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

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**FORM 10-Q**

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

For the quarterly period ended September 30, 2015.

**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: 001-37625

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**Voyager Therapeutics, Inc.**  
(Exact name of Registrant as specified in its charter)

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

**75 Sidney Street,  
Cambridge, Massachusetts**  
(Address of principal executive offices)

**46-3003182**  
(I.R.S. Employer  
Identification No.)

**02139**  
(Zip Code)

**(857) 259-5340**  
(Registrant's telephone number, including area code)

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

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes  No

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

Large accelerated filer	<input type="checkbox"/>	(Do not check if a smaller reporting company)	Accelerated filer	<input type="checkbox"/>
Non-accelerated filer	<input checked="" type="checkbox"/>		Smaller reporting company	<input type="checkbox"/>

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

The number of outstanding shares of the registrant's common stock, par value \$0.001 per share, as of December 15, 2015 was 26,749,240.

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**VOYAGER THERAPEUTICS**

**FORM 10-Q**

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**PART I. FINANCIAL INFORMATION**

**Voyager Therapeutics, Inc.**  
**Condensed Balance Sheets**  
*(amounts in thousands, except share and per share data)*  
*(unaudited)*

	<u>September 30,</u>	<u>December 31,</u>
	<u>2015</u>	<u>2014</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 66,533	\$ 7,035
Marketable securities	41,138	—
Prepaid expenses and other current assets	942	1,323
Total current assets	108,613	8,358
Property and equipment, net	3,277	2,804
Deposits and other non-current assets	1,623	335
Marketable securities	52,922	—
Total assets	\$ 166,435	\$ 11,497
<b>Liabilities, redeemable convertible preferred stock and stockholders' deficit</b>		
Current liabilities:		
Accounts payable	\$ 541	\$ 1,554
Accrued expenses	2,942	642
Deferred rent, current portion	294	278
Deferred revenue, current portion	19,589	—
Total current liabilities	23,366	2,474
Deferred rent, net of current portion	1,093	1,314
Deferred revenue, net of current portion	40,205	—
Other non-current liabilities	52	255
Preferred stock tranche liability	—	6,305
Total liabilities	64,716	10,348
Commitments and contingencies (see note 7)		
Redeemable convertible preferred stock (Series A), \$0.001 par value: 45,000,000 shares authorized at September 30, 2015 and December 31, 2014; 45,000,000 and 25,000,000 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively; aggregate liquidation preference of \$48,621 and \$26,086, at September 30, 2015 and December 31, 2014 respectively	59,863	21,979
Redeemable convertible preferred stock (Series B), \$0.001 par value: 30,000,001 and 0 shares authorized, issued and outstanding at September 30, 2015 and December 31, 2014, respectively; aggregate liquidation preference of \$93,827 and 0 at September 30, 2015 and December 31, 2014, respectively	88,995	—
Stockholders' deficit:		
Common stock, \$0.001 par value: 95,000,000, and 65,000,000 shares authorized at September 30, 2015 and December 31, 2014 respectively; 1,369,715 and 814,834 shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively	1	1
Additional paid-in capital	—	—
Accumulated other comprehensive gain	38	—
Accumulated deficit	(47,178)	(20,831)
Total stockholders' deficit	(47,139)	(20,830)
Total liabilities, redeemable convertible preferred stock and stockholders' deficit	\$ 166,435	\$ 11,497

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

**Voyager Therapeutics, Inc.**  
**Condensed Statements of Operations and Comprehensive Loss**  
*(amounts in thousands, except per share and share data)*  
**(Unaudited)**

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2015	2014	2015	2014
Collaboration revenue	\$ 4,937	\$ —	\$ 12,397	\$ —
Operating expenses:				
Research and development	6,481	2,399	18,459	5,938
General and administrative	2,475	1,257	6,752	3,933
Total operating expenses	<u>8,956</u>	<u>3,656</u>	<u>25,211</u>	<u>9,871</u>
Operating loss	(4,019)	(3,656)	(12,814)	(9,871)
Other expense, net				
Interest income (expense), net	102	—	175	(2)
Other financing expense	—	(349)	(9,750)	(1,754)
Total other expense, net	<u>102</u>	<u>(349)</u>	<u>(9,575)</u>	<u>(1,756)</u>
Net loss	<u>\$ (3,917)</u>	<u>\$ (4,005)</u>	<u>\$ (22,389)</u>	<u>\$ (11,627)</u>
Other comprehensive gain				
Net unrealized gain on available-for-sale-securities	58	—	38	—
Total other comprehensive gain	<u>58</u>	<u>—</u>	<u>38</u>	<u>—</u>
Comprehensive loss	<u>\$ (3,859)</u>	<u>\$ (4,005)</u>	<u>\$ (22,351)</u>	<u>\$ (11,627)</u>
Reconciliation of net loss to net loss attributable to common stockholders:				
Net loss	\$ (3,917)	\$ (4,005)	\$ (22,389)	\$ (11,627)
Accretion of redeemable convertible preferred stock to redemption value	(2,594)	(406)	(6,045)	(859)
Accrued dividends on Series A redeemable convertible preferred stock	(403)	—	(1,039)	—
Net loss attributable to common stockholders	<u>\$ (6,914)</u>	<u>\$ (4,411)</u>	<u>\$ (29,473)</u>	<u>\$ (12,486)</u>
Net loss per share attributable to common stockholders, basic and diluted	<u>\$ (5.25)</u>	<u>\$ (6.45)</u>	<u>\$ (25.36)</u>	<u>\$ (21.41)</u>
Weighted-average common shares outstanding, basic and diluted	<u>1,317,150</u>	<u>684,192</u>	<u>1,161,982</u>	<u>583,294</u>

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

**Voyager Therapeutics, Inc.**  
**Condensed Statements of Cash Flows**  
*(amounts in thousands)*  
*(unaudited)*

	Nine Months Ended September 30,	
	2015	2014
<b>Cash flow from operating activities</b>		
Net loss	\$ (22,389)	\$ (11,627)
Adjustments to reconcile net loss to net cash used in operating activities:		
Stock-based compensation expense	2,061	275
Depreciation	436	73
Amortization of premiums and discounts on marketable securities	261	—
Change in fair value of preferred stock tranche liability	9,750	1,754
Non-cash interest on convertible promissory notes payable	—	2
Expense related to shares issued in connection with services performed	—	250
In-kind research and development expenses	2,190	—
Deferred rent	(205)	304
Changes in operating assets and liabilities:		
Prepaid expenses and other current assets	243	(644)
Other non-current assets	14	(2)
Deferred revenue	57,604	—
Accounts payable	(1,013)	566
Accrued expenses	1,952	881
Other non-current liabilities	(186)	—
Lease incentive benefit	138	1,042
Net cash provided by (used in) operating activities	50,856	(7,126)
<b>Cash flow from investing activities</b>		
Purchases of property and equipment	(909)	(1,979)
Change in restricted cash	—	(314)
Purchases of marketable securities	(100,283)	—
Proceeds from maturities of marketable securities	6,000	—
Net cash used in investing activities	(95,192)	(2,293)
<b>Cash flow from financing activities</b>		
Proceeds from the issuance of Series A redeemable convertible preferred stock and tranche rights, net of issuance costs	19,999	15,542
Proceeds from the issuance of Series B redeemable convertible preferred stock net of discount and issuance costs	84,780	—
Deferred initial public offering costs	(954)	—
Proceeds from the issuance of common stock and restricted stock	9	72
Net cash provided by financing activities	103,834	15,614
Net increase in cash and cash equivalents	59,498	6,195
Cash and cash equivalents, beginning of period	7,035	135
Cash and cash equivalents, end of period	\$ 66,533	\$ 6,330
<b>Supplemental disclosure of cash and non-cash activities</b>		
Accretion of redeemable convertible preferred stock to redemption value	\$ 6,045	\$ 859
Exchange of promissory notes payable and accrued interest into Series A redeemable convertible preferred stock and tranche rights	—	\$ 2,929
Deferred initial public offering costs included in accrued expenses	\$ 348	—

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

VOYAGER THERAPEUTICS INC.

NOTES TO UNAUDITED CONDENSED FINANCIAL STATEMENTS

**1. Nature of business**

Voyager Therapeutics, Inc. (“the Company”) is a clinical-stage gene therapy company focused on developing life-changing treatments for patients suffering from severe diseases of the central nervous system (the “CNS”). The Company focuses on CNS diseases where it believes that an adeno-associated virus (“AAV”) gene therapy approach can have a clinically meaningful impact by either increasing or decreasing the production of a specific protein. The Company has created a product engine that enables it to engineer, optimize, manufacture and deliver its AAV-based gene therapies that have the potential to provide durable efficacy following a single administration directly to the CNS. The Company’s product engine has rapidly generated programs for five CNS indications, including advanced Parkinson’s disease, a form of monogenic amyotrophic lateral sclerosis, Friedreich’s ataxia, Huntington’s disease and spinal muscular atrophy. The Company’s most advanced clinical candidate, VY-AADC01, is being evaluated for the treatment of advanced Parkinson’s disease in an open-label, Phase 1b clinical trial with the goal of generating human proof-of-concept data in the second half of 2016.

The Company is subject to risks common to companies in the biotechnology and gene therapy industry, including but not limited to, risks of failure of pre-clinical studies, and clinical trials, the need to obtain marketing approval for its drug product candidates, the need to successfully commercialize and gain market acceptance of its drug product candidates, dependence on key personnel, protection of proprietary technology, compliance with government regulations, development by competitors of technological innovations and ability to transition from pilot-scale manufacturing to large-scale production of products.

**2. Summary of significant accounting policies**

The following is a summary of significant accounting policies followed in the preparation of these financial statements.

***Basis of Presentation***

The accompanying unaudited condensed financial statements of the Company have been prepared in accordance with accounting principles generally accepted in the United States (“GAAP”) for interim financial reporting and as required by Regulation S-X, Rule 10-01. Accordingly, they do not include all of the information and footnotes required by GAAP for complete financial statements. For further information, refer to the financial statements and footnotes included in the Company’s Registration Statement on Form S-1 for the year ended December 31, 2014 as filed with the Securities and Exchange Commission (“SEC”). Any reference in these notes to applicable guidance is meant to refer to the authoritative United States generally accepted accounting principles as found in the Accounting Standards Codification (“ASC”) and Accounting Standards Updates (“ASU”) of the Financial Accounting Standards Board (“FASB”).

On November 16, 2015 the Company completed the sale of 5,750,000 shares of its common stock in its initial public offering (the “IPO”), at a price to the public of \$14.00 per share, resulting in estimated net proceeds to the Company of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company. On October 29, 2015, in preparation for the Company’s IPO, the Company’s Board of Directors and stockholders approved a 1-for-4.25 reverse split of the Company’s common stock, which became effective on October 29, 2015. All share and per share amounts in the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital. In connection with the closing of the IPO, all of the Company’s outstanding redeemable convertible preferred stock automatically converted into shares of common stock as of November 16, 2015, resulting in the issuance by the Company of an additional 17,647,054 shares of common stock. The significant increase in shares outstanding in November 2015 is expected to impact the year-over-year comparability of the Company’s net loss per share calculations over the next year.

### ***Use of Estimates***

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, the Company's management evaluates its estimates, which include, but are not limited to, estimates related to revenue recognition, accrued expenses, valuation of the tranche rights, stock-based compensation expense, income taxes and the fair value of common stock. The Company bases its estimates on historical experience and other market specific or other relevant assumptions that it believes to be reasonable under the circumstances. Actual results may differ from those estimates or assumptions.

### ***Unaudited Interim Financial Information***

The accompanying condensed balance sheets as of September 30, 2015, the condensed statements of operations and comprehensive loss for the three and nine months ended September 30, 2015 and 2014, and the condensed statements of cash flows for the nine months ended September 30, 2015 and 2014 are unaudited. The interim unaudited financial statements have been prepared on the same basis as the annual audited financial statements; and in the opinion of management, reflect all adjustments, which include only normal recurring adjustments necessary for the fair statement of the Company's financial position as of September 30, 2015, the results of its operations and comprehensive loss for the three and nine months ended September 30, 2015 and 2014, and its cash flows for the nine months ended September 30, 2015 and 2014. The financial data and other information disclosed in these notes related to the periods ended September 30, 2015 and 2014 are unaudited. The results for the three and nine months ended September 30, 2015, are not necessarily indicative of results to be expected for the year ending December 31, 2015, any other interim periods, or any future year or period.

### ***Fair Value of Financial Instruments***

ASC Topic 820, *Fair Value Measurement* ("ASC 820"), establishes a fair value hierarchy for instruments measured at fair value that distinguishes between assumptions based on market data (observable inputs) and the Company's own assumptions (unobservable inputs). 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.

ASC 820 identifies fair value as the exchange price, or exit price, representing the amount that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants. As a basis for considering market participant assumptions in fair value measurements, ASC 820 establishes a three-tier fair value hierarchy that distinguishes between the following:

- *Level 1*—Quoted market prices in active markets for identical assets or liabilities.
- *Level 2*—Inputs other than Level 1 inputs that are either directly or indirectly observable, such as quoted market prices, interest rates, and yield curves.
- *Level 3*—Unobservable inputs developed using estimates of assumptions developed by the Company, which reflect those that a market participant would use.

To the extent that the 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 carrying amounts reflected in the balance sheets for cash and cash equivalents, prepaid expenses and other current assets, accounts payable and accrued expenses approximate their fair values, due to their short-term nature.

**Cash and Cash Equivalents**

The Company considers all highly liquid investments purchased with original maturities of 90 days or less at acquisition to be cash equivalents. Cash and cash equivalents include cash held in banks and amounts held in money market funds.

**Marketable Securities**

The Company classifies marketable securities with a remaining maturity when purchased of greater than three months as available-for-sale. Marketable securities with a remaining maturity date greater than one year are classified as non-current where the Company has the intent and ability to hold these securities for at least twelve months. Available-for-sale securities are maintained by an investment manager and may consist of U.S. Treasury securities and U.S. government agency securities. Available-for-sale securities are carried at fair value with the unrealized gains and losses included in other comprehensive income as a component of stockholders' deficit until realized. Any premium or discount arising at purchase is amortized and/or accreted to interest income and/or expense. Realized gains and losses are determined using the specific identification method and are included in other income (expense). If any adjustment to fair value reflects a decline in value of the investment, the Company considers all available evidence to evaluate the extent to which the decline is "other-than-temporary" and, if so, recognizes the unrealized loss through a charge to the Company's statement of operations and comprehensive loss.

There were no marketable securities held as of December 31, 2014.

Marketable securities as of September 30, 2015 consist of the following:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
<b>As of September 30, 2015</b>				
Money market funds included in cash and cash equivalents	\$ 65,371	\$ —	\$ —	\$ 65,371
<b>Total money market funds included in cash and cash equivalents</b>	<b>\$ 65,371</b>	<b>\$ —</b>	<b>\$ —</b>	<b>\$ 65,371</b>
Marketable securities:				
U.S. Treasury notes	50,152	23	—	50,175
U.S. Government agency bonds	43,870	15	—	43,885
<b>Total marketable securities</b>	<b>\$ 94,022</b>	<b>\$ 38</b>	<b>\$ —</b>	<b>\$ 94,060</b>
<b>Total money market funds and marketable securities</b>	<b>\$ 159,393</b>	<b>\$ 38</b>	<b>\$ —</b>	<b>\$ 159,431</b>

The estimated fair value of the Company's marketable securities balance at September 30, 2015, by contractual maturity, is as follows:

Due in one year or less	\$ 41,138
Due after one year through two years	52,922
<b>Total marketable securities</b>	<b>\$ 94,060</b>

**Restricted Cash**

At September 30, 2015 and December 31, 2014, the Company maintained restricted cash totaling approximately \$314,000 held in the form of money market accounts as collateral for the Company's facility lease obligation and credit cards. The balance is included within deposits and other non-current assets in the accompanying balance sheets.



### **Property and Equipment**

Property and equipment consists of laboratory equipment, furniture and office equipment and leasehold improvements and is stated at cost, less accumulated depreciation. Maintenance and repairs that do not improve or extend the lives of the respective assets are expensed to operations as incurred; while costs of major additions and betterments are capitalized. Depreciation is calculated over the estimated useful lives of the assets using the straight-line method.

### **Impairment of Long-Lived Assets**

The Company evaluates long-lived assets for potential impairment when events or changes in circumstances indicate the carrying value of the assets may not be recoverable. Recoverability is measured by comparing the book values of the assets to the expected future net undiscounted cash flows that the assets are expected to generate. If such assets are considered to be impaired, the impairment to be recognized is measured by the amount by which the book values of the assets exceed their fair value. The Company has not recognized any impairment losses from inception through September 30, 2015.

### **Deferred Issuance Costs**

Deferred issuance costs, which primarily consist of direct incremental legal and accounting fees relating to the Company's proposed initial public offering of common stock are capitalized as incurred. The deferred issuance costs will be offset against proceeds upon the consummation of the offering. Approximately \$1,302,000 of deferred issuance costs were incurred and capitalized as of September 30, 2015. No amounts were capitalized as of December 31, 2014. Such costs are included within other non-current assets on the balance sheet.

### **Revenue Recognition**

As of September 30, 2015, all of the Company's revenue is generated exclusively from its collaboration agreement with Genzyme Corporation, a Sanofi company ("Genzyme").

The Company recognizes revenue in accordance with ASC Topic 605, *Revenue Recognition* ("ASC 605"). Accordingly, revenue is recognized for each unit of accounting when all of the following criteria are met:

- Persuasive evidence of an arrangement exists;
- Delivery has occurred or services have been rendered;
- The seller's price to the buyer is fixed or determinable; and
- Collectability is reasonably assured.

Amounts received prior to satisfying the revenue recognition criteria are recorded as deferred revenue in the Company's balance sheet. Amounts expected to be recognized as revenue within 12 months following the balance sheet date are classified as deferred revenue, current portion. Amounts not expected to be recognized as revenue within the 12 months following the balance sheet date are classified as deferred revenue, net of current portion.

The Company analyzes the multiple element arrangements based on the guidance in ASC Topic 605-25, *Revenue Recognition—Multiple Element Arrangements* ("ASC 605-25"). Pursuant to the guidance in ASC 605-25, the Company evaluates multiple element arrangements to determine (1) the deliverables included in the arrangement and (2) whether the individual deliverables represent separate units of accounting or whether they must be accounted for as a combined unit of accounting. This evaluation involves subjective determinations and requires management to make judgements about the individual deliverables and whether such deliverables are separate from other aspects of the contractual relationship. Deliverables are considered separate units of accounting provided that: (i) the delivered item(s) has value to the customer on a standalone basis and (ii) if the arrangement includes a general right of return relative to the delivered item(s), delivery or performance of the undelivered item(s) is considered probable and substantially within control of the Company. In assessing whether an item has standalone value, the Company considers factors such as the

research, manufacturing and commercialization capabilities of the collaboration partner and the availability of the associated expertise in the general marketplace. In addition, the Company considers whether the collaboration partner can use the other deliverable(s) for their intended purpose without the receipt of the remaining element(s), whether the value of the deliverable is dependent on the undelivered item(s) and whether there are other vendors that can provide the undelivered element(s). The Company's collaboration agreement does not contain a general right of return relative to any delivered items.

Arrangement consideration that is fixed or determinable is allocated among the separate units of accounting using the relative selling price method. Then the applicable revenue recognition criteria in ASC 605 are applied to each of the separate units of accounting in determining the appropriate period and pattern of recognition. The Company determines the selling price of a unit of accounting following the hierarchy of evidence prescribed by ASC 605-25. Accordingly, the Company determines the estimated selling price for units of accounting within each arrangement using vendor specific objective evidence ("VSOE") of selling price, if available, third party evidence ("TPE") of selling price if VSOE is not available, or best estimate of selling price ("BESP") if neither VSOE or TPE is available. The Company has only used BESP to estimate the selling price, since it has not had VSOE or TPE of selling price of any units of accounting to date. Determining BESP for a unit of accounting requires significant judgement. In developing the BESP for a unit of accounting, the Company considers applicable market conditions and relevant entity specific factors, including factors that were contemplated in negotiating the agreement with the customer and estimated costs. The Company validates BESP for units of accounting by evaluating whether changes in the key assumptions used to determine the BESP will have a significant effect on the allocation of arrangement consideration between multiple units of accounting.

Options are considered substantive if, at the inception of the arrangement, the Company is at risk as to whether the collaboration partner will choose to exercise the option. Factors that the Company considers in evaluating whether an option is substantive include the cost to exercise the option, the overall objective of the arrangement, the benefit the collaborator might obtain from the arrangement without exercising the option and the likelihood the option will be exercised. When an option is considered substantive, the Company does not consider the option or item underlying the option to be a deliverable at the inception of the arrangement and the associated option fees are not included in allocable consideration, assuming the option is not priced at a significant and incremental discount. Conversely, when an option is not considered substantive, the Company would consider the option, including other deliverables contingent upon the exercise of the option, to be a deliverable at the inception of the arrangement and a corresponding amount would be included in allocable arrangement consideration. In addition, if the price of the option includes a significant incremental discount, the option would be included as a deliverable at the inception of the arrangement.

The Company recognizes arrangement consideration allocated to each unit of accounting when all of the revenue recognition criteria in ASC 605 are satisfied for that particular unit of accounting. The Company will recognize revenue associated with license options upon exercise of the option, if the underlying license has standalone value from the other deliverables to be provided subsequent to delivery of the license. If the license does not have standalone value, the amounts allocated to the license option will be combined with the related undelivered items as a single unit of accounting. The amounts allocated to the license option in the Genzyme agreement will be deferred until the option is exercised. The revenue recognition upon option exercise will be determined based on whether the license has standalone value from the remaining deliverables under the arrangement at the time of exercise.

The Company recognizes the amounts associated with research and development services, alliance joint steering committees and development advisory committees ratably over the associated period of performance. If there is no discernible pattern of performance and/or objectively measurable performance measures do not exist, then the Company recognizes revenue under the arrangement on a straight-line basis over the period the Company is expected to complete its performance obligations. Conversely, if the pattern of performance in which the service is provided to the customer can be determined and objectively measureable performance exist, then the Company recognizes revenue under the arrangement using the proportional performance method. Revenue recognized is limited to the lesser of the cumulative amount of payments received of the cumulative revenue earned determined using the straight line method or proportional performance, as applicable, as of the period end date.

At the inception of an arrangement that includes milestone payments, the Company evaluates whether each milestone is substantive and at risk to both parties on the basis of the contingent nature of the milestone. This evaluation includes an assessment of whether: (i) the consideration is commensurate with either the Company's performance to

achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company's performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. The Company evaluates factors such as clinical, regulatory, commercial and other risks that must be overcome to achieve the respective milestone and the level of effort and investment required to achieve the respective milestone in making this assessment. There is considerable judgement involved in determining whether a milestone satisfies all of the criteria required to conclude that a milestone is substantive. In accordance with ASC Topic 605-28, *Revenue Recognition—Milestone Method* ("ASC 605-28") clinical and regulatory milestones that are considered substantive, will be recognized as revenue in its entirety upon successful accomplishment of the milestone, assuming all other revenue recognition criteria are met. Milestones that are not considered substantive would be recognized as revenue over the remaining period of performance, assuming all other revenue recognition criteria are met. Revenue from commercial milestone payments will be accounted for as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

The Company will recognize royalty revenue in the period of sale of the related product(s), based on the underlying contract terms, provided that the reported sales are reliably measurable and the Company has no remaining performance obligations, assuming all other revenue recognition criteria are met.

The Company also considers the impact of potential future payments it makes in its role as a vendor to its customers or collaboration partners and evaluates if these potential future payments could be reductions of revenue from that customer. If the potential future payments to the customer are (i) a separately identifiable benefit and (ii) the fair value of the identifiable benefit can be reasonably estimated, then the payments are accounted for separately from the revenue received from the customer. If however, both of these criteria are not satisfied, then the payments are treated as a reduction of revenue.

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

Research and development costs are charged to expense as incurred in performing research and development activities. The costs include employee compensation costs, external research, consultant costs, sponsored research, in-kind services provided under the Genzyme agreement, license fees, process development and facilities costs. Facilities costs primarily include the allocation of rent, utilities and depreciation.

### ***Research Contract Costs and Accruals***

The Company has entered into various research and development contracts with research institutions and other companies. These agreements are generally cancelable. The Company records accruals for estimated ongoing research costs. When evaluating the adequacy of the accrued liabilities, the Company analyzes progress of the studies, including the phase or completion of events, invoices received and contracted costs. Significant judgments and estimates may be made in determining the accrued balances at the end of any reporting period. Actual results could differ from the Company's estimates. The Company's historical accrual estimates have not been materially different from the actual costs.

### ***Patent Costs***

The Company expenses patent application and related legal costs as incurred and classifies such costs as general and administrative expenses in the accompanying statements of operations.

### ***Stock-Based Compensation Expense***

The Company accounts for its stock-based compensation awards in accordance with ASC Topic 718 *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all stock-based payments to employees and directors, including grants of restricted stock and stock options, to be recognized as expense in the statements of operations based on their grant date fair values. Grants of restricted stock and stock options to other service providers, referred to as non-employees, are required to be recognized as expense in the statements of operations based on their

vesting date fair values. The Company estimates the fair value of options granted using the Black-Scholes option pricing model. The Company uses the value of its common stock to determine the fair value of restricted stock awards.

The Black-Scholes option pricing model requires inputs based on certain subjective assumptions, including (a) the expected stock price volatility, (b) the calculation of expected term of the award, (c) the risk-free interest rate and (d) expected dividends. Due to a lack of company-specific historical and implied volatility data of the Company's common stock, the Company has based its estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. The historical volatility is calculated based on a period of time commensurate with the expected term assumption. The computation of expected volatility is based on the historical volatility of a representative group of companies with similar characteristics to the Company, including stage of product development and life science industry focus. The Company uses the simplified method as prescribed by the SEC Staff Accounting Bulletin No. 107, *Share-Based Payment*, to calculate the expected term for stock options granted to employees as it does not have sufficient historical exercise data to provide a reasonable basis upon which to estimate the expected term. For stock options granted to non-employees, the Company utilizes the contractual term of the arrangement as the basis for the expected term assumption. The risk-free interest rate is based on a treasury instrument whose term is consistent with the expected term of the stock options. The expected dividend yield is assumed to be zero as the Company has never paid dividends and has no current plans to pay any dividends on its common stock.

The Company expenses the fair value of its stock-based compensation awards to employees on a straight-line basis over the associated service period, which is generally the period in which the related services are received. Stock-based compensation awards to non-employees are adjusted through stock-based compensation expense at each reporting period end to reflect the current fair value of such awards and are expensed on a straight-line basis.

The Company records the expense for stock-based compensation awards subject to performance-based milestone vesting over the remaining service period when management determines that achievement of the milestone is probable. Management evaluates when the achievement of a performance-based milestone is probable based on the expected satisfaction of the performance conditions as of the reporting date.

### ***Income Taxes***

Income taxes are recorded in accordance with ASC Topic 740, *Income Taxes* ("ASC 740"), which provides for deferred taxes using an asset and liability approach. Under this method, deferred tax assets and liabilities are determined based on the difference between the financial reporting and the tax reporting basis of assets and liabilities and are measured using the enacted tax rates and laws that are expected to be in effect when the differences are expected to reverse. The Company provides a valuation allowance against net deferred tax assets unless, based upon the weight of available evidence, it is more likely than not that the deferred tax assets will be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2014, the Company does not have any significant uncertain tax positions.

### ***Comprehensive Loss***

Comprehensive loss is comprised of net loss and other comprehensive loss. Other comprehensive loss consists of unrealized gains and losses on marketable securities for the nine months ended September 30, 2015. There was no other comprehensive loss for the nine months ended September 30, 2014.

### ***Net Loss Per Share***

Basic net loss per share of common stock is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period, without consideration for potentially dilutive securities. Diluted net loss per share is computed by dividing the net loss

attributable to common stockholders by the weighted-average number of shares of common stock and potentially dilutive securities outstanding for the period determined using the treasury-stock and if-converted methods.

For purposes of the diluted net loss per share calculation, redeemable convertible preferred stock and unvested restricted common stock are considered to be potentially dilutive securities, but are excluded from the calculation of diluted net loss per share because their effect would be anti-dilutive and therefore, basic and diluted net loss per share were the same for all periods presented.

The following table sets forth the outstanding potentially dilutive securities that have been excluded in the calculation of diluted net loss per share because to do so would be anti-dilutive (in common stock equivalent shares):

	<u>As of September 30,</u>	
	<u>2015</u>	<u>2014</u>
Series A redeemable convertible preferred stock	10,588,235	5,882,352
Series B redeemable convertible preferred stock	7,058,819	—
Unvested restricted common stock	2,013,140	2,505,256
Outstanding stock options	852,249	—
Total	<u>20,512,443</u>	<u>8,387,608</u>

The Company's redeemable convertible preferred stock is entitled to receive dividends based on dividends declared to common stockholders, thereby giving the preferred stockholders the right to participate in undistributed earnings of the Company above the stated dividend rate. However, preferred stockholders do not have a contractual obligation to share in the losses of the Company. As of December 31, 2014 and all prior periods reported, the Company has been in a net loss position; therefore, the Company's accounting for basic and diluted earnings per share was unaffected by the participation rights of the preferred stockholders. All of the Company's outstanding convertible preferred stock automatically converted into shares of common stock as of November 16, 2015, resulting in the issuance by the Company of an additional 17,647,054 shares of common stock.

The following table summarizes the Company's unaudited pro forma net loss per share attributable to common stockholders (in thousands, except share and per share data):

	<u>Three Months Ended</u>		<u>Nine Months Ended</u>	
	<u>September 30,</u>		<u>September 30,</u>	
	<u>2015</u>	<u>2014</u>	<u>2015</u>	<u>2014</u>
Pro forma net loss attributable to common stockholders	\$ (6,914)	\$ (4,411)	\$ (29,473)	\$ (12,486)
Weighted average number of common shares outstanding, basic and diluted	1,317,150	684,192	1,161,982	583,294
Add:				
Dilutive effect of common share equivalents resulting from common share options and preferred common shares (as converted)	—	—	—	—
	<u>1,317,150</u>	<u>684,192</u>	<u>1,161,982</u>	<u>583,294</u>
Pro forma basic and diluted net loss per share attributable to common stockholders	\$ (5.25)	\$ (6.45)	\$ (25.36)	\$ (21.41)

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

The Company has no financial instruments with off-balance sheet risk such as foreign exchange contracts, option contracts or other foreign hedging arrangements. Financial instruments that potentially subject the Company to a

concentration of credit risk are cash and cash equivalents. The Company's cash is held in accounts at a financial institution that may exceed federally insured limits. The Company has not experienced any credit losses in such accounts and does not believe it is exposed to any significant credit risk on these funds.

### **Concentration of Suppliers**

The Company is dependent on a third-party manufacturer to supply certain products for research and development activities in its programs. In particular, the Company relies and expects to continue to rely on a sole manufacturer to supply it with specific vectors related to the Company's research and development programs.

### **Segment Information**

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker in deciding how to allocate resources and assess performance. The Company and the Company's chief operating decision maker, the Company's Chief Executive Officer, views the Company's operations and manages its business as a single operating segment, which is the business of developing and commercializing gene therapies.

### **Recent Accounting Pronouncements**

In May 2014, the FASB issued ASU No. 2014-09, *Revenue From Contracts With Customers*. ASU 2014-09 amends Accounting Standards Codification ASC 605, *Revenue Recognition*, by outlining a single comprehensive model for entities to use in accounting for revenue arising from contracts with customers. ASU 2014-09 will be effective for the Company for interim and annual periods beginning after December 15, 2017. The Company is evaluating the impact that this ASU may have on its financial statements, if any.

In August 2014, the FASB issued ASU No. 2014-15, which requires management to assess an entity's ability to continue as a going concern every reporting period, and provide certain disclosures if management has substantial doubt about the entity's ability to operate as a going concern, or an express statement if not, by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. This guidance is effective for the annual period ending after December 15, 2016, and for annual periods and interim periods thereafter. Early application is permitted. The Company is in process of evaluating this guidance and determining the expected effect on its financial statements.

In April 2015, the FASB issued ASU No. 2015-03, which amends existing guidance to require the presentation of debt issuance costs in the balance sheet as a deduction from the carrying amount of the related debt liability instead of a deferred charge. ASU No. 2015-03 is effective for annual reporting periods beginning after December 15, 2015, and early adoption is permitted. The amendment must be applied retrospectively such that the balance sheet of each individual period presented is adjusted to reflect the period-specific impact of using the new guidance. Upon transition, a business must adhere to the appropriate disclosures for an adjustment in an accounting principle. Such disclosures include why the change in accounting principle is occurring, the transition method, an explanation of the prior period information that was retrospectively adjusted, and how the change impacts the financial statement line items (i.e., debt issuance cost asset and the debt liability). The Company is currently in the process of evaluating the timing and impact of adoption.

### 3. Fair Value Measurements

Assets and liabilities measured at fair value on a recurring basis as of December 31, 2014 are as follows (in thousands):

Liabilities	December 31, 2014	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Convertible preferred stock tranche liability	\$ 6,305	\$ —	\$ —	\$ 6,305
Total	<u>\$ 6,305</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$ 6,305</u>

The Company estimates the fair value of its Series A redeemable convertible preferred stock (“Series A Preferred Stock”) tranche liability at the time of issuance and subsequently remeasures it using a probability-weighted present value model that considers the probability of closing a tranche (67%), the estimated future value of Series A Preferred Stock at closing (\$1.51), and the investment required (\$20.0 million) at closing. Future values are converted to present value using a discount rate (16.2%) appropriate for probability-adjusted cash flows. These estimates are based, in part, on subjective assumptions. Changes to these assumptions as well as the Company’s stock value on the reporting date can have a significant impact on the fair value of the Series A Preferred Stock tranche liability.

The following table provides a reconciliation of all assets and liabilities measured at fair value using Level 3 significant unobservable inputs (in thousands):

	Preferred Stock Tranche Liability
Balance at December 31, 2014	\$ 6,305
Changes in fair value	9,750
Reclassification to Series A Preferred Stock	(16,055)
Balance at September 30, 2015	<u>\$ —</u>

Assets and liabilities measured at fair value on a recurring basis as of September 30, 2015 are as follows (in thousands):

Assets	September 30, 2015	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Money market funds included in cash and cash equivalents	\$ 65,371	\$ 65,371	\$ —	\$ —
Marketable securities:				
U.S. Treasury notes	50,175	50,152	—	—
U.S. government agency securities	43,885	—	43,870	—
Total	<u>\$ 159,431</u>	<u>\$ 115,523</u>	<u>\$ 43,870</u>	<u>\$ —</u>

The Company measures the fair value of money market funds and U.S. Treasuries based on quoted prices in active markets for identical securities. The Level 2 marketable securities include U.S. government and agency securities that are valued either based on recent trades of securities in inactive markets or based on quoted market prices of similar instruments and other significant inputs derived from or corroborated by observable market data. The Company did not hold any marketable securities at December 31, 2014.

#### 4. Prepaid Expenses and Other Current Assets

Prepaid expense and other current assets consist of the following (in thousands):

	<u>As of</u> <u>September 30,</u> <u>2015</u>	<u>As of</u> <u>December 31,</u> <u>2014</u>
Prepaid expenses	\$ 679	\$ 900
Other current assets	263	423
Total	<u>\$ 942</u>	<u>\$ 1,323</u>

#### 5. Property and Equipment, net

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

	<u>As of</u> <u>September 30,</u> <u>2015</u>	<u>As of</u> <u>December 31,</u> <u>2014</u>
Laboratory equipment	\$ 2,081	\$ 1,223
Furniture and office equipment	484	441
Leasehold improvements	<u>1,332</u>	<u>1,324</u>
	3,897	2,988
Less: accumulated depreciation	<u>(620)</u>	<u>(184)</u>
Property and equipment, net	<u>\$ 3,277</u>	<u>\$ 2,804</u>

The Company recorded \$63,000 and \$160,000 during the three months ended September 30, 2015 and September 30, 2014, respectively. The Company recorded \$73,000 and \$436,000 in depreciation expense during the nine months ended September 30, 2014 and September 30, 2015, respectively.

#### 6. Accrued Expenses

Accrued expenses consist of the following (in thousands):

	<u>As of</u> <u>September 30,</u> <u>2015</u>	<u>As of</u> <u>December 31,</u> <u>2014</u>
Patent costs	\$ 250	\$ 274
Research and development costs	1,460	125
Professional services	570	81
Employee compensation costs	176	85
Other	<u>486</u>	<u>77</u>
Total	<u>\$ 2,942</u>	<u>\$ 642</u>

#### 7. Commitments and Contingencies

##### *Operating Leases*

During March 2014, the Company entered into an agreement to lease its facility under a non-cancelable operating lease that expires December 15, 2019. The lease includes two renewal options, each for five year terms and at fair market value upon exercise. The lease contains escalating rent clauses which require higher rent payments in future years. The Company expenses rent on a straight-line basis over the term of the lease, including any rent-free periods.



The Company received a leasehold improvement incentive from the landlord totaling \$1,250,000. The Company recorded these incentives as a component of deferred rent and will amortize these incentives as a reduction of rent expense over the life of the lease. These leasehold improvements have been recorded as fixed assets.

Rent expense of approximately \$168,000 and \$140,000 and \$403,000 and \$590,000 was incurred during the three and nine months ended September 30, 2014 and September 30, 2015, respectively.

Future annual minimum lease payments at December 31, 2014 are as follows (in thousands):

	<b>Total Minimum Lease Payments</b>
2015	\$ 1,117
2016	1,170
2017	1,192
2018	1,214
2019	1,184
	<u>\$ 5,877</u>

## **Significant Agreements**

### **Genzyme Collaboration Agreement**

#### *Summary of Agreement*

In February 2015, the Company entered into an agreement with Genzyme (“Collaboration Agreement”), which included a non-refundable upfront payment of \$65.0 million. In addition, contemporaneous with entering into the Collaboration Agreement, Genzyme entered into a Series B Stock Purchase Agreement, under which Genzyme purchased 10,000,000 shares of Series B Preferred Stock for \$30.0 million. The fair value of the Series B Preferred Stock at the time of issuance was approximately \$25.0 million. The \$5.0 million premium over the fair value is accounted for as additional consideration under the Collaboration Agreement.

Under the Collaboration Agreement, the Company granted Genzyme an exclusive option to license, develop and commercialize (i) ex-U.S. rights to the following programs, which are referred to as Split Territory Programs; VY-AADC01 (“Parkinson’s Program”), VY-FXN01 (“Friedreich’s Ataxia Program”), a future program to be designated by Genzyme (“Future Program”) and VY-HTT01 (“Huntington’s Program”) with an incremental option to co-commercialize VY-HTT01 in the United States and (ii) worldwide rights to VY-SMN101 (“Spinal Muscular Atrophy Program”). Genzyme’s option for the Split Territory Programs and the Spinal Muscular Atrophy Program is triggered following the completion of the first proof-of-principle human clinical study (“POP Study”), on a program by program basis.

Prior to any option exercise by Genzyme, the Company will collaborate with Genzyme in the development of products under each Split Territory Program and VY-SMN101 pursuant to a written development plan and under the guidance of an Alliance Joint Steering Committee (“AJSC”), comprised of an equal number of employees from the Company and Genzyme.

The Company is required to use commercially reasonable efforts to develop products under each Split Territory Program and the Spinal Muscular Atrophy Program through the completion of the applicable POP Study. During the development of these joint programs, the activities are guided by a Development Advisory Committee (“DAC”). The DAC may elect to utilize certain Genzyme technology relating to the VY-AADC01 Program, the VY-HTT01 Program or generally with the manufacture of Split Territory Program products.

The Company is solely responsible for all costs incurred in connection with the development of the Split Territory Programs and the Spinal Muscular Atrophy Program products prior to the exercise of an option by Genzyme with the exception of the following: (i) at the Company’s request and upon mutual agreement, Genzyme will provide “in-kind” services valued at up to \$5.0 million and (ii) Genzyme shall be responsible for the costs and expenses of

activities under the Huntington's Program development plan to the extent such activities are covered by financial support Genzyme is entitled to receive from a patient advocacy group, collectively Genzyme "in-kind" and other funding.

Other than the Parkinson's Program (for which a POP Study has already been commenced), if the Company does not initiate a POP Study for a given Split Territory Program by December 31, 2026 (or for the Future Program by the tenth anniversary of the date the Future Program is nominated by Genzyme), and Genzyme has not terminated the Collaboration Agreement with respect to the collaboration program, then Genzyme shall be entitled, as its sole and exclusive remedy, to a credit of \$10.0 million for each such program against other milestone or royalty payments payable by Genzyme under the Collaboration Agreement. However, if the POP Study is not initiated due to a regulatory delay or a force majeure event, such time period shall be extended for so long as such delay continues.

With the exception of the Parkinson's Program, Genzyme is required to pay an option exercise payment of \$20.0 million or \$30.0 million for each Split Territory Program, as well as the Spinal Muscular Atrophy Program.

Upon Genzyme's exercise of its option to license a given product in a Split Territory Program ("Split Territory Licensed Product"), the Company will have sole responsibility for the development of such Split Territory Licensed Product in the United States and Genzyme shall have sole responsibility for development of such Split Territory Licensed Product in the rest of the world. The Company and Genzyme will have shared responsibility for execution of ongoing development of such Split Territory Licensed Product that is not specific to either territory, including costs associated therewith. The Company is responsible for all commercialization activities relating to Split Territory Licensed Products in the United States, including all of the associated costs. Genzyme is responsible for all commercialization activities relating to the Split Territory Licensed Products in the rest of the world, including all of the associated costs. If Genzyme exercised its co-commercialization rights, Genzyme will be the lead party responsible for all commercialization activities related to Huntington's Licensed Product in the United States.

Upon exercise of the option, Genzyme shall have the sole right to develop the Spinal Muscular Atrophy Product worldwide. Genzyme shall be responsible for all of the development costs that occur after the option exercise date for the Spinal Muscular Atrophy Program. Genzyme is also responsible for commercialization activities relating to the Spinal Muscular Atrophy Product worldwide.

Genzyme is required to pay the Company for specified regulatory and commercial milestones, if achieved, up to \$645 million across all programs. The regulatory approval milestones are payable upon either regulatory approval in the United States or regulatory and reimbursement approval in the European Union and range from \$40.0 million to \$50.0 million per milestone, with an aggregate total of \$265 million. The commercial milestones are payable upon achievement of specified annual net sales in each program and range from \$50.0 million to \$100 million per milestone, with an aggregate total of \$380 million.

In addition, to the extent any Split Territory Licensed Products or the Spinal Muscular Atrophy Licensed Product are commercialized, the Company is entitled to tiered royalty payments ranging from the mid-single digits to mid-teens based on a percentage of net sales by Genzyme. Genzyme is entitled to receive tiered royalty payments related to sales of Split Territory Licensed Product ranging from the low-single digits to mid-single digits based on a percentage of net sales by the Company depending on whether the Company uses Genzyme technology in the Split Territory Licensed Product. If Genzyme elects to co-commercialize VY-HTT01 in the United States, the Company and Genzyme will share in any profits or losses from VY-HTT01 product sales.

The Collaboration Agreement will continue in effect until the later of (i) the expiration of the last to expire of the option rights and (ii) the expiration of all payment obligations unless sooner terminated by the Company or Genzyme. The Company and Genzyme have customary termination rights including the right to terminate for an uncured material breach of the agreement committed by the other party and Genzyme has the right to terminate for convenience.

#### *Accounting Analysis*

The Collaboration Agreement includes the following deliverables: (i) research and development services for each of the Split Territory License Programs and the Spinal Muscular Atrophy Program, (ii) participation in the AJSC, (iii) participation in the DAC and (iv) the option to obtain a development and commercial license in the Parkinson's

Program and related deliverables. The Company has determined that the option to obtain a development and commercial license in the Parkinson's program is not a substantive option for accounting purposes, primarily because there is no additional option exercise payment payable by Genzyme at the time the option is exercised. Therefore, the option to obtain a license and other obligations of the Company that are contingent upon exercise of the option are considered deliverables at the inception of the arrangement. The options in the other Split Territory Programs and the Spinal Muscular Atrophy Program are considered substantive as there is substantial option exercise payments payable by Genzyme upon exercise. In addition, as a result of the uncertainties related to the discovery, research, development and commercialization activities, the Company is at risk with regard to whether Genzyme will exercise the options. Moreover, the substantive options are not priced at a significant incremental discount. Accordingly, the substantive options are not considered deliverables at the inception of the arrangement and the associated option exercise payments are not included in allocable arrangement consideration. The Company has also determined that any obligations which are contingent upon the exercise of a substantive option are not considered deliverables at the outset of the arrangement, as these deliverables are contingent upon the exercise of the options. In addition, any option exercise payments associated with the substantive options are not included in the allocable arrangement consideration.

The Company has concluded that each of the deliverables identified at the inception of the arrangement has standalone value from the other undelivered elements. Additionally, the Collaboration Agreement does not include return rights related to the initial collaboration term. Accordingly, each deliverable qualifies as a separate unit of accounting.

The Company has identified \$79.3 million of allocable arrangement consideration consisting of the \$65 million upfront fee, the \$5.0 million premium paid in excess of fair value of the Series B Preferred Stock and \$9.3 million of Genzyme "in-kind" and other funding.

The Company has allocated the allocable arrangement consideration based on the relative selling price of each unit of accounting. For all units of accounting, the Company determined the selling price using the best estimate of selling price ("BESP"). The Company determined the BESP for the service related deliverable for the research and development activities based on internal estimates of the costs to perform the services, including expected internal expenses and expenses with third parties for services and supplies, marked up to include a reasonable profit margin and adjusted for the scope of the potential license. Significant inputs used to determine the total expense of the research and development activities include, the length of time required and the number and costs of various studies that will be performed to complete the POP Study. The BESP for the AJSC and DAC have been estimated based on the costs incurred to participate in the committees, marked up to include a reasonable profit margin. The BESP for the license option was determined based on the estimated value of the license and related deliverables adjusted for the estimated probability that the option would be exercised by Genzyme.

Based on the relative selling price allocation, the allocable arrangement consideration was allocated as follows:

<u>Unit of Accounting</u>	<u>Amount</u> <u>(in thousands)</u>
Research and Development Services for:	
Huntington's Program	\$ 15,662
Parkinson's Program	6,648
Friedreich's Ataxia Program	16,315
Spinal Muscular Atrophy Program	32,050
Future Program	2,464
Committee Obligations:	
AJSC	147
DAC	227
License Option and related deliverables	5,743
<b>Total</b>	<b>\$ 79,256</b>

The Company recognizes the amounts associated with research and development services on a straight line basis over the period of service as there is no discernable pattern or objective measure of performance for the services. Similarly, the Company recognizes the amount associated with the committee obligations on a straight line basis over the

period of service consistent with the expected pattern of performance. The amounts allocated to the license option will be deferred until the option is exercised. The revenue recognition upon option exercise will be determined based on whether the license has standalone value from the remaining deliverables at the time of exercise.

The Company has evaluated all of the milestones that may be received in connection with the Split Territory Licensed Product and the Spinal Muscular Atrophy Program Licensed Product. In evaluating if a milestone is substantive, the Company assesses whether: (i) the consideration is commensurate with either the Company's performance to achieve the milestone or the enhancement of the value of the delivered item(s) as a result of a specific outcome resulting from the Company performance to achieve the milestone, (ii) the consideration relates solely to past performance and (iii) the consideration is reasonable relative to all of the deliverables and payment terms within the arrangement. All regulatory milestones are considered substantive on the basis of the contingent nature of the milestone, specifically reviewing factors such as the scientific, clinical, regulatory, and other risks that must be overcome to achieve the milestone as well as the level of effort and investment required. Accordingly, such amounts will be recognized as revenue in full in the period in which the associated milestone is achieved, assuming all other revenue recognition criteria are met. All commercial milestones will be accounted for in the same manner as royalties and recorded as revenue upon achievement of the milestone, assuming all other revenue recognition criteria are met.

During the nine month period ended September 30, 2015, the Company recognized \$12,397,000 of revenue associated with its collaboration with Genzyme related to research and development services performed during the period. As of September 30, 2015, there is \$59,794,000 of deferred revenue related to the Collaboration Agreement, which is classified as either current or noncurrent in the accompanying balance sheet based on the period the services are expected to be delivered.

Costs incurred relating to the programs that Genzyme has the option to license under the Collaboration Agreement consist of internal and external research and development costs, which primarily include: salaries and benefits, lab supplies and preclinical research studies. The Company does not separately track or segregate the amount of costs incurred under the Collaboration Agreement. All of these costs are included in research and development expenses in the Company's statement of operations during the nine months ended September 30, 2015. The Company estimates that the majority of research and development expense during the period relate to programs for which Genzyme has an option right.

#### **University of Massachusetts ("UMass") and MassBiologics Collaboration**

In January 2014, UMass and the Company entered into a Collaboration Agreement wherein the Company granted UMass 23,529 shares of common stock, valued at \$12,000, which was recorded as research and development expense. This was the only payment made under the Collaboration Agreement until it was amended by the Collaboration Agreement entered into with UMass and MassBiologics in October 2014.

On October 20, 2014, the Company entered into a Collaboration Agreement with UMass and MassBiologics (of the UMass Medical School).

Under the agreement, the Company shall (i) fund certain projects that will be conducted by UMass or MassBiologics, (ii) fund certain educational programs of UMass, including post-doctoral research at the Company's laboratories beginning in 2015, and (iii) collaborate with MassBiologics to establish scalable processes for manufacturing recombinant adeno-associated viral ("rAAV") vector products using current good manufacturing practices.

In November 2014, the parties agreed to the first project under this agreement whereby the Company will fund approximately \$2,861,000 over a sixteen month period for certain research and development services performed by MassBiologics. The project commenced in January 2015. If the agreement is terminated for any reason, the Company is obligated to fund the remaining balance of the total price of all work completed and any other out of pocket costs incurred by MassBiologics on behalf of the Company. As of December 31, 2014 and September 30, 2015, the Company had provided cumulative funding of approximately \$376,000 and \$1,058,000, respectively, which exceeded costs incurred by \$376,000 at December 31, 2014 and approximately \$701,000 funding was due at September 30, 2015. The

amount funded in excess of costs incurred is recorded in prepaid expenses as of the balance sheet at December 31, 2014 and recorded in accrued expenses for the amounts due at September 30, 2015. Research and development costs incurred by MassBiologics under the project agreement will be expensed by the Company as incurred.

### **Other Agreements**

During 2014 and 2015, the Company entered into various agreements with contract research organizations and institutions to license intellectual property. In consideration for the licensed rights the Company generally made upfront payments, which were recorded as research and development expense as the acquired technologies were considered in-process research and development. During the year ended December 31, 2014 and nine months ended September 30, 2014 and 2015, the Company paid \$830,000, \$800,000 and \$75,000, respectively, in up-front license fees. The license agreements also obligate the Company to make additional payments that are contingent upon specific clinical trial and regulatory approval milestones being achieved as well as royalties on future product sales. The agreements to license intellectual property include potential milestone payments that are dependent upon the development of products licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. The maximum aggregate potential milestone payments payable by the Company total approximately \$12.0 million. Additionally, under the terms of one agreement, the Company has options to license intellectual property to be used in the development of therapies for four additional disease indications. If the Company exercises all of the options under the agreement, it would be obligated to pay aggregate up-front fees of up to approximately \$1.5 million and milestone payments that are contingent upon clinical trial results and regulatory approval of \$5.0 million per disease indication, or up to \$20.0 million in total. As of December 31, 2014 and September 30, 2015, there have been no milestones achieved. The Company can generally terminate the license agreements upon 30-90 days prior written notice.

Additionally, certain license agreements require the Company to reimburse the licensor for certain past and ongoing patent related expenses. During the year ended December 31, 2014 and the nine months ended September 30, 2014 and September 30, 2015, the Company incurred \$839,000, \$740,000 and \$131,000 of expense, respectively, related to these reimbursable patent costs which are recorded as general and administrative expense.

### **Litigation**

The Company is not a party to any litigation and does not have contingency reserves established for any litigation liabilities as of December 31, 2014 or September 30, 2015.

### **8. Redeemable Convertible Preferred Stock**

The Company's redeemable convertible preferred stock ("Preferred Stock") has been classified as temporary equity on the accompanying balance sheets instead of in stockholders' deficit in accordance with authoritative guidance for the classification and measurement of redeemable securities as the redeemable convertible preferred stock is redeemable at the option of the holder after the redemption date, February 2021.

#### *Series A Preferred Stock*

At September 30, 2015, 45,000,000 shares of Series A Preferred Stock were authorized, issued and outstanding. These shares were issued at various closings in 2014 and 2015 for \$1.00 per share. The shares were issued in exchange for cash proceeds of \$42,039,000, net of issuance costs of \$32,000, and the exchange of outstanding redeemable Convertible Notes, including accrued interest, of approximately \$2,929,000. The Series A Preferred Stock have a liquidation preference amount of \$48,621,000 at September 30, 2015.

#### *Tranche Rights Issued with Series A Preferred Stock*

Included in the terms of the January 2014 Series A Preferred Stock Purchase Agreement were certain rights ("Tranche Rights") granted to the investors of Series A Preferred Stock purchased in January 2014, including the holders of the redeemable Convertible Notes who exchanged the redeemable Convertible Notes. The Tranche Rights obligated the investors in Series A Preferred Stock to purchase and the Company to sell an additional 18,500,000 shares of

Series A Preferred Stock at \$1.00 per share contingent upon successful near term in-licensing and progress on initial experiments and research and development planning ("Tranche Right I"). In addition, the investors are obligated to purchase and the Company is obligated to sell an additional 20,000,000 shares of Series A Preferred Stock upon the development of project engine and achievement of certain clinical milestones ("Tranche Right II"). In addition, the Tranche Rights allowed the investors the ability to purchase the additional shares at their option at any time. The Tranche Rights were transferrable by the investors, subject to approval by the Board.

The Company has concluded the Tranche Rights meet the definition of a freestanding financial instrument, as the Tranche Rights are legally detachable and separately exercisable from the Series A Preferred Stock. Therefore, the Company has allocated the proceeds between the Tranche Rights and the Series A Preferred Stock. As the Series A Preferred Stock is redeemable at the holder's option, the Tranche Rights are classified as an asset or liability and are initially recorded at fair value. The Tranche Rights are measured at fair value at each reporting period. Since the Tranche Rights are subject to fair value accounting, the Company allocated the proceeds to the Tranche Rights based on the fair value at the date of issuance with the remaining proceeds being allocated to the Series A Preferred Stock. The estimated fair value of the Tranche Rights was determined using a probability-weighted present value model that considers the probability of closing a tranche, the estimated future value of Series A Preferred Stock each closing, and the investment required at each closing. Future values are converted to present value using a discount rate appropriate for probability-adjusted cash flows.

The following table summarizes the initial value of the Tranche Rights included in the Series A Preferred Stock Purchase Agreement (in thousands):

	<b>Fair Value of Tranche Right Asset (Liability)</b>
Tranche Right I	\$ 1,495
Tranche Right II	(4,095)
Total value of Tranche Rights	<u>\$ (2,600)</u>

Tranche Right I was initially recorded as an asset of \$1,495,000 as the purchase price of the additional shares was greater than the estimated value of the Series A Preferred Stock at the expected settlement date. The Company issued 18,500,000 additional shares under Tranche Right I, in three separate closings during the year ended December 31, 2014 with total proceeds of \$18,491,000, net of issuance costs. Prior to each closing, any change in the value of Tranche Right I was recorded as other financing expense. The fair value of the portion of the Tranche Right I settled at each closing was reclassified to Series A Preferred Stock. The Company recognized income of \$261,000 related to the mark to market of Tranche Right I during the year ended December 31, 2014, which is included in other financing expense.

Tranche Right II was initially recorded as a liability of \$4,095,000 as the purchase price of the additional shares was less than the estimated price of the Series A Preferred Stock at the expected settlement date. The Company recognized expense of \$2,210,000 related to the mark to market of Tranche Right II during the year ended December 31, 2014, which is included in other financing expense.

In February 2015, Tranche Right II was settled when the Company closed the final issuance of Series A Preferred Stock. The Company recognized expense of \$9,750,000 related to the mark to market of Tranche Right II during the period ended September 30, 2015, which is included in other financing expense. The fair value of the Tranche Right II settled at closing was reclassified to Series A Preferred Stock. The initial carrying amount of the Series A Preferred Stock issued upon the closing of Tranche Right II amounted to approximately \$36,054,000 which exceeds the redemption value, therefore the carrying value is not being subsequently adjusted. However, the Company has reflected accrued dividends of approximately \$1,039,000 related to this issuance in the net loss attributable to common shareholders for the nine months ended September 30, 2015.

### *Series B Preferred Stock*

At September 30, 2015, 30,000,001 shares of Series B Preferred Stock were authorized, issued and outstanding. These shares were issued for \$3.00 per share. This issuance resulted in cash proceeds of \$89,780,000, net of issuance costs of \$220,000. Additionally, a discount of \$5,000,000 was recorded against the proceeds as the amount paid by Genzyme was in excess of fair value of the Series B Preferred Stock at issuance. The Series B Preferred Stock has a liquidation preference amount of \$93,827,000.

### *Preferred Stock*

The rights, preferences, and privileges of the Preferred Stock are listed below:

#### ***Conversion***

Shares of Preferred Stock are convertible at any time at the option of the holder into such number of shares as is determined by dividing the original issuance price by the conversion price in effect at the time. As of September 30, 2015, the conversion price was \$4.25 for Series A Preferred Stock and \$12.75 for Series B Preferred Stock, subject to adjustments to reflect the issuance of Common Stock, options, warrants, or other rights to subscribe for or to purchase Common Stock for a consideration per share, less than the conversion price then in effect and subsequent stock dividends and stock splits. In addition any reorganization, recapitalization, reclassification, consolidation or merger in which common stock is exchanged for securities, cash or other property.

All outstanding shares of Preferred Stock are automatically converted upon the completion of either an IPO resulting in gross proceeds to the Company of at least \$50.0 million or the vote or written consent of 67% of the then outstanding shares of preferred stock.

#### ***Dividends***

Holders of Preferred Stock are entitled to receive, before any cash is paid out or set aside for any Common Stock, cash dividends at a rate of 8% of the original purchase price per share annually (the "Accruing Dividends"). The dividends accrue cumulatively on a daily basis and are payable only when, and if, declared by the Board of Directors or upon liquidation or redemption.

In addition, the holders of Preferred Stock are entitled to additional dividends based on dividends declared to common stockholders, thereby giving the preferred stockholders the right to participate in undistributed earnings of the Company above the stated per share dividend rate. The preferred stockholders do not have a contractual obligation to share in the losses of the Company

No dividends have been declared since the Company's inception. Aggregate cumulative preferred dividends on Preferred Stock at September 30, 2015 were \$7,448,000.

#### ***Redemption***

The Preferred Stock is redeemable at the option of the holder after the redemption date of February 2021. The redemption value of the Preferred Stock is equal to \$3.00 per share for Series B Preferred Stock and \$1.00 per share for Series A Preferred Stock plus any accrued but unpaid dividends. Accordingly, the Preferred Stock is being accreted to redemption value through its redemption date, including accruals for cumulative dividend rights. If the initial carrying value exceeds the redemption value the carrying value is not adjusted.

#### ***Liquidation Preference***

Holders of Series B Preferred Stock and Series A Preferred Stock have preference to the assets of the Company in the event of any voluntary or involuntary liquidation, dissolution or winding-up of the Company, equal to \$3.00 per share for Series B Preferred Stock and \$1.00 per share for Series A Preferred Stock, plus any accrued but unpaid

dividends, whether or not declared, plus any dividends declared but unpaid thereon, on a pari passu basis. After the payment of the preference amounts to the holders of Series B Preferred Stock and Series A Preferred Stock, the remaining assets of the Company are to be distributed among the holders of Series A Preferred Stock and holders of Common Stock on a pro rata basis. However, if the aggregate amount which the holders of Series A Preferred Stock would be entitled to receive exceeds \$2.50 per share (subject to appropriate adjustment in the event of any stock dividend, stock split, combination, reclassification or other similar event) (the "Maximum Participant Amount"), each holder of Series A Preferred Stock will receive the greater of the Maximum Participant Amount or the amount such holder would have received if all shares of Series A Preferred Stock had been converted into Common Stock immediately prior to such liquidation.

### ***Voting Rights***

Holders of Series A Preferred Stock and Series B Preferred Stock are entitled to vote as a single class with the holders of Common Stock on all matters submitted for vote to the Stockholders of the Company. The holders of Preferred Stock are entitled to one vote for each equivalent common share on an as-converted basis. In addition, the holders of Series A Preferred Stock are entitled to elect two (2) directors. The remaining directors shall be elected by the holders of Common Stock voting together with the holders of the Series B Preferred Stock as one class on an as-converted basis.

The holders of Series A Preferred Stock and Series B Preferred Stock have certain protective rights as defined. These protective rights require the Required Vote before action can be taken to (i) increase or decrease the number of shares of Series A Preferred Stock or Series B Preferred Stock that the Company has authority to issue, (ii) change the par value of the Series A Preferred Stock or Series B Preferred Stock, (iii) amend the Certificate of Incorporation in any way that adversely affects the holders of the Series A Preferred Stock or Series B Preferred Stock.

## **9. Common Stock**

As of December 31, 2014, and September 30, 2015, the Company had authorized 65,000,000 and 95,000,000 shares of Common Stock, respectively at \$0.001 par value per share.

### ***General***

The voting, dividend and liquidation rights of the holders of the Common Stock are subject to and qualified by the rights, powers and preferences of the holders of Preferred Stock. The Common Stock has the following characteristics:

### ***Voting***

The holders of shares of Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders and written actions in lieu of meetings.

### ***Dividends***

The holders of shares of Common Stock are entitled to receive dividends, if and when declared by the Board of Directors. Cash dividends may not be declared or paid to holders of shares of Common Stock until all accrued unpaid dividends on Series A Preferred Stock and Series B Preferred Stock have been paid in accordance with their terms. No dividends have been declared or paid by the Company since its inception.

### ***Liquidation***

After payment to of their respective liquidation preferences to the holders of shares of Series A Preferred Stock and Series B Preferred Stock, the holders of shares of Common Stock are entitled to share ratably in the Company's remaining assets available for distribution to its stockholders in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon occurrence of a deemed liquidation event.



**Shares Reserved For Future Issuance**

	<u>As of</u> <u>September 30, 2015</u>	<u>As of December 31,</u> <u>2014</u>
Shares reserved for Series A Preferred Stock outstanding	10,588,235	5,882,352
Shares reserved for future issuances of Series A Preferred Stock	—	4,705,883
Shares reserved for Series B Preferred Stock outstanding	7,058,819	—
Shares reserved for vesting of restricted stock awards under the Founder Agreements	889,715	1,068,383
Shares reserved for vesting of restricted stock awards under the 2014 Option and Stock Plan	1,123,425	1,510,434
Shares reserved for exercise of stock options	852,249	—
Shares reserved for issuances under the 2014 Stock Option and Grant Plan	92,534	291,052
	<u>20,604,977</u>	<u>13,458,104</u>

**10. Stock-Based Compensation****2014 Stock Option and Grant Plan**

In January 2014 the Company adopted the Voyager Therapeutics, Inc. 2014 Stock Option and Grant Plan (the “2014 Plan”), under which it may grant incentive stock options, non-qualified stock options, restricted stock awards, unrestricted stock awards, or restricted stock units to purchase up to 823,529 shares of Common Stock to employees, officers, directors and consultants of the Company.

In April 2014 the Company amended the Plan to allow for the issuance of up to 1,411,764 shares of Common Stock. In August 2014, April 2015 and August 2015 the Company further amended the Plan to allow for the issuance of up to 2,000,000, 2,047,058 and 2,669,411 shares of Common Stock, respectively. During the year ended December 31, 2014, the Company issued only restricted stock awards under the Plan and during the nine months ended September 30, 2015 the Company only granted stock options.

The terms of stock awards agreements, including vesting requirements, are determined by the Board of Directors and are subject to the provisions of the 2014 Plan. Restricted Stock awards granted by the Company generally vest based on each grantee’s continued service with the Company during a specified period following grant. Awards granted to employees generally vest over four years, with 25% vesting on the one year anniversary and 75% vesting ratably, on a monthly basis, over the remaining three years. Awards granted to non-employee consultants generally vest monthly over a period of one to four years.

During the year ended December 31, 2014, the Company granted a total of 1,597,988 shares of restricted stock to employees and 110,960 shares of restricted stock to non-employee consultants at an original issuance price of \$0.04 per share. During the nine months ended September 30, 2015 the Company issued 794,309 stock options to employees and directors and 60,382 stock options to non-employees. As of September 30, 2015, there were 92,534 shares available for future issuance under the 2014 Plan.

**Founder Awards**

In January 2014 the Company issued 1,188,233 shares of restricted stock to its Founders at an original issuance price of \$0.0425 per share. Of the total restricted shares awarded to the Founders, 835,292 shares generally vest over one to four years, based on each Founder’s continued service to the Company in varying capacity as a Scientific Advisory Board member, consultant, director, officer or employee, as set forth in each grantee’s individual restricted stock purchase agreement. The remaining 352,941 of the shares issued will begin vesting upon the achievement of certain performance objectives as well as continued service to the Company, as set forth in the agreements.

These performance conditions are tied to certain milestone events specific to the Company’s corporate goals, including but not limited to preclinical and clinical development milestones related to the Company’s product candidates. Stock-based compensation expense associated with these performance-based awards will be recognized when the

achievement of the performance condition is considered probable, using management's best estimates. As of December 31, 2014 and September 30, 2015, management has concluded that achievement of such performance-based milestones was not probable. Accordingly, no stock-based compensation expense was recorded as of December 31, 2014 and September 30, 2015 related to these awards.

**Stock-Based Compensation Expense**

Total compensation cost recognized for all stock-based compensation awards in the statements of operations and comprehensive loss is as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	2015	2014	2015
Research and development	\$ 134	\$ 627	\$ 212	\$ 1,486
General and administrative	34	230	63	575
Total stock-compensation expense	\$ 168	\$ 857	\$ 275	\$ 2,061

**Restricted Stock**

A summary of the status of and changes in unvested restricted stock as of December 31, 2014 and September 30, 2015 was as follows:

	Shares	Weighted Average Grant Date Fair Value Per Share
<b>Unvested restricted common stock as of December 31, 2014</b>	2,578,817	\$ 0.77
Issued	—	
Vested	(553,605)	\$ 0.73
Repurchased	(12,072)	\$ 0.86
<b>Unvested restricted common stock as of September 30, 2015</b>	<b>2,013,140</b>	<b>\$ 0.77</b>

The expense related to awards granted to employees and non-employees was \$134,000 and \$457,000, respectively, for the three months ended September 30, 2015. The expense related to awards granted to employees and non-employees was \$325,000 and \$1,119,000, respectively, for the nine months ended September 30, 2015.

As of September 30, 2015, the Company had unrecognized stock-based compensation expense related to its unvested restricted stock awards of \$5,064,000, which is expected to be recognized over the remaining weighted average vesting period of 2.39 years.

The aggregate fair value of restricted stock awards vested during the year ended December 31, 2014 and the nine months ended September 30, 2015, based on estimated fair values of the stock underlying the restricted stock awards on the day of vesting, was \$253,000 and \$2,658,000 respectively.

### Stock Options

A summary of the status of, and changes in, stock options as of December 31, 2014 and September 30, 2015 was as follows:

	Shares	Weighted Average Exercise Price	Remaining Contractual Life (in years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2014	—			
Granted	854,691	\$ 7.91		
Exercised	(1,276)	\$ 7.27		
Cancelled or forfeited	(1,166)	\$ 7.27		
Outstanding at September 30, 2015	<u>852,249</u>	\$ 7.91	9.8	\$ 1,338
Exercisable at September 30, 2015	<u>71,238</u>	\$ 7.35	9.6	\$ 151
Vested and expected to vest at September 30, 2015	<u>852,249</u>	\$ 7.91	9.8	\$ 1,338

Using the Black-Scholes option pricing model, the weighted average fair value of options granted to employees and directors during the three and nine months ended September 30, 2015 was \$5.66 and \$5.39, respectively, and the weighted average fair value to non-employees was \$6.90 and \$6.23, respectively. The expense related to awards granted to employees and directors was \$215,000 and \$352,000, for the three and nine months ended September 30, 2015, respectively. There were no stock options granted during the period and year ended December 31, 2014.

The fair value of each option issued to employees and directors was estimated at the date of grant using the Black-Scholes option pricing model with the following weighted-average assumptions:

	Three Months Ended September 30, 2015	Nine Months Ended September 30, 2015
Risk-free interest rate	1.7 %	1.6 %
Expected dividend yield	—	—
Expected term (in years)	6.0	6.0
Expected volatility	78.4 %	78.6 %

Using the Black-Scholes option pricing model, the weighted average fair value of options granted to non-employees during the nine months ended September 30, 2015 was \$6.23. Unvested options granted to non-employees are revalued at each measurement period until they vest. The expense related to awards granted to non-employees was \$49,000 and \$263,000 for the three and nine months ended September 30, 2015, respectively. There were no stock options granted during the period and year ended December 31, 2014.

The fair value of each option issued to non-employees was estimated at each vesting and reporting date using the Black-Scholes option pricing model. The reporting date fair value was determined using the following weighted-average assumptions:

	As of September 30, 2015
Risk-free interest rate	2.2 %
Expected dividend yield	—
Expected term (in years)	10.0
Expected volatility	82.6 %

As of September 30, 2015, the Company had unrecognized stock-based compensation expense related to its unvested stock options of \$4,084,000 which is expected to be recognized over the remaining weighted average vesting period of 3.55 years.

#### **11. 401(k) Savings Plan**

The Company has a defined-contribution savings plan under Section 401(k) of the Internal Revenue Code (the “401(k) Plan”). The 401(k) Plan covers all employees who meet defined minimum age and service requirements, and allows participants to defer a portion of their annual compensation on a pretax basis. As currently established, the Company is not required to make and has not made any contributions to the 401(k) Plan to date.

#### **12. Related-Party Transactions**

Since inception, the Company received consulting and management services from one of its investors. In January 2014, the Company issued 470,589 shares of common stock as partial compensation for these services. The fair value of the shares was approximately \$238,000.

The total amount of consulting and management services provided by this investor was approximately \$117,000 and \$6,000, \$1,063,000 and \$115,000 during the three and nine months ended September 30, 2014 and 2015, respectively. As of September 30, 2015, the Company included approximately \$6,000, in accounts payable related to service fees charged by this investor.

During the nine month period ended September 30, 2015 the Company recognized \$12,397,000 of revenue associated with its collaboration with Genzyme related to research and development services provided during this period. The company also recognized \$2.2 million of expense during the nine months ended September 30, 2015 related to in-kind services provided by Genzyme associated with the collaboration arrangement.

#### **13. Subsequent Events**

##### ***2014 Stock Option and Grant Plan***

In October 2015, the Company amended the 2014 Plan to increase the number of shares reserved for issuance to 2,998,823 shares.

##### ***2015 Stock Option Plan***

In October 2015, the Company’s board of directors and stockholders approved the 2015 Stock Option and Incentive Plan, or 2015 Stock Option Plan, which became effective upon the completion of the IPO. The 2015 Stock Option Plan provides the Company with the flexibility to use various equity-based incentive and other awards as compensation tools to motivate our workforce. These tools include stock options, stock appreciation rights, restricted stock, restricted stock units, unrestricted stock, performance share awards and cash-based awards. The 2015 Stock Option Plan replaced the 2014 Plan. Any options or awards outstanding under the 2014 Stock Option Plan remained outstanding and effective. The number of shares initially reserved for issuance under the 2015 Stock Option Plan is the sum of (i) 1,311,812 shares of common stock and (ii) the number of shares under the 2014 Plan that are not needed to

fulfill the Company's obligations for awards issued under the 2014 Plan as a result of forfeiture, expiration, cancellation, termination or net issuances of awards thereunder. The number of shares of common stock that may be issued under the 2015 Stock Option Plan is also subject to increase on the first day of each fiscal year by up to 4% of the Company's issued and outstanding shares of common stock on the immediately preceding December 31.

#### ***2015 Employee Stock Purchase Plan***

In October 2015 the Company's board of directors and stockholders approved the 2015 Employee Stock Purchase Plan. A total of 262,362 shares of common stock were initially authorized for issuance under this plan. The 2015 Employee Stock Purchase Plan became effective upon the completion of the IPO.

#### ***Initial Public Offering***

On November 16, 2015 the Company completed the sale of 5,750,000 shares of its common stock in its IPO, at a price to the public of \$14.00 per share, resulting in net proceeds to the Company of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company.

On October 29, 2015, in preparation for the Company's IPO, the Company's board of directors and stockholders approved a 1-for-4.25 reverse split of the Company's common stock, which became effective on October 29, 2015. All share and per share amounts in the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to this reverse split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital.

In connection with the closing of the IPO, all of the Company's outstanding redeemable convertible preferred stock automatically converted into shares of common stock as of November 16, 2015, resulting in the issuance by the Company of an additional 17,647,054 shares of common stock.

## ITEM 2. MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS

*The following discussion and analysis of our financial condition and results of operations should be read in conjunction with our unaudited condensed financial statements and related notes appearing elsewhere in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our final prospectus for our initial public offering filed pursuant to Rule 424(b) under the Securities Act of 1933, as amended (the "Securities Act"), with the Securities and Exchange Commission (the "SEC") on November 12, 2015 (the "Prospectus").*

*Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this Quarterly Report. In addition, even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this Quarterly Report, they may not be predictive of results or developments in future periods.*

*The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in the Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.*

*We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.*

We are a clinical-stage gene therapy company focused on developing life-changing treatments for patients suffering from severe diseases of the central nervous system, or CNS. We focus on CNS diseases where we believe an adeno-associated virus, or AAV, gene therapy approach that either increases or decreases the production of a specific protein can slow or reduce the symptoms experienced by patients, and therefore have a clinically meaningful impact. We have created a product engine, which enables us to engineer, optimize, manufacture and deliver our AAV-based gene therapies that have the potential to provide durable efficacy following a single administration directly to the CNS. Our product engine has rapidly generated programs for five CNS indications, including advanced Parkinson's disease; a monogenic form of ALS; Friedreich's ataxia; Huntington's disease; and spinal muscular atrophy. Our most advanced clinical candidate, VY-AADC01, is being evaluated for the treatment of advanced Parkinson's disease in an open-label, Phase 1b clinical trial with the goal of generating human proof-of-concept data in the second half of 2016. Our goal is to submit an IND for one of our current preclinical programs in 2017.

Since our inception on June 19, 2013, our operations have focused on organizing and staffing our company, business planning, raising capital, establishing our intellectual property portfolio, determining which CNS indications to pursue and conducting preclinical studies and clinical trials. We do not have any product candidates approved for sale and have not generated any revenue from product sales. We have funded our operations primarily through private placements of our Series A redeemable convertible preferred stock, which we refer to as our Series A Preferred Stock, and Series B redeemable convertible preferred stock, which we refer to as our Series B Preferred Stock and together with our Series A Preferred Stock, our redeemable convertible preferred stock, and our collaboration with Genzyme, or the Genzyme Collaboration, which commenced in February 2015. From inception through September 30, 2015, we have raised an aggregate of \$135.0 million of gross proceeds from sales of our redeemable convertible preferred stock and convertible promissory notes, and additionally received a \$65.0 million up-front payment from the Genzyme Collaboration to fund our operations.

Since inception, we have incurred significant operating losses. Our net losses were \$16.3 million for the period and year ended December 31, 2014, respectively, and \$22.4 million for the nine months ended September 30, 2015. As

of September 30, 2015, we had an accumulated deficit of \$47.2 million. We expect to continue to incur significant expenses and operating losses for the foreseeable future. We anticipate that our expenses will increase significantly in connection with our ongoing activities, as we:

- continue investing in our product engine to optimize vector engineering, manufacturing and dosing and delivery techniques;
- continue development of our clinical candidate, VY-AADC01;
- initiate additional preclinical studies and clinical trials for our other programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional CNS diseases for treatment with our AAV gene therapies;
- seek marketing approvals for VY-AADC01 or other product candidates that arise from our programs that successfully complete clinical trials;
- develop a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio; and
- identify, acquire or in-license other product candidates and technologies.

On November 16, 2015 we closed our IPO whereby we sold 5,750,000 shares of common stock, at a public offering price of \$14.00 per share, including 750,000 shares of common stock issued upon the full exercise by the underwriters of their option to purchase additional shares, resulting in net proceeds to us of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

## **Financial Operations Overview**

### ***Revenue***

To date, we have not generated any revenue from product sales and do not expect to generate any revenue from product sales for the foreseeable future. For the nine months ended September 30, 2015, we recognized \$12.4 million of collaboration revenue from the Genzyme Collaboration. For additional information about our revenue recognition policy related to the Genzyme Collaboration, see the section titled “—Critical Accounting Policies and Estimates—Revenue.”

For the foreseeable future, we expect substantially all of our revenue will be generated from the Genzyme Collaboration, and any other strategic relationships we may enter into. If our development efforts are successful, we may also generate revenue from product sales.

### ***Expenses***

#### ***Research and Development Expenses***

Research and development expenses consist primarily of costs incurred for our research activities, including our program discovery efforts, and the development of our programs and product engine, which include:

- employee-related expenses including salaries, benefits and stock-based compensation expense;

- costs of funding research performed by third parties that conduct research and development, preclinical activities, manufacturing and production design on our behalf;
- the cost of purchasing lab supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials;
- consultant fees;
- facility costs including rent, depreciation and maintenance expenses; and
- fees for maintaining licenses under our third-party licensing agreements.

Research and development costs are expensed as incurred. Costs for certain activities, such as manufacturing and preclinical studies and clinical trials, are generally recognized based on an evaluation of the progress to completion of specific tasks using information and data provided to us by our vendors and collaborators.

At this time, we cannot reasonably estimate or know the nature, timing and estimated costs of the efforts that will be necessary to complete the development of our product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from sales of our product candidates. This is due to the numerous risks and uncertainties associated with developing such product candidates, including the uncertainty of:

- successful enrollment in and completion of clinical trials;
- establishing an appropriate safety profile;
- establishing commercial manufacturing capabilities or making arrangements with third-party manufacturers;
- receipt of marketing approvals from applicable regulatory authorities;
- commercializing the product candidates, if and when approved, whether alone or in collaboration with others;
- obtaining and maintaining patent and trade secret protection and regulatory exclusivity for our product candidates;
- continued acceptable safety profiles of the products following approval; and
- retention of key research and development personnel.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs, timing and viability associated with the development of that product candidate.

Research and development activities are central to our business model. We expect research and development costs to increase significantly for the foreseeable future as our development programs progress, including as we continue to support the Phase 1b clinical trial of VY-AADC01 as a treatment for advanced Parkinson's disease, and move such product candidates into additional clinical trials. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.



### General and Administrative Expenses

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development, legal and human resource functions. Other significant costs include corporate facility costs not otherwise included in research and development expenses, legal fees related to patent and corporate matters and fees for accounting and consulting services.

We anticipate that our general and administrative expenses will increase in the future to support continued research and development activities, including the continuation of the Phase 1b clinical trial of VY-AADC01 and the initiation of our clinical trials for our other product candidates. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants. We also anticipate increased expenses associated with being a public company, including costs for audit, legal, regulatory and tax-related services, director and officer insurance premiums and investor relations costs.

### Other Income (Expense)

Other income (expense) consists primarily of the re-measurement losses associated with the change in the fair value of the Series A Preferred Stock tranche rights for the Series A Preferred Stock. \$9.8 million of expense was recorded during the quarter ended March 31, 2015 related to the change in fair value of these rights. In February 2015, upon the issuance of the final tranche of Series A Preferred Stock, the tranche right liability was reclassified to Series A Preferred Stock and no further re-measurement gains or losses will be recognized related to these tranche rights.

### Critical Accounting Policies and Estimates

We have prepared our financial statements in accordance with U.S. generally accepted accounting principles. Our preparation of these financial statements requires us to make estimates, assumptions, and judgments that affect the reported amounts of assets, liabilities, expenses, and related disclosures at the date of the financial statements, as well as revenue and expenses recorded during the reporting periods. We evaluate our 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 could therefore differ materially from these estimates under different assumptions or conditions.

There have been no material changes to our critical accounting policies from those described in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” included in our final prospectus filed with the SEC on November 12, 2015.

## Results of Operations

### Comparison of Three and Nine months Ended September 30, 2015 and 2014

The following table summarizes our results of operations for the nine months ended September 30, 2015 and 2014, together with the changes in those items in dollars (in thousands):

	Three Months Ended September 30,			Nine Months Ended September 30,		
	2015	2014	\$ Change	2015	2014	\$ Change
Collaboration revenue	\$ 4,937	\$ —	\$ 4,937	\$ 12,397	\$ —	\$ (12,397)
Operating expenses:						
Research and development	6,481	2,399	4,082	18,459	5,938	(12,521)
General and administrative	2,475	1,257	1,218	6,752	3,933	(2,819)

Total operating expenses	8,956	3,656	5,300	25,211	9,871	(15,340)
Other expense, net:						
Interest income (expense), net	102	—	102	175	(2)	(177)
Other financing expense	—	(349)	349	(9,750)	(1,754)	7,996
Total other expense, net	102	(349)	451	(9,575)	(1,756)	7,819
Net loss	\$ (3,917)	\$ (4,005)	\$ —	\$ (22,389)	\$ (11,627)	\$ —

#### Revenue

Revenue was \$5.0 million and \$12.4 million for the three and nine months ended September 30, 2015, all of which related to the Genzyme Collaboration. We did not earn any revenue for the three or nine months ended September 30, 2014. In the three and nine months ended September 30, 2015 we recorded \$5.0 million and \$12.4 million in recognition of amounts allocated to research and development services for various programs under the Genzyme Collaboration, which was entered into in February 2015. Generally, the amounts allocated to these programs are expected to be recognized on a straight line basis over the period the services are provided for each program.

#### Research and Development Expense

Research and development expense increased by \$4.1 million from \$2.4 million for the three months ended September 30, 2015 to \$6.5 million and increased by \$12.5 million from \$6.0 million for the nine months ended September 30, 2014 to \$18.5 million for the nine months ended September 30, 2015. The following table summarizes our research and development expenses, for the three and nine months ended September 30, 2014 and September 30, 2015 (in thousands):

	Three Months Ended			Nine Months Ended		
	September 30,		\$ Change	September 30,		\$ Change
	2015	2014		2015	2014	
Employee and contractor related expenses	\$ 2,637	\$ 1,201	\$ 1,436	\$ 6,750	\$ 2,965	\$ 3,785
Process and platform development expenses	3,287	860	2,427	10,161	1,657	8,504
License fees	16	40	(24)	161	862	(701)
Facility expenses	359	198	161	990	252	738
Other expenses	182	100	82	397	202	195
Total research and development expenses	\$ 6,481	\$ 2,399	\$ 4,082	\$ 18,459	\$ 5,938	\$ 12,521

The increase in research and development expense for the three months ended September 30, 2015 was primarily attributable to the following:

- approximately \$2.3 million for increased costs of funding research performed by third parties that conduct research and development, preclinical and clinical activities and manufacturing and production design on our behalf and increased purchases of lab supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials, and an additional expense of approximately \$0.1 million attributable to in-kind research and development services incurred by Genzyme and provided to us under the Genzyme Collaboration;
- approximately \$1.4 million for increased research and development employee compensation costs;
- approximately \$0.2 million for increases in facility costs including rent, depreciation, and maintenance expenses; and

The increase in research and development expense for the nine months ended September 30, 2015 was primarily attributable to the following:

- approximately \$8.4 million for increased costs of funding research performed by third parties that conduct research and development, preclinical and clinical activities and manufacturing and production design on our behalf and increased purchases of lab supplies and non-capital equipment used in designing, developing and manufacturing preclinical study materials, and an additional expense of approximately \$0.1 million attributable to in-kind research and development services incurred by Genzyme and provided to us under the Genzyme Collaboration;
- approximately \$3.9 million for increased research and development employee compensation costs, which were partially offset by a \$0.1 million decrease in contractor and consulting fees and expenses;
- approximately \$0.7 million for increases in facility costs including rent, depreciation, and maintenance expenses; and
- a decrease of approximately \$0.7 million related to lower licensing costs.

#### *General and Administrative Expense*

General and administrative expense increased by \$1.2 million from \$1.3 million for the three months ended September 30, 2014 to \$2.5 million for the three months ended September 30, 2015. The increase in general and administrative expense was primarily attributable to the following:

- approximately \$0.5 million related to the increase in administrative function headcount;
- approximately \$0.4 million for increased consulting and professional services; and
- approximately \$0.2 million increase in the patent related legal fees.

General and administrative expense increased by \$2.8 million from \$3.9 million for nine months ended September 30, 2014 to \$6.8 million for nine months ended September 30, 2015. The increase in general and administrative expense was primarily attributable to the following:

- approximately \$1.4 million for increased consulting and professional services;
- approximately \$1.1 million related to the increase in administrative function headcount; and
- approximately \$0.3 million for costs related to renting and operating our corporate offices.

#### *Other Expense, Net*

Other expense increased by \$0.4 million from \$0.3 million in net expense for the three months ended September 30, 2014 to \$0.1 million in net income for the three months ended September 30, 2015, primarily related to the \$0.3 million mark to market adjustments recorded on our Series A Preferred Stock Tranche Rights liability in the three months ended September 30, 2014. Other expense increased by \$8.0 million from \$1.8 million for the nine months ended September 30, 2014 to \$9.8 million for the nine months ended September 30, 2015. The increase in expense primarily related to the mark to market adjustments recorded on our Series A Preferred Stock Tranche Rights liability during the nine months ended September 30, 2015. The increase in value of the Series A Preferred Stock Tranche Rights liability was a result of the increase in the fair value of our Series A Preferred Stock and the increase in the probability of closing the tranche during the nine months ended September 30, 2015. The Series A Preferred Stock Tranche Rights liability was settled in February 2015 upon the issuance of the final tranche of Series A Preferred Stock.

## Liquidity and Capital Resources

### Sources of Liquidity

Prior to our IPO, we had funded our operations primarily through proceeds from private placements of our redeemable convertible preferred stock and convertible promissory notes of \$135.0 million and proceeds associated with an up-front payment from the Genzyme Collaboration of \$65.0 million.

On November 16, 2015 we closed our IPO whereby we sold 5,750,000 shares of common stock, at a public offering price of \$14.00 per share, including 750,000 shares of common stock issued upon the full exercise by the underwriters of their option to purchase additional shares, resulting in net proceeds to us of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

As of September 30, 2015, we had cash, cash equivalents and marketable securities of \$160.6 million.

### Cash Flows

The following table provides information regarding our cash flows for the nine months ended September 30, 2015 and September 30, 2014 (in thousands):

	Nine Months Ended September 30,	
	2015	2014
Net cash (used in) provided by:		
Operating activities	\$ 50,856	\$ (7,126)
Investing activities	(95,192)	(2,293)
Financing activities	103,834	15,614
Net increase in cash and cash equivalents	<u>\$ 59,498</u>	<u>\$ 6,195</u>

#### Net Cash Provided by (Used in) Operating Activities

The use of cash in all periods resulted primarily from our net losses adjusted for non-cash charges and changes in components of working capital.

Net cash provided by operating activities was \$50.9 million during the nine months ended September 30, 2015 compared to \$7.1 million of cash used in operating activities during the nine months ended September 30, 2014. The increase in cash provided by operating activities was primarily due to the receipt of the \$65.0 million upfront payment from Genzyme under the Genzyme Collaboration.

#### Net Cash Used in Investing Activities

Net cash used in investing activities was \$95.2 million during the nine months ended September 30, 2015 compared to \$2.3 million during the nine months ended September 30, 2014. The cash used for investing activities for the nine months ended September 30, 2015 was primarily due to purchases of marketable securities partially offset by proceeds from maturities of marketable securities.

#### Net Cash Provided by Financing Activities

Net cash provided by financing activities was \$103.8 million during the nine months ended September 30, 2015 compared to \$15.6 million during the nine months ended September 30, 2014. The increase in cash provided by

financing activities was primarily due to the issuance of \$20.0 million of Series A Preferred Stock and \$90.0 million of Series B Preferred Stock during the nine months ended September 30, 2015, of which \$5.0 million in proceeds were in excess of the Series B Preferred Stock's fair value and were allocated to deferred revenue.

#### *Funding Requirements*

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, continue or initiate clinical trials of, and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to program sales, marketing, manufacturing and distribution to the extent that such sales, marketing and distribution are not the responsibility of potential collaborators. 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 our research and development programs or future commercialization efforts.

We expect our existing cash, cash equivalents and marketable securities will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of product discovery, preclinical studies and clinical trials for our product candidates;
- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under the Genzyme Collaboration and any other collaboration agreements we obtain;
- the ability of our collaboration partners to exercise options to extend research and development programs
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies and clinical trials is a time-consuming, expensive and uncertain process that takes many years to complete, and we may never generate the necessary data or results required to obtain marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Our commercial revenues, if any, will be derived from sales of gene therapies that we do not expect to be commercially available for many years, if at all. Accordingly, we will

need to continue to rely on additional financing to achieve our business objectives. Adequate additional financing may not be available to us on acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or redeemable convertible debt securities, your ownership interest will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends.

If we raise funds through additional collaborations, strategic alliances or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or to grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves.

### Contractual Obligations

The following table summarizes our significant contractual obligations as of payment due date by period at December 31, 2014 (in thousands):

	Total	Less Than			More than
		1 Year	1 to 3 Years	3 to 5 Years	5 Years
Operating lease commitments <sup>(1)</sup>	\$ 5,877	\$ 1,117	\$ 3,576	\$ 1,184	\$ —

- (1) We lease office space at 75 Sidney Street in Cambridge, Massachusetts under a non-cancelable operating lease that expires in December 2019.

We enter into agreements in the normal course of business with CROs and institutions to license intellectual property. We have not included these future payments in the table of contractual obligations above since the contracts are cancelable at any time by us, generally upon 30 to 90 days prior written notice.

Our agreements to license intellectual property include potential milestone payments that are dependent upon the development of products using the intellectual property licensed under the agreements and contingent upon the achievement of clinical trial or regulatory approval milestones. The maximum aggregate potential milestone payments payable by us total approximately \$12.0 million. Additionally, under the terms of one agreement, we have options to license intellectual property to be used in the development of therapies for four disease indications. If we exercise all of the options under the agreement, we would be obligated to pay aggregate up-front fees of up to approximately \$1.5 million and milestone payments that are contingent upon clinical trial results and regulatory approval of \$5.0 million per disease indication, or up to \$20.0 million in total. We may also be required to pay annual maintenance fees or minimum amounts payable ranging from low-four digits to low five-digits depending upon the terms of the applicable agreement.

### Off-Balance Sheet Arrangements

We did not have, during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined under applicable Securities and Exchange Commission rules.

### JOBS Act

In April 2012, the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an emerging growth company, or EGC, can take advantage of the extended transition period provided in Section 7(a)(2)(B) of the Securities Act of 1933, as amended, or the Securities Act, for complying with new or revised accounting standards. Thus, an EGC can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected

not to avail ourselves of this extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements under the JOBS Act. Subject to certain conditions, as an EGC, we intend to rely on certain of these exemptions, including without limitation, (i) providing an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and (ii) complying with any requirement that may be adopted by the Public Company Accounting Oversight Board, or PCAOB, regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, known as the auditor discussion and analysis. We will remain an EGC until the earlier of (i) the last day of the fiscal year in which we have total annual gross revenues of \$1.0 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (iii) the date on which we have issued more than \$1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission.

### **ITEM 3. QUANTITATIVE AND QUALITATIVE DISCLOSURES ABOUT MARKET RISK**

We are exposed to market risk related to changes in interest rates. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates, particularly because our investments, including cash equivalents, are in the form of money market fund and marketable securities and are invested in U.S. Treasury and U.S. government agency obligations.

We are not currently exposed to market risk related to changes in foreign currency exchange rates; however, we may contract with vendors that are located in Asia and Europe in the future and may be subject to fluctuations in foreign currency rates at that time.

Inflation generally affects us by increasing our cost of labor and clinical trial costs. We do not believe that inflation had a material effect on our business, financial condition or results of operations during the period and year ended December 31, 2014 and the nine months ended September 30, 2014 and September 30, 2015, respectively.

### **ITEM 4. CONTROLS AND PROCEDURES**

#### **Management's Evaluation of Disclosure Controls and Procedures**

We maintain disclosure controls and procedures (as defined in Rules 13a-15(e) or 15d-15(e) under the Securities Exchange Act of 1934, as amended, or the Exchange Act) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

As of September 30, 2015, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this report. In designing and evaluating our disclosure controls and procedures, our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2015, our disclosure controls and procedures were effective at the reasonable assurance level.

We continue to review and document our disclosure controls and procedures, including our internal controls and procedures for financial reporting, and may from time to time make changes aimed at enhancing their effectiveness and to ensure that our systems evolve with our business.

***Changes in Internal Control over Financial Reporting***

During the three months ended September 30, 2015, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Securities Exchange Act of 1934, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.



## PART II. OTHER INFORMATION

### ITEM 1. LEGAL PROCEEDINGS

As of the date of this Quarterly Report on Form 10-Q, we were not party to any legal matters or claims. In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

### ITEM 1A. RISK FACTORS

*Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this Quarterly Report on Form 10-Q and in our other public filings before making an investment decision. Our business, prospects, financial condition, or operating results could be harmed by any of these risks, as well as other risks not currently known to us or that we currently consider immaterial. If any such risks or uncertainties actually occur, our business, financial condition or operating results could differ materially from the plans, projections and other forward-looking statements included in the section titled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere in this Quarterly Report on Form 10-Q and in our other public filings. The trading price of our common stock could decline due to any of these risks, and as a result, you may lose all or part of your investment.*

#### Risks Related to Our Financial Position and Need for Capital

***We have incurred net losses since inception and anticipate that we will continue to incur losses for the foreseeable future and may never achieve or maintain profitability.***

We are a clinical-stage biotechnology company with a limited operating history, and have not yet generated revenues from the sales of our product candidates. Investment in biotechnology companies is highly speculative because it entails substantial upfront capital expenditures and significant risk that the product candidate will fail to obtain regulatory approval or become commercially viable. We have not yet demonstrated the ability to complete any clinical trials of our product candidates, obtain marketing approvals, manufacture a commercial-scale product or conduct sales and marketing activities necessary for successful commercialization. We continue to incur significant expenses related to research and development, and other operations in order to commercialize our product candidates. As a result, we are not and have never been profitable and have incurred losses since our inception. Our net loss was \$18.5 million and \$16.3 million for the nine months ended September 30, 2015 and year ended December 31, 2014, respectively. As of September 30, 2015, we had an accumulated deficit of \$41.5 million.

We historically have financed our operations primarily through private placements of our redeemable convertible preferred stock and our recent collaboration agreement with Genzyme. On November 16, 2015 we closed our IPO whereby we sold 5,750,000 shares of common stock at a public offering price of \$14.00 per share, including 750,000 shares of common stock issued upon the full exercise by the underwriters of their option to purchase additional shares, resulting in net proceeds to us of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us. To date, we have devoted substantially all of our financial resources to building our product engine, selecting product programs, conducting research and development, including preclinical development of our product candidates, building our intellectual property portfolio, building our team and establishing our collaboration with Genzyme. We expect that it could be several years, if ever, before we have a commercialized product candidate. 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:

- continue investing in our product engine to optimize vector engineering, manufacturing and dosing and delivery techniques;
- continue development of our clinical candidate, VY-AADC01;

- initiate additional preclinical studies and clinical trials for our other programs;
- continue our process research and development activities, as well as establish our research-grade and commercial manufacturing capabilities;
- identify additional CNS diseases for treatment with our AAV gene therapies;
- seek marketing approvals for VY-AADC01 or other product candidates that arise from our programs that successfully complete clinical trials;
- develop a sales, marketing and distribution infrastructure to commercialize any product candidates for which we may obtain marketing approval;
- maintain, expand and protect our intellectual property portfolio; and
- identify, acquire or in-license other product candidates and technologies.

To become and remain profitable, we must develop and eventually commercialize product candidates with significant market potential, which will require us to be successful in a range of challenging activities. These activities can include completing preclinical studies and clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products that are approved 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 significant or large enough 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 not be able to generate sufficient revenue from the commercialization of our product candidates and may never be profitable.***

Our ability to generate revenue and achieve profitability depends on our ability, alone or with our collaborative partners, to successfully complete the development of, and obtain the regulatory approvals necessary to commercialize, our current and future product candidates. Our lead product candidate VY-AADC01 is being evaluated in a Phase 1b clinical trial, and we do not anticipate generating revenues from product sales for the next several years, and we may never succeed in doing so. Our ability to generate future revenues from product sales depends heavily on our and our collaborators' success in:

- completing preclinical and clinical development of our product candidates and identifying new product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical trials;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval by establishing a sales force, marketing and distribution infrastructure or, alternatively, collaborating with a commercialization partner;
- qualifying for adequate coverage and reimbursement by government and third-party payors for our product candidates if and when approved;

- maintaining and enhancing a sustainable, scalable, reproducible and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate, in both amount and quality, products and services to support clinical development and the market demand for our product candidates, if approved;
- obtaining market acceptance of our product candidates as a viable treatment option;
- addressing any competing technological and market developments;
- implementing additional internal systems and infrastructure, as needed;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter and performing our obligations in such collaborations;
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how;
- avoiding and defending against third-party interference or infringement 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 U.S. Food and Drug Administration, or the FDA, European Medicines Agency, or EMA, or other regulatory authorities to perform preclinical studies and clinical trials 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.

***We will need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our product development efforts or other operations.***

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, initiate further clinical trials of and seek marketing approval for, 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. Given the completion of our IPO on November 16, 2015, 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 acceptable terms, we would be forced to delay, reduce or eliminate certain of our research and development programs.

Our operations have consumed significant amounts of cash since inception. As of September 30, 2015, our cash, cash equivalents and marketable securities were \$160.6 million. We expect that our existing cash, cash equivalents and marketable securities, will enable us to fund our operating expenses and capital expenditure requirements for at least the next 24 months.

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

- the scope, progress, results and costs of product discovery, preclinical studies and clinical trials for our product candidates;

- the scope, prioritization and number of our research and development programs;
- the costs, timing and outcome of regulatory review of our product candidates;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the achievement of milestones or occurrence of other developments that trigger payments under the Genzyme Collaboration and any other collaboration agreements we obtain;
- the ability of our collaboration partners to exercise options to extend research and development programs
- the extent to which we are obligated to reimburse, or entitled to reimbursement of, clinical trial costs under collaboration agreements, if any;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- the extent to which we acquire or in-license other product candidates and technologies;
- the costs of securing manufacturing arrangements for commercial production; and
- the costs of establishing or contracting for sales and marketing capabilities if we obtain regulatory approvals to market our product candidates.

Identifying potential product candidates and conducting preclinical studies 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 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 collaboration agreements, will be derived from or based on sales of products that may not be commercially available for many years, if at all. 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. To the extent that additional capital is raised through the issuance of debt, the agreement governing such debt may contain restrictive covenants related to our capital raising and other financial and operational matters, which may make it more difficult for us to obtain additional capital and to pursue business operations, including potential acquisitions. Furthermore, the issuance of additional securities, whether equity or debt, by us, or the possibility of such issuance, may cause the market price of our common stock to decline and our existing stockholders may not agree with the terms of such financings. Adequate additional financing may not be available to us on acceptable terms, or at all.

#### **Risks Related to the Development and Regulatory Approval of Our Product Candidates**

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

We have concentrated our research and development efforts to date on our product engine, identifying our initial targeted disease indications, and our initial product candidates, and our future success depends on our successful development of viable AAV gene therapy product candidates. Currently, only one of our product candidates, VY-AADC01, is in clinical development, and the remainder of our product candidates are in preclinical development. There can be no assurance that we will not experience problems or delays in developing our product candidates and 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.

The clinical trial requirements of the FDA, the EMA and other regulatory authorities and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the product candidate. The regulatory approval process for novel product candidates such as gene therapies can be more expensive and take longer than for other, better known or more extensively studied product candidates. Only one gene therapy product, uniQure N.V.'s, or uniQure, Glybera, has received marketing authorization from the European Commission and no gene therapy products have received marketing authorization in the United States. It is difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or the European Union or how long it will take to commercialize our product candidates. Approvals by the European Commission may not be indicative of what the FDA may require for approval and different or additional pre-clinical studies or clinical trials may be required to support regulatory approval in each respective jurisdiction. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product candidate to market could decrease our ability to generate sufficient product revenue, and our business, financial condition, results of operations and prospects may be harmed.

***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, or 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 National Institute of Health, or NIH, are also potentially subject to review by the NIH Office of Biotechnology Activities' Recombinant DNA Advisory Committee, or the RAC. The ongoing Phase 1b clinical trial of VY-AADC01 is being conducted at UCSF and therefore is subject to oversight by these authorities. Even though 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 permitted its initiation. Conversely, the FDA may place an IND on a clinical hold even if the RAC has provided a favorable review or an exemption from in-depth, public review. In addition, NIH-funded institutions need to have their institutional biosafety committee as well as their institutional review board, or IRB, review proposed clinical trials to assess the safety of the trial. The ongoing Phase 1b clinical trial of VY-AADC01 has been reviewed by the University of California San Francisco Institutional Review Board, or UCSF IRB, and such trial will need to be re-reviewed by the UCSF IRB if the protocol for the trial is further amended. In addition, adverse developments in clinical trials of gene therapy products conducted by us or others may cause the FDA or other oversight bodies to change the requirements for approval of any of our product candidates. Similarly, EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines. The EMA and agencies at both the federal and state level in the United States have expressed an interest in further regulating new biotechnologies, including gene therapy.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these 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. Delays as a result of increased or lengthier regulatory approval process and further restrictions on development of our product candidates can be costly and could negatively impact our or our collaborators' ability to complete clinical trials and commercialize our current and future product candidates in a timely manner, if at all.

***Positive results from preclinical studies and early-stage clinical trials may not be indicative of efficacy in late-stage clinical trials.***

All of our product candidates are in early stages of development. Study designs and results from previous clinical trials are not necessarily predictive of our future clinical trial designs or results, and initial results may not be confirmed upon full analysis of the complete trial or study data. Our product candidates may fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through preclinical studies and initial clinical trials.

A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage clinical trials. If a larger population of patients does not experience positive results, if these results are not reproducible, or if our products show diminishing activity over time, our products may not receive approval from the EMA or the FDA. Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, we may encounter regulatory delays or rejections as a result of many factors, including changes in regulatory policy during the period of product development. Failure to confirm favorable results from earlier trials by demonstrating the safety and effectiveness of our products in late-stage clinical trials with larger patient populations could harm our business and we may never succeed in commercialization or generating product revenue.

***The dosing and delivery techniques being employed in the ongoing VY-AADC01 Phase 1b clinical trial are different from those used in prior trials, and dosing and delivery must be further optimized in this trial or we may not generate the human proof-of-concept data we seek.***

The clinical trial results of some of our collaborators have been negatively affected by factors that had not been fully anticipated prior to examination of the trial results. For example, the magnitude of the clinical responses seen in the Phase 1 clinical trial of VY-AADC01 conducted by UCSF were similar to placebo effects observed in previous surgical therapies in Parkinson's disease. As a result, we are unable to rely on the results of this Phase 1 trial for an indication of the efficacy of treatment with VY-AADC01. We believe that there is a need to optimize the delivery, dose and volume of infusion of VY-AADC01 to substantially increase the coverage of the putamen, the region of the brain targeted by VY-AADC01, to achieve a clinical benefit. However, we can provide no assurances that we will be able to optimize these parameters and thereby achieve sufficient coverage of the putamen to achieve a clinical benefit.

The ongoing Phase 1b clinical trial of VY-AADC01 incorporates several design features that are different from those used in UCSF's previously completed Phase 1 clinical trial, in an attempt to increase the area of the putamen, particularly the posterior putamen, which receives VY-AADC01 treatment. Larger infusion volumes of VY-AADC01 are being employed along with higher doses of VY-AADC01. In addition, the Clearpoint System, which is manufactured by MRI Interventions, Inc., is being used during the surgical procedure to provide accurate placement of the cannula, or small tube used in the procedure, in the putamen to allow for real-time, intra-operative MRI to assist the physician in visualizing the delivery of VY-AADC01 to the putamen and to avoid specific blood vessels during the surgical procedure, with the goal of reducing the risk of hemorrhages. In the prior Phase 1 clinical trial of VY-AADC01 conducted by UCSF, physicians surgically administered VY-AADC01 without the use of the Clearpoint System, and therefore did not have real-time visualization of treatment delivery.

Due to the nature of the techniques being used in the Phase 1b clinical trial and the numerous variables that can be changed, it is possible that the data generated from this trial may not provide evidence of clinical benefit. For example, physicians may use needles of differing lengths in the infusion procedure, or may use differing infusion speeds or infusion angles. These differences could affect the dose of VY-AADC01 that ultimately reaches the putamen, leading to highly variable results.

Furthermore, we plan to use a slightly modified version of VY-AADC01 in future clinical trials compared to the version of VY-AADC01 used in the prior Phase 1 clinical trial and in the ongoing Phase 1b clinical trial. In order to use the modified version of VY-AADC01 in our future clinical trials, we will need to complete preclinical studies that demonstrate comparability between the current version and the modified version of VY-AADC01. Although we believe the current version of VY-AADC01 to be substantially similar to the modified version, we can provide no assurances that we will be able to successfully complete such preclinical studies and demonstrate comparability.

***We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or we may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.***

Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of the product candidates. Clinical trials are expensive, time-consuming and outcomes are uncertain.

We have very limited experience with clinical trials. To date, we have neither commenced nor completed any clinical trials. The ongoing Phase 1b clinical trial of VY-AADC01 is being conducted by UCSF. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial failure can occur at any stage of testing.

Identifying and qualifying patients to participate in clinical trials of our product candidates is critical to our success. 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 clinical trials in a timely manner. Patient enrollment and trial completion is affected by factors including:

- perceived risks and benefits of AAV gene therapy-based approaches for the treatment of CNS diseases;
- size of the patient population and process for identifying patients;
- design of the trial protocol;
- eligibility and exclusion criteria;
- patients with preexisting antibodies to the vector that preclude their participation in the trial;
- perceived risks and benefits of the product candidate under study;
- availability of competing therapies and clinical trials;
- severity of the disease under investigation;
- availability of genetic testing for potential patients;
- proximity and availability of clinical trial sites for prospective patients;
- ability to obtain and maintain patient consent;

- risk that enrolled patients will drop out before completion of the trial;
- our inability to locate appropriately trained physicians to conduct such clinical trials, which may be particularly difficult for the VY-AADC01 clinical trial, in which we are using the ClearPoint System, which is only available at a small number of academic medical centers in the United States;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

Further, we plan to seek marketing approvals in the United States, the European Union and other jurisdictions, which may require that we conduct clinical trials in foreign countries. 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 clinical research organizations, or 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 AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; 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 treatment.

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 ongoing or planned clinical trials, any of which would harm our business, financial condition, results of operations and prospects.

Other events that may prevent successful or timely completion of 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;
- imposition of a clinical hold by regulatory authorities as a result of a serious adverse event or after an inspection of our clinical trial operations or trial sites;
- failure by us, any CROs we engage or any other third parties to adhere to clinical trial requirements;
- failure to perform in accordance with the FDA's good clinical practices, or GCP, or applicable regulatory guidelines in the European Union;
- failure by physicians to adhere to delivery protocols leading to variable results;



- 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 patients complete participation in a trial or return for post-treatment follow-up;
- clinical trial sites or patients dropping out of a trial;
- selection of clinical endpoints that require prolonged periods of clinical observation or analysis of the resulting data;
- occurrence of serious adverse events associated with the product candidate that are viewed to outweigh its potential benefits;
- occurrence of serious adverse events in trials of the same class of agents conducted by other sponsors; or
- changes in regulatory requirements and guidance that require amending or submitting new clinical protocols.

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

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

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

***Our product candidates or the process for administering our product candidates may cause undesirable side effects or have other properties that could delay or prevent their regulatory approval, limit their commercial potential or result in significant negative consequences following any potential marketing approval.***

In past clinical trials that were conducted by others with non-AAV vectors, several significant side effects were caused by gene therapy treatments, including reported cases of leukemia and death. Other potential side effects could include an immunologic reaction and insertional oncogenesis, which is the process whereby the insertion of a functional gene near a gene that is important in cell growth or division results in uncontrolled cell division, which could potentially enhance the risk of malignant transformation. If our vectors demonstrate a similar adverse effect, or other adverse events, we may be required to halt or delay further 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 side effects. VY-AADC01 and VY-HTT01 will be administered directly to the targeted cells in the brain, requiring the patient to undergo brain surgery. In a previous Phase 1 clinical trial conducted by UCSF, three patients experienced hemorrhages caused by the surgical procedure for administering VY-AADC01. We are using the ClearPoint System, which has only been used in limited gene therapy neurosurgeries to date, in the ongoing Phase 1b clinical trial of VY-AADC01 to provide accurate placement of the cannula in the putamen, to allow for real-time, intra-operative MRI to assist the physician in visualizing the delivery of VY-AADC01 to the putamen and to avoid specific blood vessels during the duration of the surgical procedure, with the goal of reducing the risk of hemorrhages. One patient in the ongoing Phase 1b trial at UCSF experienced two serious adverse events, or SAEs, a pulmonary embolism, or blood clot in the lungs, and related heart arrhythmia, or irregular heartbeat, which were determined to be related to the surgical procedure and prolonged immobility, not VY-AADC01. If other side effects were to occur in connection with the surgical procedure, our clinical trials could be suspended or terminated.

If in the future we are unable to demonstrate that such side effects were caused by the administration process or related procedures, 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 any future SAEs are not product-related, and regulatory authorities do not order us to cease further development of our product candidates, 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 REMS to ensure that the benefits outweigh its risks, which may include, among other things, a medication guide outlining the risks of the product for distribution to patients and a communication plan to health care practitioners. Furthermore, if we or others later identify undesirable side effects caused by our product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend 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 product candidates and could significantly harm our business, prospects, financial condition and results of operations.

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

Regulatory authorities in some jurisdictions, including the United States and the European Union, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act of 1983, the FDA may designate a product candidate as an orphan drug or biological product if it is intended to treat a rare disease or condition, which is generally defined 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 or biological product will be recovered from sales in the United States. In the European Union, EMA's Committee for Orphan Medicinal Products 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 nine months if the biologics license application, or 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.

We believe that all of our current programs may qualify for orphan drug designation. 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 or biological products 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 or biological product for the same condition if the FDA concludes that the latter drug or biological product is not the same drug or biological product 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.

***A potential breakthrough therapy designation by the FDA for our product candidates may not lead to a faster development or regulatory review or approval process, and it does not increase the likelihood that our product candidates will receive marketing approval.***

We may seek a breakthrough therapy designation for some of our product candidates. A breakthrough therapy is defined as a drug or biological product that is intended, alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition, and preliminary clinical evidence indicates that the drug or biological product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. For drugs or biological products that have been designated as breakthrough therapies, interaction and communication between the FDA and the sponsor of the trial can help to identify the most efficient path for clinical development while minimizing the number of patients placed in ineffective control regimens. Drugs designated as breakthrough therapies by the FDA are also eligible for accelerated approval.

Designation as a breakthrough therapy is within the discretion of the FDA. Accordingly, even if we believe one of our product candidates meets the criteria for designation as a breakthrough therapy, the FDA may disagree and instead determine not to make such designation. In any event, the receipt of a breakthrough therapy designation for a product candidate may not result in a faster development process, review or approval compared to drugs considered for approval under conventional FDA procedures and does not assure ultimate approval by the FDA. In addition, even if one or more of our product candidates qualify as breakthrough therapies, the FDA may later decide that the drugs or biological products no longer meet the conditions for qualification.

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

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

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

Even if we obtain any regulatory approval for our product candidates, they will be subject to ongoing regulatory requirements for manufacturing, labeling, packaging, storage, advertising, promotion, sampling, record-keeping and submission of safety and other post-market information. Any regulatory approvals that we receive for our product candidates also may be subject to a REMS, limitations on the approved indicated uses for which the product may be marketed or to the conditions of approval, or contain requirements for potentially costly post-marketing testing, including Phase 4 clinical trials, and surveillance to monitor the quality, safety and efficacy of the product. For example, the holder of an approved BLA, is obligated to monitor and report adverse events and any failure of a product to meet the specifications in the BLA. 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 FDA rules and are subject to FDA review, in addition to other potentially applicable federal and state laws.

In addition, product manufacturers and their facilities are subject to payment of user fees and continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current good manufacturing practices, or cGMP, requirements and adherence to commitments made in the BLA or foreign marketing application. If we, or a regulatory authority, discover previously unknown problems with a product, such as adverse events of unanticipated severity or frequency, or problems with the facility where the product is manufactured or disagrees with

the promotion, marketing or labeling of that product, a regulatory authority may impose restrictions relative to that product, the manufacturing facility or us, including requiring recall or withdrawal of the product from the market or suspension of manufacturing.

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

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

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

In addition, FDA policies, and those of equivalent foreign regulatory agencies, 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 harm our business, financial condition, results of operations and prospects.

***We face significant competition in an environment of rapid technological change and the possibility that our competitors may achieve regulatory approval before us or develop therapies that are more advanced or effective than ours, which may harm our business and 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, including bluebird bio, Inc., Applied Genetic Technologies Corporation, Asklepios BioPharmaceutical, Inc., Audentes Therapeutics, Inc., Avalanche Biotechnologies, Inc., Dimension Therapeutics, Inc., GenSight Biologies SA,

NightstaRx Ltd, REGENXBIO Inc., uniQure and Spark Therapeutics, Inc. 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.

The main competitors for our specific programs include:

- VY-AADC01 will compete with a variety of therapies currently marketed and in development for advanced Parkinson's disease, including deep brain stimulation marketed by Medtronic plc, St. Jude Medical Inc. and other medical device companies, DUOPA/Duodopa marketed by AbbVie Inc., as well as AMT-090 or AAV-GDNF in development at uniQure, OXB-102/Prosavin in development at Oxford Biomedica plc and ND0612H in development at NeuroDerm Ltd.;
- VY-SOD101 for a monogenic form of ALS will potentially compete with ISIS 333611 being developed by Isis Pharmaceuticals, Inc., or Isis, in collaboration with Biogen Idec., or Biogen and Tirasemtiv being developed by Cytokinetics, Inc., or Cytokinetics;
- VY-FXN01 for Friedreich's ataxia will potentially compete with RG2833 being developed by BioMarin Pharmaceutical Inc., AAV-FXN being developed by AAVLife, and frataxin targeted gene therapy being developed by Agilis Biotherapeutics, LLC in collaboration with Intrexon Corporation and BB-FA being developed by BioBlast Pharma Ltd., or BioBlast;
- VY-HTT01 for Huntington's disease will potentially compete with ISIS-HTTRx being developed by Isis in collaboration with F. Hoffman-La Roche Ltd., or Roche, gene editing approach being developed by Sangamo Biosciences, Inc. in collaboration with Shire plc, and another gene therapy being developed by uniQure; and
- VY-SMN101 for spinal muscular atrophy will potentially compete with ChariSMA being developed by AveXis Inc., ISIS-SMN<sub>RX</sub> being developed by Isis and Biogen, LMI-070 being developed by Novartis AC, RO6885247 being developed by PTC Therapeutics, Inc. and Roche, BBrm1 being developed by BioBlast and CK-2127107 being developed by Cytokinetics in collaboration with Astellas Pharma US, Inc.

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 us, 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 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 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. If we are not able to compete effectively against potential competitors, our business will not grow and our financial condition and operations will be harmed.

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

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

### **Risks Related to Third Parties**

***To date, all of our revenue has been derived from our collaboration with Genzyme, and if this collaboration agreement were to be terminated, our business financial condition, results of operations and prospects would be harmed.***

In February 2015, we entered into a collaboration agreement with Genzyme to leverage our combined expertise and assets in gene therapy for CNS diseases. Under the agreement, we received an upfront commitment of approximately \$100 million. Pursuant to the agreement, we granted Genzyme an exclusive option to license, develop and commercialize (i) ex-U.S. rights to our advanced Parkinson's disease, Friedreich's ataxia and Huntington's disease programs and a future program, or the Split Territory Programs, with an incremental option to co-commercialize the product candidate from our Huntington's disease program in the United States and (ii) worldwide rights to our SMA program. If Genzyme exercises an option for a Split Territory Program, except for our advanced Parkinson's disease program, it is required to make an option exercise payment to us. Furthermore, Genzyme shall pay up to \$645 million in the aggregate upon the achievement of specified regulatory and commercial milestones, and will pay us tiered royalty payments based on a percentage of net sales of product candidates from the programs for which it is exercised its option, or the Optioned Programs.

Following Genzyme's exercise of an option for a program, Genzyme will have sole responsibility for the development and commercialization of the product candidates from such program in the applicable territory. Genzyme will have the sole discretion to determine and direct its efforts and resources, including the ability to discontinue all efforts and resources, it applies to the development and, if approval is obtained, commercialization and marketing of the product candidates covered by the Optioned Programs in the applicable territories. Genzyme may not be effective in obtaining approvals for the product candidates developed from the Optioned Programs or in marketing, or arranging for necessary supply, manufacturing or distribution relationships for, any approved products. Furthermore, Genzyme may

change its strategic focus or pursue alternative technologies in a manner that results in reduced, delayed or no revenue to us. Genzyme has a variety of marketed products and product candidates under collaboration with other companies, including some of our competitors, and its own corporate objectives may not be consistent with our best interests. If Genzyme fails to develop, obtain regulatory approval for or ultimately commercialize any product candidate from the Optioned Programs in the applicable territories, or if Genzyme terminates our collaboration, our business, financial condition, results of operations and prospects would be harmed. In addition, any dispute or litigation proceedings we may have with Genzyme in the future could delay development programs, create uncertainty as to ownership of or access to intellectual property rights, distract management from other business activities and generate substantial expense.

***We expect to rely on the ClearPoint System for the foreseeable future for the delivery of our product candidates that are injected directly into targeted regions of the brain. If there are any issues with the ClearPoint System or the manufacturer of the ClearPoint System, our business could be adversely affected.***

The ClearPoint System is being used in the ongoing Phase 1b clinical trial of VY-AADC01 as a treatment for advanced Parkinson's disease, and we expect to continue to use the ClearPoint System in future clinical trials of VY-AADC01 and any other of our product candidates that are injected directly into the brain. Therefore, any issues with the ClearPoint System, such as a finding that use of the ClearPoint System causes adverse events or a product recall, or the manufacturer of the ClearPoint System, such as bankruptcy or a decision to stop production of the system due to lack of profitability, could delay the development or commercialization of certain of our product candidates, which would have an adverse effect on our business and results of operations.

***We may enter into collaborations in the future with other third parties. If these collaborations are not successful, our business could be adversely affected.***

We may enter into additional collaborations in the future. Our ability to generate revenues from our collaborations will depend on our and our collaborators' abilities to successfully perform the functions assigned to each of us in these arrangements. In addition, our 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 collaborator is responsible could be harmful to the public perception and prospects of our gene therapy platform.

Our relationship with any future collaborations may pose several risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- the clinical trials conducted as part of these collaborations may not be successful;
- 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 collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- 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 trials;
- 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;



- collaborators could independently develop, or develop with third parties, products that compete directly or indirectly with our product candidates if the 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 collaborators as competitive with their own product candidates or products, which may cause collaborators to cease to devote resources to the commercialization of our product candidates;
- a collaborator with marketing and distribution rights to one or more of our product candidates that achieve regulatory approval may not commit sufficient resources to the marketing and distribution of any such product candidate;
- disagreements with collaborators, including disagreements over 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;
- 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 or inventorship of intellectual property developed pursuant to our collaborations;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability;
- the terms of our collaboration agreement may restrict us from entering into certain relationships with other third parties, thereby limiting our options; and
- collaborations may be terminated for the convenience of the 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 collaborations do not result in the successful development and commercialization of products, or if one of our collaborators terminates its agreement with us, we may not receive any future research funding or milestone or royalty payments under the collaboration. If we do not receive the funding we expect under these agreements, our development of product candidates could be delayed and we may need additional resources to develop our product candidates. In addition, if one of our collaborators terminates its agreement with us, we may find it more difficult to attract new collaborators and the perception of us in the business and financial communities could be adversely affected. All of the risks relating to product development, regulatory approval and commercialization described in this Quarterly Report apply to the activities of our collaborators.

If we decide to enter into future collaborations, we could face significant competition in seeking appropriate collaborators and the negotiation process is time-consuming and complex. Our ability to reach a definitive collaboration agreement with any future collaborators 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 several 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 them with our existing operations and company culture.

***We expect to rely on third parties to conduct, supervise and monitor our clinical trials, and if these third parties perform in an unsatisfactory manner, our business could be harmed.***

We expect to rely on CROs and clinical trial sites to ensure our clinical trials are conducted properly and on time. While we will have agreements governing their activities, we will have limited influence over their actual performance. We will control only certain aspects of our CROs' activities. Nevertheless, we will be responsible for ensuring that each of our clinical studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards, and our reliance on the CROs does not relieve us of our regulatory responsibilities. For example, the only clinical trial of any of our product candidates or programs is being conducted by UCSF. If UCSF terminated the clinical trial of VY-AADC01, we would be required to find another party to conduct any new trials. We may be unable to find a new party to conduct new trials of our product candidates or obtain clinical supply of our product candidates or AAV vectors for such trials.

We and our CROs are required to comply with the FDA's GCPs for conducting, recording and reporting the results of IND-enabling studies and clinical studies to assure that the data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical trial participants are protected. The FDA enforces these GCPs through periodic inspections of trial sponsors, principal investigators and clinical trial sites. If we or our CROs fail to comply with applicable GCPs, the clinical data generated in our future clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving any marketing applications. Upon inspection, the FDA may determine that our clinical trials did not comply with GCPs. In addition, our future clinical trials will require a sufficient number of patients to evaluate the safety and effectiveness of our product candidates. Accordingly, if our CROs fail to comply with these regulations or fail to recruit a sufficient number of patients, we may be required to repeat such clinical trials, which would delay the regulatory approval process.

Our CROs are not our employees, and we are therefore unable to directly monitor whether or not they devote sufficient time and resources to our clinical and nonclinical programs. These CROs may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities that could harm our competitive position. If our CROs do not successfully carry out their contractual duties or obligations, fail to meet expected deadlines, or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements, or for any other reasons, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval for, or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase, and our ability to generate revenues could be delayed.

### **Risks Related to Manufacturing**

***Gene therapies are novel, complex and difficult to manufacture. We could experience manufacturing problems that result in delays in the development or commercialization of our product candidates or otherwise harm our business.***

The manufacturing process used to produce our product candidates is complex, novel and has not been validated for commercial use. 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 our suppliers and collaborators.

Our product candidates require processing steps that are more complex than those required for most chemical pharmaceuticals. Moreover, unlike chemical pharmaceuticals, the physical and chemical properties of a biologic such as ours generally cannot be fully characterized. As a result, assays of the finished product may not be sufficient to ensure that the product will perform in the intended manner. Accordingly, we and our collaborators employ multiple steps to control the 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 or our collaborators may encounter problems achieving adequate quantities and quality of clinical-grade materials that meet the FDA, EMA or other applicable standards or specifications with consistent and acceptable production yields and costs.

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

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

Any problems in our or our collaborators' manufacturing process or facilities 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 our or our collaborators' manufacturing process could restrict our ability to meet market demand for our products.

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

Before we can begin to commercially manufacture our product candidates in our own facility, or the facility of a collaborator, we must obtain regulatory approval from the FDA for our manufacturing process and our collaborator's facility. A manufacturing authorization must also be obtained from the appropriate European Union regulatory authorities. To date, no cGMP gene therapy manufacturing facility in the United States has received approval from the FDA for the manufacture of an approved gene therapy product and, therefore, the timeframe required for us to obtain such approval is uncertain. In addition, we must pass a pre-approval inspection of our or our collaborator's manufacturing facility by the FDA before any of our product candidates can obtain marketing approval. In order to obtain approval, we will need to ensure that all of our processes, methods and equipment are compliant with cGMP, and perform extensive audits of vendors, contract laboratories and suppliers. If any of our vendors, contract laboratories or suppliers is found to be out of compliance with cGMP, we may experience delays or disruptions in manufacturing while we work with these third parties to remedy the violation or while we work to identify suitable replacement vendors. The cGMP requirements govern quality control of the manufacturing process and documentation policies and procedures. In complying with cGMP, we will be obligated to expend time, money and effort in production, record keeping and quality control to assure that the product meets applicable specifications and other requirements. If we fail to comply with these requirements, we would be subject to possible regulatory action and may not be permitted to sell any products that we may develop.

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

The 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 our third-party manufacturers or us could harm our business, financial condition, results of operations and prospects.

If our third-party manufacturers or we fail to comply with applicable cGMP regulations, FDA and foreign 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 could be harmed.

Additionally, if supply from any third-party manufacturers is delayed or interrupted, there could be a significant disruption in the supply of our clinical or commercial material. We have an agreement in place with MassBiologics pursuant to which we are collaborating to establish scalable processes for manufacturing recombinant AAV vector products using cGMP, but we do not have any agreement in place with MassBiologics or any other party to manufacture clinical or commercial material. Therefore, if we are unable to enter into an agreement with MassBiologics or another manufacturer to manufacture clinical or commercial material, or if our agreement with MassBiologics were terminated, we would have to find suitable alternative manufacturers. This could delay our or our collaborators' ability to conduct clinical trials or commercialize our current and future product candidates. The 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 the manufacturing process for our products, shortages of raw materials or failure of any of our key suppliers to deliver necessary components could result in delays in our clinical development or marketing schedules.***

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

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

***Interruptions in the supply of product candidates or inventory loss may harm our operating results and financial condition.***

Our product candidates are manufactured using technically complex processes requiring specialized facilities, highly specific raw materials and other production constraints. The complexity of these processes, as well as strict government standards for the manufacture and storage of our product candidates, subjects us to manufacturing risks. While product candidate batches released for use in clinical trials or for commercialization undergo sample testing, some defects may only be identified following product release. In addition, process deviations or unanticipated effects of approved process changes may result in these intermediate products not complying with stability requirements or specifications. Our product candidates must be stored and transported at temperatures within a certain range. If these environmental conditions deviate, our product candidates' remaining shelf-lives could be impaired or their efficacy and safety could be negatively impacted, making them no longer suitable for use.

The occurrence, or suspected occurrence, of manufacturing and distribution difficulties can lead to lost inventories and, in some cases, product recalls, with consequential reputational damage and the risk of product liability. The investigation and remediation of any identified problems can cause production delays, substantial expense, lost sales and delays of new product launches. Any interruption in the supply of finished products or the loss thereof could hinder our ability to timely distribute our products and satisfy customer demand. Any unforeseen failure in the storage of the product or loss in supply could delay our clinical trials and, if our product candidates are approved, result in a loss of our market share and negatively affect our business, financial condition, results of operations and prospects.

## **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 generated through our product engine. Research programs to identify new product candidates require substantial technical, financial and human resources. Although VY-AADC01 is currently in clinical development and our other product candidates are in preclinical development, we may fail to identify other potential product candidates for clinical

development for several reasons. For example, our research may be unsuccessful in identifying potential product candidates or our potential product candidates may be shown to have harmful side effects, may be commercially impracticable to manufacture or may have other characteristics that may make the products unmarketable or unlikely to receive marketing approval.

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

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

***Our future success depends on our ability to retain key members of our management team, and to attract, retain and motivate qualified personnel.***

We are highly dependent on Steven M. Paul, M.D., our President and Chief Executive Officer as well as other members of our management team, the loss of whose services may adversely impact the achievement of our objectives. While we have entered into employment agreements or offer letters 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 might impede the achievement of our research, development and commercialization objectives.

Recruiting and retaining other qualified employees, consultants and advisors for our business, including scientific and technical personnel, also will be critical to our success. There currently is a shortage of skilled individuals with substantial gene therapy experience, which is likely to continue. As a result, competition for skilled personnel, including in gene therapy research and vector manufacturing, is intense and the turnover rate can be high. We may not be able to attract and retain personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies and academic institutions for individuals with similar skill sets. In addition, failure to succeed in preclinical or clinical trials or applications for marketing approval may make it more challenging to recruit and retain qualified personnel. The inability to recruit, or loss of services of certain executives, key employees, consultants or advisors, may impede the progress of our research, development and commercialization objectives and could 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 activities and, in the longer term, build a commercial infrastructure to support commercialization of any of our product candidates that are approved for sale. We can provide no assurances that we will have sufficient resources in the future to manage all of our planned programs. 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 intend to adopt a code of conduct applicable to all of our employees, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from government investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, financial condition, results of operations and prospects, including the imposition of significant fines or other sanctions.

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

In the United States, there have been, and continue to be, several legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the Affordable Care Act, was passed, which substantially changes the way health care is financed by both the government and private insurers, and significantly impacts the U.S. pharmaceutical industry. The Affordable Care Act, among other things: (i) imposes a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected; (ii) increases the minimum Medicaid rebates owed by manufacturers under the Medicaid Drug Rebate Program; (iii) extends manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals enrolled in Medicaid managed care organizations; (iv) establishes an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents; (v) expands the availability of lower pricing under the 340B drug pricing program by expanding the types of entities eligible to participate in the program; and (vi) establishes a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturer's outpatient drugs to be covered under Medicare Part D; (vii) expands entities eligible for discounts under the Public Health Services pharmaceutical pricing program; and (viii) initiates a new Patient Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research. 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.

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 and fraud and abuse and enforcement. Continued implementation of the Affordable Care Act 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 adopted since the Affordable Care Act was enacted, including aggregate reductions to Medicare payments to providers of up to 2% per fiscal year effective April 1, 2013. These reductions will stay in effect through 2024 unless additional congressional action is taken. Additionally, in

January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding, which could have a material adverse effect on customers for our drugs, if approved, and, accordingly, our financial operations. Further, as part of the proposed 2016 budget, President Obama has sought to reduce the current 12-year exclusivity period that a reference biologic is granted to a seven-year exclusivity period.

For each state that does not choose to expand its Medicaid program, there may be fewer insured patients overall, which could impact the sales, business and financial condition of manufacturers of branded prescription drugs. Where patients receive insurance coverage under any of the new options made available through the Affordable Care Act, the possibility exists that manufacturers may be required to pay Medicaid rebates on that resulting drug utilization, a decision that could impact manufacturer revenues. The U.S. federal government also has announced delays in the implementation of key provisions of the Affordable Care Act. The implications of these delays for our and our partners' business and financial condition, if any, are not yet clear.

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 will pay for healthcare products and services, which could result in reduced demand for our product candidates or additional pricing pressures. We cannot predict what healthcare reform initiatives may be adopted in the future. Further federal, state and foreign legislative and regulatory developments are likely, and we expect ongoing initiatives to increase pressure on drug pricing. Such reforms could have an adverse effect on anticipated revenues from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates.

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

If we obtain FDA approval for any of our product candidates and begin commercializing those products in the United States, our operations will be directly, or indirectly through our prescribers, customers and purchasers, subject to various federal and state laws and regulations, including, without limitation, the federal Anti-Kickback Statute, the federal civil and criminal False Claims Act, and the Physician Payments Sunshine Act and regulations. These laws will impact, among other things, our proposed sales, marketing and educational programs. In addition, we may be subject to data privacy laws by both the federal government and the states in which we conduct our business. Such laws that may constrain the business or financial arrangements and relationships through which we conduct our operations include, but are not limited to:

- the federal Anti-Kickback Statute, which prohibits, among other things, persons and 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, to induce or in return for either the referral of an individual for, or 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. Further, the Affordable Care Act amends the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it;
- the federal civil and criminal false claims laws and civil monetary penalty laws, including the civil False Claims Act, 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, or making a false statement to avoid, decrease, or conceal an obligation to pay money to the federal government. The Affordable Care Act 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 civil False Claims Act;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created additional federal criminal statutes that prohibit a person from knowingly and willfully executing or attempting to execute 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, or 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 Payments Sunshine Act, that requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children’s Health Insurance Program, with specific exceptions, to report annually to CMS information related to 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, by the 90<sup>th</sup> day of each subsequent calendar year, and disclosure of such information is made by CMS on a publicly available website; and
- state and/or foreign law equivalents of each of the above federal laws, such as state anti-kickback and false claims laws that may apply to arrangements and claims involving health care items or services reimbursed by non-governmental third party payors; 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; state laws that require pharmaceutical companies to comply with the pharmaceutical industry’s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government; and state and foreign 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, disgorgement, contractual damages, reputational harm, diminished profits and future earnings, imprisonment and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

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

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



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

We will face an inherent risk of product liability exposure related to the testing of our product candidates in clinical trials and may face an even greater risk if we commercialize any products that we may develop. If we cannot successfully defend ourselves against claims that our product candidates caused injuries, we could incur substantial liabilities. Regardless of merit or eventual outcome, liability claims may result in:

- decreased demand for 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 commercialize any product candidates that we may develop; and
- injury to our reputation and significant negative media attention.

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

***If we fail to comply with environmental, health and safety laws and regulations, we could become subject to fines or penalties or incur costs that could harm our business.***

We 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 also produce hazardous waste products. We generally contract with third parties for the disposal of these materials and wastes. We cannot eliminate the risk of contamination or injury from these materials. In the event of contamination or injury resulting from our use of hazardous materials, we could be held liable for any

resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties.

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

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations, which have tended to become more stringent over time. These current or future laws and regulations may impair our 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 harm our business, financial condition, results of operations and prospects.

***Unfavorable global economic conditions could adversely affect our business, financial condition or results of operations.***

Our results of operations could be adversely affected by general conditions in the global economy and in the global financial markets. The most recent global financial crisis caused extreme volatility and disruptions in the capital and credit markets. A severe or prolonged economic downturn, such as the most recent global financial crisis, could result in a variety of risks to our business, including weakened demand for our product candidates and our ability to raise additional capital when needed on acceptable terms, if at all. This is particularly true in the European Union, which is undergoing a continued severe economic crisis. A weak or declining economy could strain our suppliers, possibly resulting in supply disruption, or cause delays in payments for our services by third-party payors or our 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 adversely impact our business.

***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 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 disruption of our 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 development and commercialization of our product candidates could be delayed.

**Risks Related to the Commercialization of Our Product Candidates**

***The affected populations for our product candidates may be smaller than we or third parties currently project, which may affect the addressable markets for our product candidates.***

Our projections of the number of people who have the diseases we are seeking to treat, as well as the subset of people with these diseases who have the potential to benefit from treatment with our product candidates, are estimates based on our knowledge and understanding of these diseases. The total addressable market opportunity for our product candidates will ultimately depend upon a number of factors including the diagnosis and treatment criteria included in the final label, if approved for sale in specified indications, acceptance by the medical community, patient access and product pricing and reimbursement. Prevalence estimates are frequently based on information and assumptions that are not exact and may not be appropriate, and the methodology is forward-looking and speculative. The process we have

used in developing an estimated prevalence range for the indications we are targeting has involved collating limited data from multiple sources. Accordingly, the prevalence estimates included in this Quarterly Report should be viewed with caution. Further, the data and statistical information used in this Quarterly Report, including estimates derived from them, may differ from information and estimates made by our competitors or from current or future studies conducted by independent sources.

The use of such data involves risks and uncertainties and is subject to change based on various factors. Our estimates may prove to be incorrect and new studies may change the estimated incidence or prevalence of the diseases we seek to address. The number of patients with the diseases we are targeting in the United States, the European Union and elsewhere may turn out to be lower than expected or may not be otherwise amenable to treatment with our products, or new patients may become increasingly difficult to identify or access, all of which would harm our results of operations and our business.

***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, we may be unable to generate any product revenue.***

We currently have no sales and marketing organization. To successfully commercialize any products that may result from our clinical 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. Under our collaboration agreement with Genzyme, we have granted Genzyme an exclusive option to license, develop and commercialize ex-U.S. rights to our advanced Parkinson's disease program, our Friedreich's ataxia program, a future program to be designated by Genzyme and our Huntington's disease program. Additionally, we have granted Genzyme an incremental option to co-commercialize our Huntington's disease program in the United States and to worldwide rights to our spinal muscular atrophy program. If Genzyme exercises any of these options, except for our advanced Parkinson's disease program, we would be eligible to receive specified option fees. In addition we would be eligible to receive specified milestone payments and royalties for any product developed in such programs. In the future, we may enter into collaborations regarding other of our product candidates with other entities to utilize their established marketing and distribution capabilities, but we may be unable to enter into such agreements on favorable terms, if at all. If any current or future 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 harm our business, financial condition, results of operations and prospects.

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

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

third-party payors. Coverage and reimbursement by a third-party payor may depend upon several factors, including the third-party payor's determination that use of a product is:

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

No uniform policy requirement for coverage and reimbursement for drug products exists among third-party payors. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, 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 each different 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. Assuming we obtain coverage for a given product by a third-party payor, the resulting reimbursement payment rates may not be adequate or may require co-payments that patients find unacceptably high. Patients who are prescribed medications for the treatment of their conditions, and their prescribing physicians, generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Patients are unlikely to use our products unless coverage is provided and reimbursement is adequate to cover all or a significant portion of the cost of our products. Therefore, coverage and adequate reimbursement is critical to new product acceptance. Additionally, there may be significant delays in obtaining coverage and reimbursement for newly approved drugs and biologics, and coverage may be more limited than the purposes for which the drug is approved by the FDA or comparable foreign regulatory authorities.

There is significant uncertainty related to third-party coverage and reimbursement of newly approved products. 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. Currently, no gene therapy product has been approved for coverage and reimbursement by the Centers for Medicare & Medicaid Services, or CMS, the agency responsible for administering the Medicare program. It is difficult to predict what CMS 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. Moreover, reimbursement agencies in the European Union may be more conservative than CMS. For example, several cancer drugs have been approved for reimbursement in the United States and have not been approved for reimbursement in certain European Union Member States. It is difficult to predict what third-party payors will decide with respect to the coverage and reimbursement for our product candidates, especially given that the cost of our product candidates is likely to be very high and pricing of such products is highly uncertain.

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, Canada and other countries may put pricing pressure on us. For example, one gene therapy product was approved in the European Union in 2012 but is yet to be widely available commercially. In many countries, the prices of medical products are subject to varying price control mechanisms as part of national health systems. 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 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, such as average sales price, or ASP, average manufacturer price, or AMP, and Actual Acquisition Cost. The existing data for reimbursement based on some of these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. 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. The downward pressure on healthcare costs in general, particularly prescription drugs and surgical procedures and other treatments, has become intense. As a result, increasingly high barriers are being erected to the entry of new products such as ours.

***The commercial success of any of our product candidates will depend upon its degree of market acceptance by physicians, patients, third-party payors and others in the medical community.***

Ethical, social and legal concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from the FDA in the United States, EMA in the European Union and other regulatory authorities internationally, 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 gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the European Commission;
- patient awareness of, and willingness to seek, genotyping;
- the willingness of physicians to prescribe new therapies;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;

- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is launched.

***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 adversely affect our ability to conduct our business or obtain regulatory approvals for our product candidates.***

Gene therapy remains a novel technology, with no gene therapy product approved to date in the United States and only one gene therapy product approved to date in the European Union. Public perception may be influenced by claims that gene therapy is unsafe, and gene therapy may not gain the acceptance of the public or the medical community. In particular, our success will depend upon physicians who specialize in the treatment of genetic diseases targeted by our product candidates, prescribing treatments that involve the use of our product candidates in lieu of, or in addition to, existing treatments with which they are familiar and for which greater clinical data may be available. More restrictive government regulations or negative public opinion would have an adverse effect on 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 non-AAV vectors. Serious adverse events in our clinical trials, or other clinical trials involving gene therapy products or our competitors' products, even if not ultimately attributable to the relevant product candidates, and the resulting publicity, could result in increased government regulation, unfavorable public perception, potential regulatory delays in the testing or approval of our product candidates, stricter labeling requirements for those product candidates that are approved and a decrease in demand for any such product candidates.

***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 harm our business.***

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

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;

- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability; and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

Further, in many foreign countries it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the Foreign Corrupt Practices Act. Although we expect to implement policies and procedures designed to comply with these laws and policies, there can be no assurance that our employees, contractors and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

### **Risks Related to Our Intellectual Property**

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

We are 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 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 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 included in all of our licenses.

In some circumstances, particularly in licenses with academic institutions, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain the patents, covering technology that we license from third parties. Therefore, we cannot be certain that these patents and applications will be prosecuted, maintained and enforced in a manner consistent with the best interests of our business. 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 adversely affected. 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.

Further, in many of our license agreements we are responsible for bringing any actions against any third party for infringing on the patents we have licensed. Certain of our license agreements also require us to meet development thresholds to maintain the license, including establishing a set timeline for developing and commercializing products and minimum yearly 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 intellectual property of the licensor that is not subject to the licensing agreement;
- the sublicensing of patent and other rights under our collaborative development relationships;
- our diligence obligations under the license agreement and what activities satisfy those diligence obligations;
- the inventorship or ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and

- the priority of invention of patented technology.

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

If we fail to comply with our obligations under these license 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.

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 commercialize our products and technology may be adversely affected.***

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 product candidates and 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. For example, in some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which may compromise our ability to obtain patent protection for certain inventions related to or building upon such prior work. Consequently, we will not be able to obtain any such patents to prevent others from using our technology for, and developing and marketing competing products to treat, these indications. 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.

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 product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all.



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

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

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

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

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

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

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

We sometimes collaborate with non-profit and academic institutions to accelerate our preclinical research or development under written agreements with these institutions. Typically, these institutions provide us with an option to negotiate a license to any of the institution's rights in technology resulting from the collaboration. 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 be required to expend significant time and resources to redesign our 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 develop or commercialize the affected product candidates, which could harm our business significantly.

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

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

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

Filing, prosecuting and defending patents on 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. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patent protection to develop their own products and, further, may export otherwise infringing products to territories where we have patent protection, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property protection, particularly those relating to biotechnology products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. For example, an April 2014 report from the Office of the United States Trade Representative identified a number of countries, including India and China, where challenges to the procurement and enforcement of patent rights have been reported. Several countries, including India and China, have been listed in the report every year since 1989. 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 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 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 lack of novelty, obviousness, lack of written description or non-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 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 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 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 harm our business.***

Our commercial success depends upon our ability and the ability of our collaborators to develop, manufacture, market and sell our product candidates and use our proprietary technologies without infringing the proprietary rights and intellectual property of third parties. The biotechnology and pharmaceutical industries are characterized by extensive and complex litigation regarding patents and other intellectual property rights. We may in the future become party to, or be threatened with, adversarial proceedings or litigation regarding intellectual property rights with respect to our product candidates and technology, including interference proceedings, post grant review and *inter partes* review before the USPTO. Third parties may assert infringement claims against us based on existing patents or patents that may be granted in the future, regardless of their merit. There is a risk that third parties may choose to engage in litigation with us to enforce or to otherwise assert their patent rights against us. Even if we believe such claims are without merit, a court of competent jurisdiction could hold that these third-party patents are valid, enforceable and infringed, which could adversely affect our ability to commercialize our product candidates or any other of our 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 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 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 manufacturing and commercializing our product candidates or force us to cease some of our business operations, which could harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on 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. To counter infringement or unauthorized use claims or to defend against claims of infringement 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 have a substantial adverse effect on 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 litigation or other proceedings could adversely affect our ability to compete in the marketplace.

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

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

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

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

Recent patent reform legislation could increase the uncertainties and costs surrounding the prosecution of patent applications and the enforcement or defense of issued patents. On September 16, 2011, the Leahy-Smith America Invents Act, or 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 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, or Supreme Court. On March 20, 2012, the Supreme Court issued a decision in *Mayo Collaborative Services v. Prometheus Laboratories, Inc.*, or *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.*, or *Myriad*, a case involving patent claims held by Myriad Genetics, Inc. 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.

On March 4, 2014, the USPTO issued a guidance memorandum to patent examiners titled 2014 Procedure For Subject Matter Eligibility Analysis Of Claims Reciting Or Involving Laws Of Nature/Natural Principles, Natural Phenomena, and/or Natural Products. These guidelines instruct USPTO examiners on the ramifications of the *Prometheus* and *Myriad* rulings and apply the *Myriad* ruling to natural products and principles including all naturally occurring nucleic acids. 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 have an adverse effect on 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.

Outside the United States, other courts have also begun to address the patenting of genetic material. In August 2015, the Australian High Court ruled that isolated genes cannot be patented in Australia. The decision did not address methods of using genetic material. Any ruling of a similar scope in other countries could affect the scope of our intellectual property rights.

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

Depending upon the timing, duration and specifics of any FDA marketing approval of our product candidates, one or more of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, or 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.

***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 adversely affected.***

We have registered trademarks with the USPTO for the mark “Voyager Therapeutics” and the Voyager logo. 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 adversely affected. 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 adversely impact 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 but that are not covered by the claims of the patents that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to make the inventions covered by the issued patent or pending patent application that we license or may own in the future;
- we, or our license partners or current or future collaborators, might not have been the first to file patent applications covering certain of our or their inventions;
- others may independently develop similar or alternative technologies or duplicate any of our technologies without infringing our owned or licensed intellectual property rights;
- it is possible that our pending 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 have an adverse effect on our business; and
- we may choose not to file a patent for certain trade secrets or know-how, and a third party may subsequently file a patent covering such intellectual property.

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

***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 currently rely on certain third parties to manufacture all or part of our product candidates and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our product engine and pipeline, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees 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. Despite the contractual provisions employed when working with third parties, 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 discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may harm our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

#### **Risks Related to Ownership of Our Common Stock**

***An active trading market for our common stock may not be sustained, and you may not be able to resell your shares at or above the price you paid.***

Although we have listed our common stock on The NASDAQ Global Select Market, an active trading market for our shares may not be sustained. In the absence of an active trading market for our common stock, investors may not be able to sell their common stock at or above the price at which they acquired their shares or at the time that they would like to sell. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

***Our executive officers, directors, principal stockholders and their affiliates exercise significant influence over our company.***

The holdings of our executive officers, directors, principal stockholders and their affiliates, including investment funds affiliated with Third Rock Ventures and Funds affiliated with Fidelity Management Research Company, or Fidelity, represent beneficial ownership, in the aggregate, of approximately 67% of our outstanding common stock as of September 30, 2015. As a result, these stockholders, if they act together, will be able to influence our management and affairs and the outcome of matters submitted to our stockholders for approval, including the election of directors and any sale, merger, consolidation, or sale of all or substantially all of our assets. In addition, this concentration of ownership might adversely affect the market price of our common stock by:

- delaying, deferring or preventing a change of control of us;
- impeding a merger, consolidation, takeover or other business combination involving us; or
- discouraging a potential acquirer from making a tender offer or otherwise attempting to obtain control of us.

***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 will rely in part on the research and reports that industry or financial analysts publish about us or our business. If no or few analysts maintain coverage of us, the trading price of our stock would likely decrease. 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.

***Future sales of our common stock may cause our stock price to decline.***

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity securities.

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

Our stock price is likely to be volatile. The stock market in general, and the market for biopharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. The market price for our common stock may be influenced by many factors, including:

- regulatory action and results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;



- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in the section titled “Risk Factors” and elsewhere in this Quarterly Report.

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.

In the past, following periods of volatility in the market price of a company’s securities, securities class-action litigation often has been instituted against that company. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management’s attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

***We have broad discretion in how we use the proceeds from our IPO and may not use these proceeds effectively, which could affect our results of operations and cause our stock price to decline.***

We will have considerable discretion in the application of the net proceeds from our recently completed IPO. As a result, investors will be relying upon management’s judgment with only limited information about our specific intentions for the use of the balance of the net proceeds from our IPO. We may use the net proceeds for purposes that do not yield a significant return or any return at all for our stockholders. In addition, pending their use, we may invest the net proceeds from this the IPO in a manner that does not produce income or that loses value.

***We are an “emerging growth company,” and the reduced disclosure requirements applicable to emerging growth companies may make our common stock less attractive to investors.***

For so long as we remain an “emerging growth company,” or EGC, as defined in the JOBS Act, we are permitted and intend to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not EGCs. These exemptions include:

- not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002;
- not being required to comply with 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;
- reduced disclosure obligations regarding executive compensation; and
- exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We may take advantages of these exemptions until we are no longer an EGC. We would cease to be an EGC upon the earlier of: (i) the last day of the fiscal year in which we have total annual gross revenues of \$1.0 billion or more; (ii) the last day of the fiscal year following the fifth anniversary of the date of the completion of our IPO; (iii) the date on which we have issued more than \$1.0 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission or SEC, which means the first day of the year following the first year in which the market value of our common stock that is held by non-affiliates exceeds \$700 million as of June 30th.

We may choose to take advantage of some, but not all, of the available exemptions. In particular, we have not included all of the executive compensation information that would be required if we were not an EGC. We cannot predict whether investors will find our common stock less attractive if we rely on certain or all of these exemptions.

In addition, the JOBS Act provides that an EGC may take advantage of an extended transition period for complying with new or revised accounting standards. This allows an EGC to delay the adoption of certain accounting standards until those standards would otherwise 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. 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 and may decline.

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

As a public company, and particularly after we are no longer an EGC, we will incur significant legal, accounting and other expenses that we did not incur as a private company. In addition, the Sarbanes-Oxley Act of 2002 and rules subsequently implemented by the SEC and The NASDAQ Stock Market have imposed various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. Our management and other personnel will need to devote a substantial amount of time to these compliance initiatives. Moreover, these rules and regulations will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that these rules and regulations may make it more difficult and more expensive for us to obtain director and officer liability insurance, and we may be required to accept reduced policy limits and coverage or incur substantially higher costs to obtain the same or similar coverage. As a result, it may be more difficult for us to attract and retain qualified people to serve on our board of directors, our board committees or as executive officers.

Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, we will be required to furnish a report by our management on our internal control over financial reporting. However, while we remain an EGC, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that neither we nor our independent registered public accounting firm will 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 amended and restated certificate of incorporation and bylaws and Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.***

Provisions in our amended and restated certificate of incorporation and bylaws may discourage, delay or prevent a merger, acquisition or other change in control of us that stockholders may consider favorable, including transactions in which you might otherwise receive a premium for your shares. These provisions also could limit the price that investors might be willing to pay in the future for shares of our common stock, thereby depressing the market price of our common stock. In addition, because our board of directors is responsible for appointing the members of our management team, these provisions may frustrate or prevent any attempts by our stockholders to replace or remove our

current management by making it more difficult for stockholders to replace members of our board of directors. Among other things, these provisions:

- establish a classified board of directors such that not all members of the board are elected at one time;
- allow the authorized number of our directors to be changed only by resolution of our board of directors;
- limit the manner in which stockholders can remove directors from the board;
- establish advance notice requirements for stockholder proposals that can be acted on at stockholder meetings and nominations to our board of directors;
- require that stockholder actions must be effected at a duly called stockholder meeting and prohibit actions by our stockholders by written consent;
- limit who may call stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a stockholder rights plan, or so-called “poison pill,” that would work to dilute the stock ownership of a potential hostile acquirer, effectively preventing acquisitions that have not been approved by our board of directors; and
- require the approval of the holders of at least 75% of the votes that all our stockholders would be entitled to cast to amend or repeal certain provisions of our amended and restated certificate of incorporation or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner.

***Our amended and restated certificate of incorporation designates the Court of Chancery of the State of Delaware as the sole and exclusive forum for certain types of actions and proceedings 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.***

Our amended and restated certificate of incorporation, provides that, unless we consent in writing to an alternative forum, the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (i) any derivative action or proceeding brought on our behalf, (ii) any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers and employees to us or our stockholders, (iii) any action asserting a claim arising pursuant to any provision of the Delaware General Corporation Law, our certificate of incorporation or our bylaws or (iv) any action asserting a claim that is governed by the internal affairs doctrine, in each case subject to the Court of Chancery having personal jurisdiction over the indispensable parties named as defendants therein. Any person purchasing or otherwise acquiring any interest in any shares of our capital stock shall be deemed to have notice of and to have consented to this provision of our amended and restated certificate of incorporation. This choice of forum provision may limit a stockholder’s ability to bring a claim in a judicial forum that it finds favorable for disputes with us or our directors, officers or employees, which may discourage such lawsuits against us and our directors, officers and employees even though an action, if successful, might benefit our stockholders. Stockholders who do bring a claim in the Court of Chancery could face additional litigation costs in pursuing any such claim, particularly if they do not reside in or near the State of Delaware. The Court of Chancery may also reach different judgments or results than would other courts, including courts where a stockholder considering an action may be located or would otherwise choose to bring

the action, and such judgments or results may be more favorable to us than to our stockholders. Alternatively, if a court were to find this provision of our amended and restated certificate of incorporation inapplicable to, or unenforceable in respect of, one or more of the specified types of actions or proceedings, we may incur additional costs which could have a material adverse effect on our business, financial condition or results of operations.

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

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

## **ITEM 2. UNREGISTERED SALES OF EQUITY SECURITIES AND USE OF PROCEEDS**

### ***Unregistered Sales of Equity Securities***

During the nine months ended September 30, 2015, we granted stock options to purchase an aggregate of 854,696 shares of our common stock, with exercise prices ranging from \$7.27 to \$8.33 per share, to our employees, directors and consultants pursuant to our 2014 Stock Incentive Plan.

We deemed the grants of stock options described above as exempt pursuant to Section 4(a)(2) of the Securities Act or to be exempt from registration under the Securities Act in reliance on Rule 701 of the Securities Act as offers and sales of securities under compensatory benefit plans and contracts relating to compensation in compliance with Rule 701. Each of the recipients of securities in any transaction exempt from registration had either received or had adequate access, through employment, business or other relationships, to information about us.

No underwriters were used in the foregoing transactions.

### ***Use of Proceeds from Initial Public Offering of Common Stock***

On November 16, 2015 we closed our IPO whereby we sold 5,750,000 shares of common stock, at a public offering price of \$14.00 per share, including 750,000 shares of common stock issued upon the full exercise by the underwriters of their option to purchase additional shares, resulting in net proceeds to the Company of \$72.7 million after deducting underwriting discounts and commissions and estimated offering expenses payable by the Company. The underwriters were Cowen and Company, LLC, Piper Jaffray & Co., Wedbush Securities Inc. and Nomura Securities International, Inc.

We raised approximately \$72.7 million in net proceeds after deducting underwriting discounts and commissions and estimated offering expenses payable by us. None of these expenses consisted of direct or indirect payments made by us to directors, officers or persons owning 10% or more of our common stock or to their associates, or to our affiliates. There has been no material change in the planned use of proceeds from our initial public offering as described in our final prospectus filed with the SEC on November 12, 2015 pursuant to Rule 424(b)(4). We invested the funds received in cash equivalents and other short-term investments in accordance with our investment policy.

## **ITEM 3. DEFAULTS UPON SENIOR SECURITIES**

None.

## **ITEM 4. MINE SAFETY DISCLOSURES**

Not Applicable.

## **ITEM 5. OTHER INFORMATION**

Not Applicable.

**ITEM 6. EXHIBITS**

The exhibits filed or furnished as part of this Quarterly Report are set forth on the Exhibit Index, which is incorporated herein by reference.

**INDEX TO EXHIBITS**

<b>Exhibit No.</b>	<b>Description</b>	<b>Incorporated by Reference to:</b>			
		<b>Form or Schedule</b>	<b>Exhibit No.</b>	<b>Filing Date with SEC</b>	<b>SEC File Number</b>
3.1	Amended and Restated Certificate of Incorporation of the Registrant.	8-K	3.1	11/16/2015	001-37625
3.2	Amended and Restated By-Laws of the Registrant.	8-K	3.2	11/16/2015	001-37625
4.1	Specimen Common Stock Certificate of the Registrant.	S-1	4.1	10/28/2015	333- 207367
31.1	Certification of Chief Executive Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.				
31.2	Certification of Chief Financial Officer pursuant to Exchange Act Rules 13a-14 or 15d-14.				
32.1+	Certification of Chief Executive Officer and Principal Chief Officer pursuant to Exchange Act Rules 13a-14(b) or 15d-14(b) and 18 U.S.C. Section 1350.				
101.INS	XBRL Instance Document.				
101.SCH	XBRL Taxonomy Extension Schema Document.				
101.CAL	XBRL Taxonomy Extension Calculation Document.				
101.LAB	XBRL Taxonomy Extension Definition Linkbase Document.				
101.PRE	XBRL Taxonomy Extension Labels Linkbase Document.				
101.DEF	XBRL Taxonomy Extension Presentation Link Document.				

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+ The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Quarterly Report on Form 10-Q and will not be deemed to be “filed” for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

**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.

Date: December 17, 2015

**VOYAGER THERAPEUTICS**

By: /s/ Steven M. Paul, M.D.  
Steven M. Paul, M.D.  
President and Chief Executive Officer; Director  
(Principal Executive Officer)

By: /s/ J. Jeffrey Goater  
Jeffrey Goater  
Senior Vice President, Finance and Business  
Development  
(Principal Financial and Accounting Officer)

**Certification**

I, Steven M. Paul, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2015 of Voyager Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: December 17, 2015

/s/ Steven M. Paul

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Steven M. Paul  
*President and Chief Executive Officer*  
*(Principal Executive Officer)*

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**Certification**

I, J. Jeffrey Goater, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2015 of Voyager Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) for the registrant and have:
  - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b. (Paragraph omitted pursuant to SEC Release Nos. 33-8238/34-47986 and 33-8392/34-49313);
  - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer and I have disclosed, based on my most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: December 17, 2015

/s/ J. Jeffrey Goater

J. Jeffrey Goater

*Senior Vice President, Finance and Business Development  
(Principal Financial Officer)*

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**CERTIFICATION PURSUANT TO  
18 U.S.C. SECTION 1350,  
AS ADOPTED PURSUANT TO  
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the quarterly report on Form 10-Q of Voyager Therapeutics, Inc. (the "Company") for the period ended September 30, 2015, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certify, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his or her knowledge:

- 1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
- 2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: December 17, 2015

/s/ Steven M. Paul

Steven M. Paul  
*President and Chief Executive Officer*  
*(Principal Executive Officer)*

Date: December 17, 2015

/s/ J. Jeffrey Goater

J. Jeffrey Goater  
*Senior Vice President, Finance and Business Development*  
*(Principal Financial Officer)*

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