We intend to devote the majority of our current capital to clinical development and regulatory approval of our product candidates. Because of the numerous risks and uncertainties associated with the development and commercialization of gene therapy product candidates, we are unable to estimate the amount of increased capital outlays and operating expenditures necessary to complete the development of product candidates. Additionally, our estimates are based on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;

Table of Contents

- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- our planned expansion of the licensing of our NAV Technology Platform;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials, if any, for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity, if any;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities 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 or collaborations 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, and no products have been commercialized by us or our NAV Technology Licensees using our NAV Technology Platform to date. 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. 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.

Contractual Obligations, Commitments and Contingencies

There have been no material changes to our contractual obligations, commitments and contingencies from the information provided in Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations,” included in our Annual Report on Form 10-K for the year ended December 31, 2017.

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.

32

Table of Contents

Item 3. Quantitative and Qualitative Disclosures about Market Risk.

For additional information regarding market risk, refer to Item 7A, “Quantitative and Qualitative Disclosures About Market Risk,” included in our most recent Annual Report on Form 10-K for the year ended December 31, 2017. There have been no material changes to our exposure to market risk during the three months ended March 31, 2018.

Item 4. Controls and Procedures.

Conclusion Regarding the Effectiveness of Disclosure Controls and Procedures

Our management, with the participation of our Chief Executive Officer and our Chief Financial Officer, evaluated the effectiveness of our disclosure controls and procedures as of March 31, 2018. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is accumulated and communicated to the company’s management, including its principal executive and principal financial officers, as appropriate to allow timely decisions regarding required disclosure.

Based on this evaluation, our Chief Executive Officer and Chief Financial Officer concluded that, as of March 31, 2018, our disclosure controls and procedures were effective at a reasonable assurance level.

Changes in Internal Control over Financial Reporting

During the quarter ended March 31, 2018, we implemented appropriate changes to our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) to support the revenue recognition and disclosure requirements of Topic 606, which we adopted on January 1, 2018. There were no other changes in our internal control over financial reporting that occurred during the quarter ended March 31, 2018 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Limitations on the Effectiveness of Controls

Control systems, no matter how well conceived and operated, are designed to provide a reasonable, but not an absolute, level of assurance that the objectives of the control system are met. Further, the design of a control system must reflect the fact that there are resource constraints, and the benefits of controls must be considered relative to their costs. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that all control issues and instances of fraud, if any, have been detected. Because of the inherent limitations in any control system, misstatements due to error or fraud may occur and not be detected.

Table of Contents

PART II – OTHER INFORMATION

Item 1. Legal Proceedings.

From time to time, we are party to various lawsuits, claims or other legal proceedings that arise in the normal course of our 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 on Form 10-Q and in our other public filings in evaluating our business, including our Annual Report on Form 10-K for the year ended December 31, 2017, which we filed with the SEC on March 6, 2018. Any of the following risks could materially and adversely affect our business, financial condition or results of operations. In addition, these risks could cause actual results and developments to differ materially and adversely from those projected in the forward-looking statements contained in this Quarterly Report on Form 10-Q (please read the Information Regarding Forward-Looking Statements appearing at the beginning of this Form 10-Q). 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. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere.

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 U.S. 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. Only a few gene therapy products have been approved in the United States, the European Union or elsewhere. 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 the United States, the European Union or elsewhere, or how long it will take to commercialize our product candidates. Furthermore, approvals by one regulatory authority may not be indicative of what other regulatory authorities may require for approval, and approvals of ex vivo gene therapy products may not be indicative of what may be required for approval of in vivo gene therapy products.

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 U.S. 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, according to NIH, the RAC will 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 application (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 preclinical studies or 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.

Table of Contents

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. The development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant European Union guidelines, and we may be required to comply with new guidelines concerning the development and marketing authorization for gene therapy medicinal products.

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

Our business depends substantially on the success of our lead product candidates. If we are unable to obtain regulatory approval for, or successfully commercialize, our lead product candidates, our business will be materially harmed.

Our lead product candidates are in the early stages of development and will require 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 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 or patient availability to complete the necessary 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, and modifications to the design of our clinical trials could delay their enrollment, commencement or completion;
- subjects in our clinical trials 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 undertaken by licensees under a license we grant of certain intellectual property related to our NAV Technology Platform (our NAV Technology Licensees), or undertaken by others using AAV, may die or suffer other adverse effects for reasons that may or may not be related to our NAV Technology Platform or AAV;
- 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 the expected conditions, 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.

Table of Contents

Of the large number of biologics and drugs in development in the biopharmaceutical 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 in large part upon our ability to identify, develop and commercialize products based on our NAV Technology Platform. RGX-111, RGX-121, RGX-314 and RGX-501 are our only clinical programs and 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 for multiple 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 tested only two of our product candidates in our own clinical trials.

Gene therapy development has inherent risks. Only two of our product candidates, RGX-314 and RGX-501, have ever been used in a clinical trial, our lead product candidates have limited preclinical results 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 current or planned clinical trials will be successful, and any safety concerns observed in any one of our current or 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 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.

Table of Contents

Because we are developing product candidates for the treatment of certain diseases in which there is little clinical experience and we are using new endpoints or methodologies, there is increased risk that the FDA, the EMA or other 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 other 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. The EMA and other regulatory authorities in the European Union and other countries may make similar comments with respect to these endpoints and data.

The results from our preclinical studies or clinical trials for our product candidates may not support as broad a marketing approval as we seek, and the FDA, 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 product candidates should be applicable for the treatment of patients with certain conditions, the results from our preclinical and planned clinical trials may not support as broad of a marketing approval as we seek. Even if we obtain regulatory approval for our product candidates, we may be required by the FDA, the EMA or other regulatory bodies to conduct additional clinical trials to support approval of our product candidates for patients diagnosed with different mutations of the respective diseases to which our product candidates relate. This could result in our experiencing significant increases in costs and substantial delays in obtaining, or never obtaining, marketing approval for our product candidates to treat patients. The inability to market our product candidates to treat patients for the intended indications 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 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, the need and length of time required to discontinue other treatment or for other reasons, the timeline for recruiting patients, conducting studies and obtaining regulatory approval of our product candidates may be delayed, perhaps significantly. For example, due to the novel mechanism of our product candidates, we may implement a screening and clinical protocol that is innovative for gene therapy clinical trials, including requiring the discontinuation of some current therapies for a certain period of time before treatment administration. 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;

Table of Contents

- need and length of time required to discontinue other potential treatment options;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective subjects;
- ability to obtain and maintain subject consent;
- risk that enrolled subjects will drop out before completion of the trial;
- patient referral practices of physicians; and
- ability to monitor subjects adequately during and after treatment.

Our current product candidates are being developed to treat a variety of conditions, many of which are rare. We plan to seek marketing approvals worldwide. 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, the EMA or other 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 commencement and 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, including 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;

Table of Contents

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

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

Additionally, if the results of our 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 or other requirements;
- have regulatory authorities withdraw, vary 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.

Success in preclinical studies or early clinical trials may not be indicative of results obtained in later trials.

Results from preclinical studies or early stage clinical trials are not necessarily predictive of future clinical trial results, and interim results of a clinical trial are not necessarily indicative of final results. Our product candidates and our NAV Technology Licensees' product candidates may fail to show the desired safety and efficacy in clinical development despite positive results in preclinical studies or having successfully advanced through initial clinical trials. The clinical trial process may fail to demonstrate that any of our product candidates or our NAV Technology Licensees' product candidates are safe for humans and effective for indicated uses. This failure may cause us or the relevant NAV Technology Licensee to abandon the relevant product candidate, which could materially and adversely affect our business, financial condition, results of operations and prospects.

There is a high failure rate for drugs and biologic products proceeding through clinical trials. Many companies in the biotechnology and pharmaceutical industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in preclinical testing and earlier-stage clinical trials. Data obtained from preclinical and clinical activities are subject to varying interpretations, which 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. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will generate the same results or otherwise provide adequate data to demonstrate the efficacy and safety of a product candidate. The design of a clinical trial can determine whether its

results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. Our company has limited experience in designing clinical trials and we may be unable to design and execute a clinical trial to support regulatory approval. Any of these factors could materially and adversely affect our business, financial condition, results of operations and prospects.

Table of Contents

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

Additionally, if any of our product candidates receives marketing approval, the FDA could require us to adopt a Risk Evaluation and Mitigation Strategy (REMS) and other regulatory authorities could impose other specific obligations as a condition of approval to ensure that the benefits of our product candidates outweigh their risks, which could delay approval of our product candidates. 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 or patients; 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 the FDA requires us to adopt a REMS for our products and we are unable to comply with its requirements, the FDA may deem our products to be misbranded and we may be subject to civil money penalties. The European Commission, the EMA and other 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.

Table of Contents

We may be unable to obtain orphan drug designation or exclusivity for some product candidates. 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.

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 studies 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 narrower 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.

Table of Contents

Regulatory authorities also may approve a product candidate for more limited indications than requested 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 (the 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. The FDA has articulated 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 companion diagnostic device at the same time that FDA approves the therapeutic product. The FDA's 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. 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, companion diagnostics are subject to the European Union Directive on in vitro diagnostic medical devices and its implementation in the European Union Member States. Recently revised European Union laws on in vitro diagnostics will apply beginning in 2022 and provide stricter requirements for in vitro diagnostic medical devices and impose additional obligations on manufacturers of in vitro diagnostic medical devices that may impact the development and authorization of our product candidates in the European Union. For example, the new regulation extends the requirement for performance assessment procedures and requires greater involvement of notified bodies in the development of in vitro diagnostic medical devices. This may result in additional regulatory and premarket requirements to market new in vitro diagnostic medical devices. Companies producing in vitro diagnostic medical devices will be required to have a responsible person to oversee regulatory compliance. In addition, the new regulation modifies the risk classification of in vitro diagnostic medical devices in a manner that could increase the number of products classified in higher risk classes that are subject to stricter regulation.

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, or obligations imposed as a condition for marketing authorization by other regulatory authorities, 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, in the United States, 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. FDA guidance 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 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 the European Union, the advertising and promotion of our product candidates may be subject to laws governing promotion of medicinal products, interactions with physicians, misleading and comparative advertising and unfair commercial practices. In addition, other legislation adopted by individual European Union Member States may apply to the advertising and promotion of medicinal products. These laws require that promotional materials and advertising in relation to medicinal products comply with the product's Summary of Product Characteristics (SmPC), as approved by the competent authorities. The SmPC is the document that provides information to physicians concerning the safe and effective use of the medicinal product. It forms an intrinsic and integral part of the marketing authorization granted for the medicinal product. Promotion of a medicinal product that does not comply with the SmPC is considered to constitute off-label promotion. The off-label promotion of medicinal products is prohibited in the European Union. The applicable laws also prohibit the direct-to-consumer advertising of prescription-only medicinal products. Violations of the rules governing the promotion of medicinal products in the European Union could be penalized by administrative measures, fines and imprisonment. These laws may further limit or restrict the advertising and promotion of our products to the general public and may also impose limitations on our promotional activities with health care professionals.

Table of Contents

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.

In the European Union, marketing authorization holders and manufacturers of medicinal products are subject to comprehensive regulatory oversight by the EMA and the competent authorities of the individual European Union Member States both before and after grant of the manufacturing and marketing authorizations. This includes control of compliance with cGMP rules. We and our third party manufacturers would be required to ensure that all of our processes, methods, and equipment are compliant with cGMP. Failure by us or by any of our third party partners, including suppliers, manufacturers and distributors, to comply with European Union laws and the related laws of individual European Union Member States governing the conduct of clinical trials, manufacturing approval, marketing authorization of medicinal products and marketing of such products following authorization may result in administrative, civil or criminal penalties.

In addition, European Union legislation related to pharmacovigilance, or the assessment and monitoring of the safety of medicinal products, provides that the EMA and the competent authorities of the European Union Member States have the authority to require companies to conduct additional post-approval clinical efficacy and safety studies. The legislation also governs the obligations of marketing authorization holders with respect to additional monitoring, adverse event management and reporting. Under the pharmacovigilance legislation and its related regulations and guidelines, we may be required to conduct ongoing assessments of the risks and benefits of marketed products, including the possible requirement to conduct additional clinical studies, which may be time-consuming and expensive and could materially decrease our profitability.

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, vary 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.

Table of Contents

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

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

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

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

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

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

Approval of a product candidate in the United States by the FDA does not ensure approval of such product candidate by regulatory authorities in other countries or jurisdictions, and approval by one foreign regulatory authority does not ensure approval by regulatory authorities in other foreign countries or by the FDA. Sales of our product candidates outside of the United States will be subject to foreign regulatory requirements governing clinical trials and marketing approval. Even if the FDA grants marketing approval for a product candidate, comparable regulatory authorities of foreign countries also must approve the manufacturing and marketing of the product candidates in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and more onerous than, those in the United States, including additional preclinical studies or clinical trials. In many countries outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that country. In some cases, the price that we intend to charge for our products, if approved, is also subject to approval. We intend to submit a marketing authorization application to EMA for approval of our product candidates by the European Commission in the European Union. However, obtaining such approval

from the European Commission following the opinion of EMA is a lengthy and expensive process. Even if a product candidate is approved, the FDA or the European Commission, as the case may be, may limit the indications for which the product may be marketed, require extensive warnings on the product labeling or require expensive and time-consuming additional clinical trials or reporting as conditions of approval. Regulatory authorities in countries outside of the United States and the European Union also have requirements for approval of product candidates with which we must comply prior to marketing in those countries. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our product candidates in certain countries.

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

Table of Contents

Risks Related to Our Financial Position

We have incurred cumulative net losses and have had few profitable quarters since inception. We expect to normally incur losses for the foreseeable future and may never again achieve or maintain profitability.

Since inception, we have incurred cumulative net losses. We historically have financed our operations primarily through private and public offerings of our equity securities 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 clinical development of our product candidates, as well as to building out our team. We expect that it could be several years, if ever, before we commercialize a 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 normally generate only limited revenue, if any, from our current NAV Technology Licensees and any future NAV Technology Licensees in the near term. We expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. The net losses we incur may fluctuate significantly from quarter to quarter. We anticipate that our expenses will increase substantially if, and as, we:

- further develop our sublicensing activities and NAV Technology Platform;
- continue our research studies and preclinical and clinical development of our 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 additional activities relating to manufacturing, including building out additional laboratory and manufacturing capacity;
- 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;
- expand our medical affairs efforts;
- 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 product candidates, commencing and completing clinical trials of 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 may 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 expect to require substantial future capital in order to complete research studies, preclinical and clinical development for our current product candidates and any future product candidates, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical and clinical trials of our 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, 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.

Table of Contents

Our operations have consumed significant amounts of cash since inception. We expect that our cash, cash equivalents and marketable securities as of the end of the period to which this filing relates will enable us to fund our operating expenses and capital expenditure requirements for at least the next 12 months from the date of this report, based on our current business plan.

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

- the timing of enrollment, commencement and completion of our clinical trials;
- the results of our clinical trials;
- the results of our preclinical studies for our product candidates and any subsequent clinical trials;
- our planned expansion of the licensing of our NAV Technology Platform;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials for our product candidates;
- the costs associated with building out additional laboratory and manufacturing capacity, if any;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future product sales, medical affairs, marketing, manufacturing and distribution activities 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 or collaborations 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, and no products have been commercialized by us or our NAV Technology Licensees using our NAV Technology Platform to date. 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. 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.

Table of Contents

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

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

Our ability to generate revenue from product candidate sales depends on our ability, alone or with partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our product candidates. All of our revenues to date have been from sublicensing our NAV Technology Platform, the sale of licensed reagents to third-parties for use in research and development and grant revenue generated through research and development grant programs offered by the U.S. federal government and the European Union. We expect grant revenue to be minimal in future periods, as we currently do not expect to receive any new grant awards. We do not dedicate 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 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 regulatory and marketing approval is obtained 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 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 product candidates, if approved;
- obtaining market acceptance of product candidates as a viable treatment option;

Table of Contents

- 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.

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.

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

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

Changes in accounting standards and disagreements and differing views by the SEC, the Financial Accounting Standards Board (FASB) or various other bodies with respect to the interpretations, estimates and judgments required for the preparation of our financial statements could result in the restatement of our financial statements or other potential adverse effects.

We are subject to complex tax laws, regulations, accounting principles and interpretations thereof. The preparation of our financial statements requires us to interpret accounting principles and guidance and make estimates and judgments that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported revenue generated and expenses incurred during the reporting periods. Our interpretations, estimates and judgments are based on our historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for the preparation of our financial statements. U.S. generally accepted accounting principles are subject to interpretation by the SEC,

FASB and various other bodies formed to interpret and create appropriate accounting principles and guidance. In the event that these rules change with respect to a matter that is or may become relevant to our business, such as revenue recognition, asset impairment and fair value determinations, inventories, business combinations and intangible asset valuations, leases and litigation, or in the event that one of these bodies disagrees with our accounting recognition, measurement or disclosure or any of our accounting interpretations, estimates or assumptions, it may have a significant effect on our reported results and may retroactively affect previously reported results. The need to restate our financial results could, among other potential adverse effects, result in us incurring substantial costs, affect our ability to timely file our periodic reports until such restatement is completed, divert the attention of our management and employees from managing our business, result in material changes to our historical and future financial results, result in investors losing confidence in our operating results, subject us to securities class action litigation, and cause our stock price to decline.

Table of Contents

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 (Section 404) requires that we evaluate and determine the effectiveness of our internal control over financial reporting and provide a management report on internal control over financial reporting. When we are no longer an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012 (the JOBS Act), our management report on internal control over financial reporting will need to be attested to by our independent registered public accounting firm. We have not had, and 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. Had our independent registered public accounting firm performed an evaluation of the effectiveness of our internal control over financial reporting in accordance with Section 404, it is possible that material weaknesses may have been identified.

If we have, or fail to identify, a material weakness in our internal control over financial reporting, we may not detect errors on a timely basis, the accuracy and timing of our financial reporting may be adversely affected and our financial statements may be materially misstated. In addition, our internal control over financial reporting will not prevent or detect all errors and fraud. Because of the inherent limitations in all control systems, no evaluation of controls can provide absolute assurance that misstatements due to error or fraud will not occur or that all control issues and instances of fraud will be detected.

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

Changes in U.S. federal, state and local or foreign tax laws, interpretations of existing tax laws, or adverse determinations by tax authorities, could increase our tax burden or otherwise adversely affect our financial condition or results of operations.

We are subject to taxation at the U.S. federal, state and local levels and in foreign jurisdictions. Our future tax rates and cash flows could be affected by changes in statutory rates and other legislative changes, changes in the valuation of our deferred tax assets and liabilities, changes in the composition of earnings in jurisdictions with differing tax rates, changes in determinations regarding the jurisdictions in which we are subject to taxation, and our ability to repatriate earnings from foreign jurisdictions. From time to time, governments may make substantive changes to their tax rules and the application thereof, which could result in materially higher corporate taxes than would be incurred under existing tax laws and could otherwise adversely affect our financial condition or results of operations.

We are subject to ongoing and periodic tax audits. An unfavorable outcome from any tax audit could result in higher tax costs, penalties or interest, or adjustments to our tax credits or net operating losses (NOLs), which could adversely affect our financial condition or results of operations.

We have incurred substantial net losses since inception and expect to normally incur losses for the foreseeable future. Under the Internal Revenue Code of 1986, as amended (the Code), we can carry forward our NOLs and other unused tax attributes, such as tax credits, to offset our future taxable income, if any, until such NOLs or other tax attributes are used or expire. If we undergo an “ownership change,” generally defined as a greater than 50% change by value in

our equity ownership over a three-year period, the Code would limit our ability to use carryovers of our pre-ownership change NOLs, tax credits and certain other tax attributes to reduce our tax liability for periods after the ownership change. Therefore, an ownership change could result in increased U.S. tax liability for us if we generate taxable income in a future period.

In December 2017, the Tax Cuts and Jobs Act of 2017 (the TCJA) was signed into law, which significantly reforms the Code. The TCJA, among other things, contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for NOLs to 80% of current year taxable income, elimination of NOL carrybacks, one-time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain significant exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modification or repeal of many business deductions and credits, including the orphan drug tax credit. The overall impact of the TCJA is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the TCJA. The impact of the TCJA on our securityholders is also uncertain and could be adverse. Prospective investors should consult with their legal and tax advisors with respect to the TCJA and the potential tax consequences of investing in or holding our securities.

Table of Contents

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., could 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 AAV vectors discovered at Penn in the laboratory of James M. Wilson. This license agreement has been amended from time to time. In February 2009, we also entered into a sponsored research agreement (the 2009 SRA with Penn, under which we funded the nonclinical research of Dr. Wilson relating to AAV gene therapy and obtained an option to acquire an exclusive worldwide license in certain intellectual property created pursuant to the 2009 SRA. In December 2014, we entered into another sponsored research agreement (the 2014 SRA) with Penn, under which we fund related nonclinical research of Dr. Wilson. The 2009 SRA and the 2014 SRA have each been amended from time to time.

Under the 2014 SRA, as amended, 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 U.S. 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. Under our 2014 SRA with Penn, as amended, we have agreed to fund research at Penn through 2020. Although we are currently developing our internal nonclinical research and development capabilities, a loss of our relationship with Penn or Dr. Wilson could materially harm our business.

We rely on third parties to conduct certain aspects of our clinical trials. If these third parties do not meet our deadlines or otherwise conduct the trials as required, our clinical development programs could be delayed or unsuccessful and we may not be able to obtain regulatory approval for or commercialize our product candidates when expected or at all.

We do not have the ability to conduct all aspects of our preclinical testing or clinical trials ourselves. We are dependent on third parties to conduct the clinical trials for RGX-501 and certain aspects of our clinical trials for other product candidates and, therefore, the timing of the initiation and completion of these trials may be controlled by such third parties and may occur on substantially different timing from our estimates. Specifically, we rely on Penn to conduct our Phase I/II clinical trial for RGX-501 and we may also rely on CROs, medical institutions, clinical investigators, consultants or other third parties to conduct our trials in accordance with our clinical protocols and regulatory requirements.

There is no guarantee that Penn or any other third party on which we rely for administration and conduct of our clinical trials will devote adequate time and resources to such trials or perform as contractually required. If any such third party fails to meet expected deadlines, fails to adhere to our clinical protocols or otherwise performs in a substandard manner, our clinical trials may be extended, delayed, or terminated, which could materially harm our business. Additionally, if any of our clinical trial sites terminates for any reason, we may experience the loss of follow-up information on subjects enrolled in our ongoing clinical trials unless we are able to transfer those subjects to another qualified clinical trial site. Furthermore, principal investigators for our clinical trials may serve as scientific advisors or consultants to us from time to time and may receive cash or equity compensation in connection with such services. If these relationships and any related compensation result in perceived or actual conflicts of interest, the integrity of the data generated at the applicable clinical trial site may be jeopardized, which could result in substantial delays in our clinical trials and materially harm our business.

We have in the past, and in the future may, enter into licensing agreements or collaborations with third parties licensing parts of our NAV Technology Platform for the development of product candidates. If these licensing arrangements or collaborations 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 current and future licensees and collaborators, 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 licensees' and collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our licensees and collaborators have the ability to abandon research or development projects and terminate applicable agreements. Moreover, an unsuccessful outcome in any clinical trial for which our licensee or collaborator is responsible could be harmful to the public perception and prospects of our NAV Technology Platform or product candidates.

Table of Contents

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 suffer adverse effects, including death;
- licensees or collaborators may not pursue development and commercialization of any product candidates that achieve regulatory approval or may elect not to continue or renew development or commercialization programs based on clinical trial results, changes in the licensees' or collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- licensees or collaborators may delay clinical trials, provide insufficient funding for clinical trials, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- we may not have access to, or may be restricted from disclosing, certain information regarding product candidates being developed or commercialized under a collaboration and, consequently, may have limited ability to inform our stockholders about the status of such product candidates;
- licensees or collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the licensees or collaborators believe that competitive products are more likely to be successfully developed or can be commercialized under terms that are more economically attractive than ours;
- product candidates developed in collaboration with us may be viewed by our licensees or collaborators as competitive with their own product candidates or products, which may cause licensees or collaborators to cease to devote resources to the commercialization of our product candidates;
- a licensee or collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- licensees or collaborators may breach their reporting, payment, intellectual property or other obligations to us, which could prevent us from complying with our contractual obligations to GSK and Penn;
- disagreements with licensees or 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 license agreement or 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 licensees or collaborators terminates its agreement with us, we may find it more difficult to attract new licensees or collaborators and the perception of us in the business and financial communities could be harmed. Each of our licensees and collaborators is subject to similar risks with respect to product development, regulatory approval and commercialization, and any such risk could result in its business being harmed, which could adversely affect our collaboration.

Table of Contents

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.

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

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

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

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

Because we 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 collaborate with, or may collaborate with in the future, will sometimes 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. 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.

Table of Contents

Risks Related to Manufacturing

Products intended for use in 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 development, manufacturing and testing agreements with third parties to manufacture supplies of our product candidates, in addition to our internal manufacturing laboratory. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers.

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, EMA or other applicable foreign standards or specifications with consistent and acceptable production yields and costs.

In addition, the FDA, the EMA and other 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 foreign regulatory authorities may require that we not distribute a lot until the competent authority authorizes its release. Slight deviations in the manufacturing process, including those affecting quality attributes and stability, may result in unacceptable changes in the product that could result in lot failures or product recalls. Lot failures or product recalls could cause us to delay clinical trials or product launches which could be costly to us and otherwise harm our business, financial condition, results of operations and prospects.

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

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

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, which includes a review of the manufacturing process and facility. A

manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities and may be required by other foreign regulatory authorities. The timeframe required to obtain such approval or authorization is uncertain. 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, contract laboratories or suppliers. 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.

Table of Contents

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 most of the 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.

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, EMA or other regulatory authority action, including injunction, recall, seizure or total or partial suspension of product manufacture or manufacturing authorization.

Failure to comply with ongoing manufacturing regulatory requirements could cause us to suspend production or put in place costly or time-consuming remedial measures.

Regulatory authorities may, at any time following approval of a product for sale, audit the manufacturing facilities for such product. If any such inspection or audit identifies a failure to comply with applicable regulations, or if a violation of product specifications or applicable regulations occurs independent of such an inspection or audit, the relevant regulatory authority may require remedial measures that may be costly or time-consuming to implement and that may include the temporary or permanent suspension of a clinical trial or commercial sales or the temporary or permanent closure of a manufacturing facility. Any such remedial measures imposed upon us or any of our third-party manufacturers could materially harm our business, financial condition, results of operations and prospects.

If we or any of our third party-manufacturers fail to comply with applicable cGMP regulations, regulatory authorities can impose regulatory sanctions including, among other things, refusal to approve a pending application for a new product candidate or suspension or revocation of a pre-existing approval. Such an occurrence may cause our business, financial condition, results of operations and prospects to be materially harmed.

Additionally, if supply from a manufacturing facility is interrupted, there could be a significant disruption in commercial supply of our products. An alternative manufacturer would need to be qualified, through a supplement to its regulatory filing, which could result in further delay. Regulatory authorities also may require additional trials if a new manufacturer is relied upon for commercial production. Switching manufacturers may involve substantial costs and could result in a delay in our desired clinical and commercial timelines.

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 and 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 and other components required in our manufacturing process are derived from biologic sources, and we normally rely on suppliers to provide raw materials and other components. 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 may be beyond our control and 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.

Table of Contents

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.

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 only a few gene therapy products approved to date in the United States, the European Union or elsewhere. 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 related to clinical trials we conduct, clinical trials involving our NAV Technology Platform conducted by others or any gene therapy products, even if such adverse events are not ultimately attributable to the relevant product candidates or products, may 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.

Table of Contents

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, the EMA and other regulatory 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 the conditions our lead product candidates are intended to treat, 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.

Table of Contents

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 prices 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. 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.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products, including potential one-time gene therapies. In the United States, third-party payors, including government payors such as the Medicare and Medicaid programs, play an important role in determining the extent to which new drugs and biologics will be covered and reimbursed. The Medicare and Medicaid programs increasingly are used as models for how private payors and government payors develop their coverage and reimbursement policies. It is difficult to predict what the Centers for Medicare & Medicaid Services (CMS), the agency responsible for administering the Medicare program, will decide with respect to coverage and reimbursement for fundamentally novel products such as ours, as there is no body of established practices and precedents for these types of products. We cannot be assured that Medicare or Medicaid will cover any of our products, if approved, or provide reimbursement at adequate levels to realize a sufficient return on our investment. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates.

Outside the United States, international operations generally are subject to extensive government price controls and other market regulations, and increasing emphasis on cost-containment initiatives in the European Union and other countries may put pricing pressure on us. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. It also can take a significant amount of time after approval of a product to secure pricing and reimbursement for such product in many counties outside the United States. In general, the prices of medicines under such systems are substantially lower than in the United States. Other countries allow companies to fix their own prices for medical products, but monitor and control company profits. Additional foreign price controls or other changes in pricing regulation could restrict the amount that we are able to charge for our product candidates. Accordingly, in markets outside the United States, the reimbursement for our

products may be reduced compared with the reimbursement in the United States and may be insufficient to generate commercially reasonable product revenues.

Moreover, increasing efforts by government and third-party payors in the United States and abroad to cap or reduce healthcare costs may cause such organizations to limit both coverage and the level of reimbursement for new products approved and, as a result, they may not cover or provide adequate payment for our product candidates. Payors increasingly are considering new metrics as the basis for reimbursement rates, and the existing data for reimbursement based on some of these metrics is limited. Therefore, it may be difficult to project the impact of these evolving reimbursement metrics on the willingness of payors to cover candidate products that we or our partners are able to commercialize. We expect to experience pricing pressures in connection with the sale of any of our product candidates due to the trend toward managed healthcare, the increasing influence of health maintenance organizations and additional legislative changes.

Table of Contents

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 lead 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. 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, any of which could materially harm our business, which could include:

- 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.

Government price controls or other changes in pricing regulation could restrict the amount that we are able to charge for any of our product candidates, if approved, which would adversely affect our revenue and results of operations.

We expect that coverage and reimbursement of drugs and biologics may be increasingly restricted in the United States and internationally. The escalating cost of health care has led to increased pressure on the health care industry to reduce costs. In particular, pricing by biopharmaceutical companies recently has come under increased scrutiny and continues to be subject to intense political and public debate in the United States and abroad. Government and private third-party payors have proposed health care reforms and cost reductions of drugs and biologics. A number of federal and state proposals to control the cost of health care have been made in the United States. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state bills designed to, among other things, bring more transparency to pricing, review the relationship between pricing and manufacturer patient programs and reform government program reimbursement methodologies. In some international markets, the government controls drug and biologic pricing, which can affect profitability.

We cannot predict the extent to which our business may be affected by these or other potential future legislative or regulatory developments. However, future price controls or other changes in pricing regulation or negative publicity related to the pricing of drugs and biologics generally could restrict the amount that we are able to charge for our future products, if any, which could adversely affect our revenue and results of operations.

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.

Table of Contents

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, 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 is, and will continue to 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 any of our key executives, employees, consultants or advisors may impede the progress of our research, development, licensing and commercialization objectives and materially harm our business, financial condition, results of operations and prospects.

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

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

development and growth goals.

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

We are exposed to the risk of fraud or other misconduct by our employees, principal investigators, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with FDA regulations or the regulations applicable in the European Union and other jurisdictions, provide accurate information to the FDA, the European Commission and other regulatory authorities, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct also could involve the improper use of information obtained in the course of clinical trials or interactions with the FDA or other regulatory authorities, which could result in regulatory sanctions and cause serious harm to our reputation. We have adopted a code of business 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.

Table of Contents

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

In the United States and some foreign jurisdictions, there have been, and continue to be, several legislative and regulatory initiatives regarding the healthcare system that could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities or affect our ability to profitably sell any product candidates for which we obtain marketing approval. 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.

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, or the rules adopted under PPACA, is unknown at this time. Furthermore, PPACA may be modified, repealed or replaced with new regulations, and the full impact of any such modification, repeal or replacement is unknown at this time. For example, the TCJA repeals the individual mandate of PPACA beginning in 2019, which may reduce the number of individuals covered by health benefits. We cannot predict the ultimate content, timing or effect of any potential PPACA modification, repeal or replacement or any other healthcare reform legislation, or the effect of such potential changes on our business.

Additional changes that may affect our business include those governing enrollment in federal healthcare programs, reimbursement changes, rules regarding prescription drug benefits under the health insurance exchanges, rules regarding fraud and abuse, and enforcement. Continued implementation of PPACA, or the repeal or replacement of PPACA, and the passage of additional laws and regulations may result in the expansion of new programs such as Medicare payment for performance initiatives, and may impact existing government healthcare programs, such as by improving the physician quality reporting system and feedback program.

Other legislative changes have been proposed and adopted in the United States since PPACA was enacted. For example, the Budget Control Act of 2011, 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, including Medicare payments to healthcare providers of up to 2.0% per fiscal year, starting in 2013. In January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several categories of healthcare providers and increased the statute of limitations period for the government to recover overpayments to providers

from three to five years. 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 result in reduced demand for our product candidates or additional pricing pressures and thereby adversely affect our business, financial condition and results of operations.

Various states, such as California, have also taken steps to consider and enact laws or regulations that are intended to increase the visibility of the pricing of biopharmaceutical products with the goal of reducing the prices at which such products can be sold. Because these various actual and proposed legislative changes are intended to operate on a state-by-state level rather than a national one, we cannot predict what the full effect of these legislative activities may be on our business in the future.

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.

Table of Contents

The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than European Union, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most European Union Member States have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing European Union and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize any products for which we obtain marketing approval. Furthermore, healthcare legislative reform measures in countries outside the United States and the European Union may materially delay or restrict our business activities or otherwise materially harm our business.

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.

In the United States, the research, manufacturing, distribution, sale and promotion of drugs and biologics are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including CMS, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S. Department of Justice offices of the U.S. Attorney, and state and local governments.

If we obtain the approval of the FDA, the European Commission or other regulatory authorities 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, and similar laws in foreign jurisdictions. 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. Liability may be established under the federal Anti-Kickback Statute without proving actual knowledge of the 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. PPACA provides and recent government cases against pharmaceutical and medical device manufacturers support the view that Federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may implicate the False Claims Act;
- the federal Health Insurance Portability and Accountability Act of 1996 (HIPAA), which created new federal criminal statutes that prohibit a person from knowingly and willfully executing a scheme or from making false or fraudulent statements to defraud any healthcare benefit program, regardless of the payor (e.g., public or private);

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act (HITECH), and its implementing regulations, and as amended again by the final HIPAA omnibus rule, Modifications to the HIPAA Privacy, Security, Enforcement, and Breach Notification Rules Under HITECH and the Genetic Information Nondiscrimination Act;
- Other Modifications to HIPAA, published in January 2013, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization by entities subject to the rule, such as health plans, health care clearinghouses and health care providers;
- federal transparency laws, including the federal Physician Payment Sunshine Act, that require disclosure of payments and other transfers of value provided to physicians and teaching hospitals, and ownership and investment interests held by physicians and other healthcare providers and their immediate family members and applicable group purchasing organizations; and

Table of Contents

- 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, presently governed by the provisions of the Data Protection Directive, will be replaced with the General Data Protection Regulation (GDPR). GDPR will impose several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, the security and confidentiality of the personal data, data breach notification and using third party processors in connection with the processing of the personal data. GDPR will also impose strict rules on the transfer of personal data out of the European Union to the United States. Failure to comply with the requirements of GDPR and the applicable national data protection laws of the European Union Member States may result in fines and other administrative penalties. GDPR will introduce substantial fines for breaches of the data protection rules. GDPR was adopted in April 2016 and is expected to become enforceable in 2018. Once it is enforceable, GDPR may increase our responsibility and liability in relation to personal data that we process. To comply with the new data protection rules imposed by GDPR we may be required to put in place additional mechanisms ensuring compliance. This may be onerous and adversely affect 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.

Table of Contents

Although we maintain product liability insurance coverage, this insurance may not be adequate to cover all liabilities that we may incur. We anticipate that we will evaluate the need to increase our insurance coverage each time we commence a clinical trial and may from time to time purchase additional coverage for clinical trials. We may need to increase our product liability insurance coverage if we successfully commercialize any product candidates. Insurance coverage is increasingly expensive and 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, our development partners, including our NAV Technology Licensees, or our third-party manufacturers or suppliers 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, our development partners, including our NAV Technology Licensees, and our third-party manufacturers and suppliers 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 operations and the operations of our development partners and third-party manufacturers and suppliers 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 the use of hazardous materials by us, our development partners or our third-party manufacturers or suppliers, 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 for certain costs and expenses we may incur due to work-related injuries to our employees, this insurance may not provide adequate coverage against potential liabilities. Although we maintain insurance for claims that may be asserted against us in connection with our storage or disposal of biologic, hazardous or radioactive materials, this insurance may not be adequate to cover all liabilities that we may incur in connection with such claims.

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.

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. A severe or prolonged economic downturn 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. For example, the global financial crisis of 2007-2008 and the ongoing European economic crisis caused extreme volatility and disruptions in the capital and credit markets. 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.

Additionally, in June 2016, a majority of United Kingdom (UK) voters voted for the UK to exit the European Union (Brexit) and in March 2017, the UK government provided official legal notification to the European Union that the UK will exit the European Union. The timing and completion of Brexit is subject to judicial and parliamentary developments in the UK, as well as any legal challenges. The economic effects of Brexit will depend on any agreements the UK makes to retain access to European Union markets either during a transitional period or more permanently. Brexit could adversely affect European and worldwide economic or market conditions and could contribute to instability in global financial markets. Brexit is likely to lead to legal uncertainty and potentially divergent national laws and regulations as the UK determines which European Union laws to replace or replicate. Any of these effects of Brexit, and any other effects we cannot anticipate, could adversely affect our business, business opportunities, results of operations, financial condition and cash flows.

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 or supply 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

Table of Contents

in the event of a serious disaster or similar event. Our third party manufacturing and supply facilities, as well as substantially all of our current supply of product candidates, are located in a small number of geographic locations, and should a natural disaster, power outage or other event occur that affects one of our third party manufacturing or supply facilities, manufacturing or supply delays may result should we need to transfer manufacturing or supply operations to another facility. 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 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.

We are increasingly dependent on information technology systems, infrastructure and data.

We are increasingly dependent upon information technology systems, infrastructure and data. Our computer systems may be vulnerable to service interruption or destruction, malicious intrusion and random attack. Security breaches pose a risk that sensitive data, including intellectual property, trade secrets or personal information may be exposed to unauthorized persons or to the public. Cyber-attacks are increasing in their frequency, sophistication and intensity, and have become increasingly difficult to detect. Cyber-attacks could include the deployment of harmful malware, denial-of service, social engineering and other means to affect service reliability and threaten data confidentiality, integrity and availability. Our key business partners face similar risks, and a security breach of their systems could adversely affect our security posture. While we continue to invest in data protection and information technology, there can be no assurance that our efforts will prevent service interruptions, or identify breaches in our systems, that could adversely affect our business and operations and/or result in the loss of critical or sensitive information, which could result in financial, legal, business or reputational harm.

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 three months ended March 31, 2018 and the year ended December 31, 2017 consisted primarily of license revenue. One customer accounted for approximately 99% of our total revenue for the three months ended March 31, 2018. This customer also accounted for approximately 93% of our accounts receivable, current and non-current, as of March 31, 2018. One customer accounted for approximately 68% of our total revenue for the year ended December 31, 2017. No other customer accounted for more than 10% of revenue for the year ended December 31, 2017. Future license revenue is uncertain due to the contingent nature of our licenses granted to third-parties.

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 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.

Table of Contents

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

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. 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 GSK and Penn, each of which is important to our business, and other entities 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 or our licensees 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.

Table of Contents

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 avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (the Hatch-Waxman Amendments) to conduct research and clinical trials and may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our patents may be challenged in the courts or patent offices in the United States and abroad. For example, in June 2017, a third party filed an opposition with the European Patent Office challenging the validity of a European patent owned by Penn for the AAV8 vector, which we have exclusively licensed. 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;

Table of Contents

- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- the priority of invention of patented technology.

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

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

We currently have rights to intellectual property, through licenses from third parties, to 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 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 may 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 U.S. Patent and Trademark Office (the 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. 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 our license agreements with Penn and GSK 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 Minnesota, our rights are limited to those countries and territories, including the United States, in which a licensed patent has been 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

Table of Contents

made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

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

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

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

In addition to the protection afforded by patents, we rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable or that we elect not to patent, processes for which patents are difficult to enforce and any other elements of our technology, product candidate discovery and development processes that involve proprietary know-how, information or technology that is not covered by patents. However, trade secrets can be difficult to protect and some courts inside and outside the United States are less willing or unwilling to protect trade secrets. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We cannot guarantee that we have entered into such agreements with each party that may have or have had access to our trade secrets or proprietary

technology and processes. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors.

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

Our commercial success depends, in part, upon our ability to license our NAV Technology Platform, and on our NAV Technology Licensees' ability to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing or otherwise violating the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and inter

Table of Contents

partes review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could materially harm our ability to license our technology platform or commercialize our lead product candidates or any future product candidates or technologies covered by the asserted third-party patents. In order to successfully challenge the validity of any such U.S. patent in federal court, we would need to overcome a presumption of validity. As this burden is a high one requiring us to present clear and convincing evidence as to the invalidity of any such U.S. patent claim, there is no assurance that a court of competent jurisdiction would invalidate the claims of any such U.S. patent. If we are found to infringe a third party's valid and enforceable intellectual property rights, we could be required to obtain a license from such third party to continue licensing, developing, manufacturing and marketing our product candidates and technology. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors and other third parties access to the same technologies licensed to us, and it could require us to make substantial licensing and royalty payments. We could be forced, including by court order, to cease licensing, developing, manufacturing and commercializing the infringing technology or product candidates. In addition, we could be found liable for monetary damages, including treble damages and attorneys' fees, if we are found to have willfully infringed a patent or other intellectual property right. A finding of infringement could prevent us from licensing our technology platform or manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could similarly harm our business, financial condition, results of operations and prospects.

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

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

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

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

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

Table of Contents

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

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

The patent positions of companies engaged in the development and commercialization of biologics and pharmaceuticals are particularly uncertain. Two cases involving diagnostic method claims and "gene patents" have recently been decided by the Supreme Court of the United States (the 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 has issued a number of guidance memoranda 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. The USPTO's guidance may 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.

Table of Contents

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

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

We have registered trademarks with the USPTO for the marks “NAV” and “REGENXBIO,” as well as for the REGENXBIO logos. 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 current or future license partners or 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 current or future license partners or 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.

Table of Contents

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

Risks Related to Ownership of Our Common Stock

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for holders of our common stock.

Our stock price is likely to be volatile. In recent years, the stock market in general, and the market for biotechnology or pharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of our common stock at or above the price they paid for their shares. The market price of our common stock could be subject to wide fluctuations in response to various factors, many of which are beyond our control. These factors include those discussed elsewhere in this "Risk Factors" section and others such as:

- the delay or failure in initiating or completing preclinical studies or clinical trials, or unsatisfactory results of these trials;
- announcements about us or about our competitors including clinical trial results, regulatory approvals, or new product candidate introductions;
- developments concerning our current or future development partners, licensors or product candidate manufacturers;
- developments or changing views regarding the use of gene therapy;
- litigation and other developments relating to our patents or other proprietary rights or those of our competitors;
- conditions in the pharmaceutical or biotechnology industries and the economy as a whole;
- governmental regulation and legislation;
- the recruitment or departure of members of our board of directors, management team or other key personnel;
- 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 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 cause us to incur substantial costs to defend such claims and divert our management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

Our quarterly operating results may fluctuate substantially, which may cause the price of our common stock to fluctuate substantially.

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

- any variations in the level of expenses related to our NAV Technology Platform and lead product candidates;

- the addition or termination of any clinical trials;
- any regulatory or clinical developments affecting our lead product candidates, any future product candidates or our NAV Technology Licensees' product candidates;
- our execution of any collaborative, licensing or similar arrangements, including with our NAV Technology Licensees, and the timing of any payments we may make or receive under these arrangements; and

Table of Contents

- the nature and terms of any stock-based compensation grants and any intellectual property infringement lawsuits 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. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

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 additional analysts do not commence coverage of us, the trading price of our stock may decrease. Additionally, 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.

Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish proprietary rights.

We may seek to raise additional capital through public or private equity offerings, debt financings, strategic partnerships, licensing arrangements or other means. We have an effective shelf registration statement on file with the SEC, which could allow us to access capital in a timely manner. 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 or 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 have broad discretion in the use of our cash and cash equivalents and may not use them effectively.

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.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be our stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock, and we currently intend to retain all of our future earnings, if any, to finance the development and growth of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. Therefore, our stockholders are not likely to receive any dividends on our common stock for the foreseeable future or at all and their ability to receive a return on their 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 our stockholders have purchased it.

Our executive officers, directors and principal stockholders own a significant percentage of our stock and maintain the ability to exert substantial influence over matters subject to stockholder approval.

Our executive officers, directors, holders of more than five percent of our capital stock and their respective affiliates beneficially own a significant percentage of our outstanding capital stock. As a result, these stockholders may be able to exert substantial influence over all matters submitted to our stockholders for approval, as well as our management and affairs. 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 concentration of voting power could delay or prevent an acquisition of our company on terms that other stockholders may desire or result in management of our company with which our public stockholders disagree.

Table of Contents

Substantial future sales of shares by existing stockholders, including pursuant to our equity incentive plans, or the perception that such sales may occur, could cause our stock price to decline, even if our business is performing well.

If our existing stockholders, particularly our directors and executive officers and the entities 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.

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 and Rule 144 and Rule 701 under the Securities Act of 1933, as amended. Additionally, some of our existing stockholders have demand and piggyback rights to require us to register with the SEC up to a certain number of shares of our common stock. 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 registered 5,057,458 shares of common stock held by certain of our stockholders in a registration statement on Form S-3 filed with the SEC on December 16, 2016 and declared effective as of January 6, 2017. Such stockholders are able to freely trade such shares of common stock.

Furthermore, certain of our employees, directors, officers or affiliates have entered into Rule 10b5 1 plans providing for transactions of our securities from time to time. Under a Rule 10b5 1 plan, a broker executes trades pursuant to parameters established by the securityholder when entering into the plan, without further direction from the securityholder. Accordingly, sales under these plans may occur at any time, including possibly before, simultaneously with, or immediately after significant events involving us. A Rule 10b5 1 plan may be amended or terminated in some circumstances. If any additional shares of our 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. We do not undertake to report the entry into, or the amendment or termination of, any Rule 10b5 1 plans adopted by our employees, directors, officers or affiliates in the future, except to the extent required by law.

An active trading market for our common stock may not be sustained.

Shares of our common stock began trading on The Nasdaq Global Select Market on September 17, 2015. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares may not continue to develop or be sustained. If an active market for our common stock does not continue to develop or is not sustained, it may be difficult for our stockholders to sell shares without depressing the market price for the shares, or at all.

If we engage in future acquisitions or strategic partnerships, this may increase our capital requirements, dilute our stockholders, cause us to incur debt or assume contingent liabilities and subject us to other risks.

We may evaluate various acquisitions and strategic partnerships, including licensing or acquiring complementary products, intellectual property rights, technologies, or businesses. Any potential acquisition or strategic partnership may entail numerous risks, including:

- increased operating expenses and cash requirements;
- the assumption of additional indebtedness or contingent liabilities;
- assimilation of operations, intellectual property and products of an acquired company, including difficulties associated with integrating new personnel;

- the diversion of our management's attention from our existing product programs and initiatives in pursuing such a strategic merger or acquisition;
- retention of key employees, the loss of key personnel, and uncertainties in our ability to maintain key business relationships;
- risks and uncertainties associated with the other party to such a transaction, including the prospects of that party and their existing products or product candidates and regulatory approvals; and
- our inability to generate revenue from acquired technology and/or products sufficient to meet our objectives in undertaking the acquisition or even to offset the associated acquisition and maintenance costs.

In addition, if we undertake acquisitions, we may issue dilutive securities, assume or incur debt obligations, incur large one-time expenses and acquire intangible assets that could result in significant future amortization expense. Moreover, we may not be able to locate suitable acquisition opportunities and this inability could impair our ability to grow or obtain access to technology or products that may be important to the development of our business.

Table of Contents

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

As a public company, we incur, and particularly after we are no longer an emerging growth company, we will incur further, 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 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, these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance as a public company.

Pursuant to Section 404, we are 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. In an effort to achieve compliance with Section 404 within the prescribed period, we have incurred costs associated with the documentation and evaluation of our internal control over financial reporting. In this regard, we have dedicated internal resources, engaged outside consultants and adopted a detailed work plan to assess and document the adequacy of internal control over financial reporting. We will need to continue to improve our 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. This continuous process is both costly and challenging and 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 under 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. Among other things, these provisions:

- establish a classified board of directors so that not all members of our board are elected at one time;
- permit the board of directors to establish the number of directors;
- provide that directors may only be removed “for cause”;
- require super-majority voting to amend some provisions in our restated certificate of incorporation and amended and restated bylaws;
- authorize the issuance of “blank check” preferred stock that our board of directors could use to implement a stockholder rights plan;
- eliminate the ability of our stockholders to call special meetings of stockholders;
- prohibit stockholder action by written consent, which requires all stockholder actions to be taken at a meeting of our stockholders;

- provide that the board of directors is expressly authorized to make, alter or repeal our bylaws; and
- establish advance notice requirements for nominations for election to our board of directors or for proposing matters that can be acted upon by stockholders at annual stockholder meetings.

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

Table of Contents

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 or our directors, officers or employees.

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. Alternatively, if a court were to find the choice of forum provision contained in our restated certificate of incorporation to be inapplicable or unenforceable in an action, we may incur additional costs associated with resolving such action in other jurisdictions, which could adversely affect our business and financial condition.

Our business could be negatively affected as a result of the actions of activist stockholders.

Proxy contests have been waged against many companies in the biopharmaceutical industry over the last several years, and proxy advisory firms may recommend changes to our business operations, provisions in our restated certificate of incorporation or amended and restated bylaws, or the composition of our board of directors or its committees. If faced with a proxy contest or other type of shareholder activism, or a proxy advisory firm recommendation that is adverse to a management proposal, we may not be able to respond successfully to the contest or dispute, which would be disruptive to our business. Even if we are successful, our business could be adversely affected by such a contest or dispute involving us or our partners because:

- responding to proxy contests or other actions by activist stockholders, or adverse proxy advisory firm recommendations, can be costly and time-consuming, disrupting operations and diverting the attention of management and employees;
- perceived uncertainties as to future direction may result in the loss of potential acquisitions, collaborations or licensing opportunities, and may make it more difficult to attract and retain qualified personnel and business partners; and
- if individuals are elected to our board of directors with a specific agenda, it may adversely affect our ability to effectively and timely implement our strategic plan and create additional value for our stockholders.

These actions could cause our stock price to decrease and experience periods of increased volatility.

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

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). Therefore, the information that we provide stockholders may be different than what is available with respect to other public companies.

Table of Contents

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

We will remain an emerging growth company until the earliest of: (1) the end of the fiscal year in which the market value of our common stock that is held by non-affiliates exceeds $700 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 billion or more during such fiscal year; (3) the date on which we issue more than $1 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.

None.

Item 3. Defaults Upon Senior Securities.

None.

Item 4. Mine Safety Disclosures.

Not Applicable.

Item 5. Other Information.

None.

Table of Contents

Item 6. Exhibits.

Exhibit Number	Description	Incorporated by Reference			Filed or Furnished Herewith
		Form	Exhibit Number	Filing Date	
3.1	Restated Certificate of Incorporation	8-K	3.1	9/22/15	
3.2	Amended and Restated Bylaws	8-K	3.2	9/22/15	
10.1	Fourth Amendment to Lease dated April 20, 2018 between the Registrant and BMR-Medical Center Drive LLC				X
31.1	Certification of the Chief Executive Officer, as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
31.2	Certification of the Chief Financial Officer as required by Section 302 of the Sarbanes-Oxley Act of 2002				X
32.1	Certifications of the Chief Executive Officer and Chief Financial Officer as required by 18 U.S.C. 1350				X
101	The following materials from the Registrant's Quarterly Report on Form 10-Q for the fiscal quarter ended March 31, 2018 formatted in XBRL (eXtensible Business Reporting Language): (i) Consolidated Balance Sheets (ii) Consolidated Statements of Operations and Comprehensive Income (Loss) (iii) Consolidated Statements of Cash Flows (iv) Notes to Consolidated Financial Statements				X

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.

Table of Contents

SIGNATURES

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

REGENXBIO Inc.

Dated: May 8, 2018 /s/ Kenneth T. Mills
Kenneth T. Mills
President and Chief Executive Officer

(Principal Executive Officer)

Dated: May 8, 2018 /s/ Vittal Vasista
Vittal Vasista
Chief Financial Officer

(Principal Financial Officer and Principal Accounting Officer)