CATABASIS PHARMACEUTICALS INC
Form 10-Q
November 13, 2018
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2018

OR

o 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: 001-37467

Catabasis Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**26-3687168**
(State or Other Jurisdiction of Incorporation or Organization)	(IRS Employer Identification No.)
One Kendall Square Bldg. 1400E, Suite B14202 Cambridge, Massachusetts	**02139**
(Address of Principal Executive Offices)	(Zip Code)

(617) 349-1971

(Registrant s Telephone Number, Including Area Code)

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** x **No** o

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). **Yes** x **No o**

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

Large accelerated filer o	Accelerated filer o
Non-accelerated filer x	Smaller reporting company x

Emerging growth company x

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

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

As of October 31, 2018, there were 71,038,419 shares of the registrant s Common Stock, par value $0.001 per share, outstanding.

Table of Contents

TABLE OF CONTENTS

Table of Contents

CAUTIONARY NOTE CONCERNING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Quarterly Report on Form 10-Q, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans and objectives of management and expected market growth are forward-looking statements. The words anticipate, believe, continue, could, estimate, expect, intend, may, plan, potential, should, target, would and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

These forward-looking statements include, among other things, statements about:

- our expectations regarding our ability to successfully conduct the PolarisDMD trial, and our expectations regarding the timing, design and results of such trial, including reporting top-line results of this trial in the second quarter of 2020 and the potential consistency of data produced by this trial with prior results from our MoveDMD® trial, as well as any new data and analyses relating to the safety profile and potential clinical benefits of edasalonexent;
- our plans to identify, develop and commercialize novel therapeutics based on our SMART Linkers℠ drug discovery platform;
- ongoing and planned clinical trials for edasalonexent and other product candidates, whether conducted by us or by any future collaborators, including the timing of initiation of these trials and of the anticipated results;
- our plans to enter into collaborations for the development and commercialization of product candidates;
- the potential benefits of any future collaboration;
- our ability to receive research and development funding and achieve anticipated milestones under any future collaborations;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our ability to identify additional products or product candidates with significant commercial potential;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;

- developments relating to our competitors and our industry; and
- the impact of government laws and regulations.

We may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, and you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the Risk Factors section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements

Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Balance Sheets

(In thousands, except share and per share data)

(Unaudited)

	September 30, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 19,876	$ 16,369
Short-term investments	23,363	
Prepaid expenses and other current assets	1,302	1,094
Total current assets	44,541	17,463
Property and equipment, net	45	321
Restricted cash	113	113
Total assets	$ 44,699	$ 17,897
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 1,047	$ 773
Accrued expenses	2,730	2,432
Current portion of notes payable, net of discount		2,479
Other liability	348	332
Total current liabilities	4,125	6,016
Deferred rent, net of current portion	55	89
Total liabilities	4,180	6,105
Commitments (Note 7)		
Stockholders equity:		
Preferred stock, $0.001 par value per share, 5,000,000 shares authorized and no shares issued and outstanding		
Common stock, $0.001 par value per share, 150,000,000 shares authorized; 71,038,419 and 23,645,247 shares issued and outstanding at September 30, 2018 and December 31, 2017, respectively	71	24
Additional paid-in capital	231,697	183,202
Accumulated other comprehensive loss	(5)	
Accumulated deficit	(191,244)	(171,434)
Total stockholders equity	40,519	11,792
Total liabilities and stockholders equity	$ 44,699	$ 17,897

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations

(In thousands, except share and per share data)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Revenue	$	$ 250	$	$ 250
Operating expenses:				
Research and development	3,897	4,776	13,383	14,693
General and administrative	2,111	2,426	6,900	7,189
Total operating expenses	6,008	7,202	20,283	21,882
Loss from operations	(6,008)	(6,952)	(20,283)	(21,632)
Other income (expense):				
Interest expense	(10)	(105)	(100)	(381)
Interest and investment income	177	45	252	128
Other income (loss), net	162	(5)	321	18
Total other income (expense), net	329	(65)	473	(235)
Net loss	$ (5,679)	$ (7,017)	$ (19,810)	$ (21,867)
Net loss per share - basic and diluted	$ (0.08)	$ (0.31)	$ (0.45)	$ (1.03)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	71,038,419	22,563,174	43,603,950	21,163,591

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Statements of Comprehensive Loss

(In thousands)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Net loss	$ (5,679)	$ (7,017)	$ (19,810)	$ (21,867)
Other comprehensive income:				
Unrealized (losses) gains on short-term investments	(5)		(5)	4
Total other comprehensive income:	(5)		(5)	4
Comprehensive loss	$ (5,684)	$ (7,017)	$ (19,815)	$ (21,863)

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Statements of Cash Flows

(In thousands)

(Unaudited)

	Nine Months Ended September 30,	
	2018	**2017**
Operating activities		
Net loss	$ (19,810)	$ (21,867)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	111	237
Stock-based compensation expense	1,340	1,501
Accretion of discount/premium on investment securities	(4)	26
Non-cash interest expense	37	128
Gain on disposal of property and equipment	(297)	(30)
Services received in non-monetary exchange	8	
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(120)	(98)
Accounts payable	274	(203)
Accrued expenses	267	(903)
Deferred rent	(3)	10
Net cash used in operating activities	(18,197)	(21,199)
Investing activities		
Purchases of short-term investments	(39,364)	
Sales and maturities of short-term investments	16,000	14,910
Purchases of property and equipment		(57)
Proceeds from sale of property and equipment	365	30
Net cash (used in) provided by investing activities	(22,999)	14,883
Financing activities		
Proceeds from public offering, net of issuance costs	38,886	
Proceeds from at-the-market offering, net of issuance costs	8,313	6,910
Proceeds from exercise of common stock options and warrants	4	23
Payments on borrowing	(2,500)	(2,500)
Net cash provided by financing activities	44,703	4,433
Net increase (decrease) in cash, cash equivalents and restricted cash	3,507	(1,883)
Cash, cash equivalents and restricted cash, beginning of period	16,482	23,709
Cash, cash equivalents and restricted cash, end of period	$ 19,989	$ 21,826
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 79	269

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Notes to Condensed Consolidated Financial Statements

(Unaudited)

1. Organization and Operations

The Company

Catabasis Pharmaceuticals, Inc. (the Company) is a clinical-stage biopharmaceutical company. The Company s lead program is edasalonexent, formerly known as CAT-1004, an oral small molecule designed to inhibit NF-kB, or nuclear factor kappa-light-chain-enhancer of activated B cells, in development for the treatment of Duchenne muscular dystrophy (DMD). The Company believes edasalonexent has the potential to be a foundational therapy for all patients affected by DMD, regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. The United States Food and Drug Administration has granted orphan drug, fast track and rare pediatric disease designations to edasalonexent for the treatment of DMD. The European Commission has granted orphan medicinal product designation to edasalonexent for the treatment of DMD. The Company was incorporated in the State of Delaware on June 26, 2008.

Liquidity

In October 2017, the Company entered into a sales agreement with Cowen and Company LLC (Cowen) pursuant to which the Company could issue and sell shares of common stock for an aggregate maximum offering amount of $10.0 million under an at-the-market offering program (the ATM Program). Shares sold pursuant to the sales agreement were sold pursuant to a shelf registration statement, which became effective on July 19, 2016. The Company paid Cowen 3% of the gross proceeds from any common stock sold through these sales agreements.

During the nine months ended September 30, 2018, the Company sold an aggregate of 5,390,255 shares of common stock pursuant to the ATM Program, at an average price of $1.59 per share, for gross proceeds of $8.6 million, resulting in net proceeds of $8.3 million after deducting sales commissions and offering expenses. The ATM Program was fully utilized as of February 2018.

On June 20, 2018, the Company entered into an underwriting agreement with Oppenheimer & Co. Inc. relating to an underwritten public offering (the June 2018 Financing) of 42,000,000 shares of the Company s common stock, par value $0.001 per share, and accompanying warrants to purchase up to 42,000,000 shares of common stock, at a combined price to the public of $1.00 per share, for gross proceeds of approximately $42.0 million, and net proceeds of $38.9 million.

As of September 30, 2018, the Company had an accumulated deficit of $191.2 million. The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since its inception.

The Company is subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its drug candidates, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology and regulatory approval and market acceptance of the Company s products. The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidates.

As of September 30, 2018, the Company had available cash, cash equivalents and short-term investments of $43.2 million. Based on the Company s current operating plan, the Company believes it has sufficient cash, cash equivalents and short-term investments to fund operations into the second quarter of 2020, and therefore the substantial doubt about the Company s ability to continue as a going concern disclosed in the Company s annual report on Form 10-K filed with the Securities and Exchange Commission on March 15, 2018 (the 2017 Annual Report on Form 10-K) has been alleviated.

Table of Contents

2. Summary of Significant Accounting Policies

Basis of Presentation and Principles of Consolidation

The accompanying financial statements and the related disclosures are unaudited and have been prepared in accordance with United States generally accepted accounting principles (U.S. GAAP). Additionally, certain information and footnote disclosures normally included in the Company s annual financial statements have been condensed or omitted from this report. Accordingly, these condensed financial statements should be read in conjunction with the financial statements as of and for the year ended December 31, 2017 and notes thereto included in the 2017 Annual Report on Form 10-K.

Apart from the adoption of Accounting Standards Update (ASU) 2016-18, *Statement of Cash Flows (Topic 230) Restricted Cash* (ASU 2016-18) as described below, the unaudited interim condensed consolidated financial statements have been prepared on the same basis as the audited financial statements. In the opinion of the Company s management, the accompanying unaudited interim condensed consolidated financial statements contain all adjustments which are necessary to fairly present the Company s financial position as of September 30, 2018, the results of its operations for the three and nine months ended September 30, 2018 and 2017 and its cash flows for the nine months ended September 30, 2018 and 2017. Such adjustments are of a normal and recurring nature. The results for the three and nine months ended September 30, 2018 are not necessarily indicative of the results for the year ending December 31, 2018, or for any future period.

The accompanying condensed consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary, Catabasis Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation.

Use of Estimates

The preparation of the Company s condensed consolidated financial statements in conformity with U.S. GAAP requires management to make estimates and assumptions that affect the amounts reported in the condensed consolidated financial statements and accompanying notes. Actual results could differ from such estimates.

The Company utilizes certain estimates to record expenses relating to research and development contracts. These contract estimates, which are primarily related to the length of service of each contract and the amount of service provided as of each measurement date, are determined by the Company based on input from internal project management, as well as from third-party service providers.

Stock-Based Compensation

The Company accounts for its stock-based compensation awards in accordance with Accounting Standards Codification (ASC) Topic 718, *Compensation Stock Compensation (* ASC 718). ASC 718 requires all share-based payments to employees, including grants of employee stock

options, to be recognized in the statements of operations based on their grant date fair values. For stock options granted to employees and to members of the board of directors for their services on the board of directors, the Company estimates the grant date fair value of each option award using the Black-Scholes option-pricing model. The use of the Black-Scholes option-pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the Common Stock consistent with the expected term of the option, risk-free interest rates and expected dividend yields of the Common Stock.

For awards subject to service-based vesting conditions, the Company recognizes stock-based compensation expense equal to the grant date fair value of stock options on a straight-line basis over the requisite service period.

The Company expenses restricted stock awards based on the fair value of the award on a straight-line basis over the associated service period of the award.

Share-based payments issued to non-employees are recorded at their fair values, and are periodically revalued as the equity instruments vest and are recognized as expense over the related service period in accordance with the provisions of ASC Topic 505, *Equity*. For equity instruments granted to non-employees, the Company recognizes stock-based compensation expense on a straight-line basis.

During the three and nine months ended September 30, 2018 and 2017, the Company recorded stock-based compensation expense for employee and non-employee stock options, which was allocated as follows in the condensed consolidated statements of operations (in thousands):

Table of Contents

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Research and development	$ 168	$ 198	$ 505	$ 595
General and administrative	248	317	835	906
Total	$ 416	$ 515	$ 1,340	$ 1,501

Net Loss Per Share

Basic net loss per share is calculated by dividing net loss by the weighted average shares outstanding during the period, without consideration for Common Stock equivalents. Diluted net loss per share is calculated by adjusting weighted average shares outstanding for the dilutive effect of Common Stock equivalents outstanding for the period, determined using the treasury-stock method. For purposes of the Company s dilutive net loss per share calculation, stock options and warrants to purchase Common Stock were considered to be Common Stock equivalents but were excluded from the calculation of diluted net loss per share, as their effect would be anti-dilutive; therefore, basic and diluted net loss per share were the same for all periods presented.

The following Common Stock equivalents were excluded from the calculation of diluted net loss per share for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2018	2017	2018	2017
Stock options	4,489,457	2,839,407	4,489,457	2,839,407
Common stock warrants	42,024,566	24,566	42,024,566	24,566
	46,514,023	2,863,973	46,514,023	2,863,973

Cash, Cash Equivalents and Restricted Cash

The reconciliation of cash, cash equivalents and restricted cash reported within the applicable balance sheet that sum to the total of the same such amount shown in the statement of cash flows is as follows:

	September 30,	
	2018	2017
Cash and cash equivalents	$ 19,876	$ 21,713
Restricted cash	113	113
Total	$ 19,989	$ 21,826

Recent Accounting Pronouncements - Adopted

In October 2016, the Financial Accounting Standards Board (FASB) issued ASU 2016-18, which requires that a statement of cash flows explain the change during the period in the total of cash, cash equivalents, and restricted cash or restricted cash equivalents. Therefore, amounts described as restricted cash and restricted cash equivalents should be included with cash and cash equivalents when reconciling the

beginning-of-period and end-of-period total amounts shown on the statement of cash flows. The Company adopted ASU 2016-18 in the period beginning January 1, 2018. Upon adoption of ASU 2016-18 the Company revised the presentation as well as caption of certain items within the unaudited condensed consolidated statements of cash flows to conform to the current period presentation. These revisions had no impact on the net cash used in operating activities or cash, cash equivalents and restricted cash at end of period.

In February 2018, the FASB issued ASU 2018-03, *Recognition and Measurement of Financial Assets and Financial Liabilities*. This standard made improvements to address certain aspects of recognition, measurement, presentation, and disclosure of financial instruments. This amendment is effective for annual reporting periods beginning after December 15, 2017, and interim reporting periods beginning after June 15, 2018. The Company adopted the standard in the period ending September 30, 2018. As of September 30, 2018, there was no material impact on the Company s consolidated financial statements, and any future impact from adoption will be dependent on future transactions involving financial assets and financial liabilities.

Table of Contents

Recent Accounting Pronouncements - Not Yet Adopted

From time to time, new accounting pronouncements are issued by the FASB or other standard setting bodies and adopted by the Company as of the specified effective date. Unless otherwise discussed, the Company believes that the impact of recently issued standards that are not yet effective will not have a material impact on its financial position or results of operations upon adoption.

In February 2016, the FASB issued ASU 2016-02, *Leases*. This standard amends the existing guidance to require lessees to present most leases on their balance sheets but recognize corresponding expenses on their statements of operations. The FASB added a transition option to the new leases standard that allows entities to not apply the new guidance in the comparative periods they present in their financial statements in the year of adoption. The FASB also provided a practical expedient that gives lessors an option to combine non-lease and associated lease components when certain criteria are met and requires a lessor to account for the combined component in accordance with the new revenue standard if the associated non-lease components are the predominant component. This standard is effective for annual reporting periods beginning after December 15, 2018, but early adoption is permitted. The Company will adopt this standard as of January 1, 2019 using the modified retrospective approach recording any cumulative adjustment to retained earnings. The adoption of this standard is expected to have a material impact on lease assets and lease liabilities but will not materially impact consolidated net earnings. The Company will elect a package of practical expedients permitted under the transition guidance within the new standard, which among other things, allows the Company to carryforward the historical lease classification.

In July 2018, the FASB issued ASU 2018-07, *Compensation- Stock Compensation (Topic 718)*. This standard simplifies several areas of the accounting for nonemployee share-based payment transactions. This amendment is effective for annual reporting periods beginning after December 15, 2018, and the Company adopted this standard early on October 1, 2018. The Company does not expect any material impact on the Company s consolidated financial statements.

In August 2018, the FASB issued ASU 2018-13, *Fair Value Measurement (Topic 820)*. This standard includes amendments regarding changes in unrealized gains and losses, the range and weighted average of significant unobservable inputs used to develop Level 3 fair value measurements, and disclosure requirements of measurement uncertainty. This amendment is effective for annual reporting periods beginning after December 15, 2019. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

Summary of Significant Accounting Policies

The Company s significant accounting policies are described in Note 2, Summary of Significant Accounting Policies, in the 2017 Annual Report on Form 10-K, and there were no significant changes to such policies in the nine months ended September 30, 2018 that had a material impact on the Company s results of operations or financial position.

3. Financial Instruments

The tables below present information about the Company s assets and liabilities that are measured at fair value on a recurring basis as of September 30, 2018 and December 31, 2017 and indicate the fair value hierarchy of the valuation techniques the Company utilized to determine

such fair value. In general, fair values determined by Level 1 inputs utilize observable inputs such as quoted prices in active markets for identical assets or liabilities. Fair values determined by Level 2 inputs utilize data points that are either directly or indirectly observable, such as quoted prices, interest rates and yield curves. Fair values determined by Level 3 inputs utilize unobservable data points for which there is little or no market data, which require the Company to develop its own assumptions for the asset or liability. There were no transfers between fair value measurement levels during the nine months ended September 30, 2018 or 2017.

The Company s investment portfolio may include fixed income securities that do not always trade on a daily basis. As a result, the pricing services used by the Company apply other available information as applicable through processes such as benchmark yields, benchmarking of like securities, sector groupings and matrix pricing to prepare valuations. The Company validates the prices provided by its third party pricing services by obtaining market values from other pricing sources and analyzing pricing data in certain instances. The Company also invests in certain reverse repurchase agreements which are collateralized by deposits in the form of U.S. Government Securities and Obligations for an amount no less than 102% of their value. The Company does not record an asset or liability for the collateral as the Company is not permitted to sell or re-pledge the collateral. The collateral has at least the prevailing credit rating of U.S. Government Treasuries and Agencies. The Company utilizes a third party custodian to manage the exchange of funds and ensure that collateral received is maintained at 102% of the value of the reverse repurchase agreements on a daily basis.

Below is a summary of assets measured at fair value on a recurring basis (in thousands):

Table of Contents

	As of September 30, 2018			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market funds	$ 9,427	$	$	$ 9,427
Corporate debt securities		1,244		1,244
U.S reverse repurchase agreements		3,000		3,000
Short-term investments:				
Corporate debt securities		10,363		10,363
U.S reverse repurchase agreements		13,000		13,000
Total assets	$ 9,427	$ 27,607	$	$ 37,034

	As of December 31, 2017			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market funds	$ 13,588	$	$	$ 13,588
Total assets	$ 13,588	$	$	$ 13,588

At September 30, 2018 the Company s cash equivalents consisted of money market funds, U.S. reverse repurchase agreements, and corporate debt securities. At December 31, 2017, the Company s cash equivalents consisted of money market funds. At September 30, 2018, and December 31, 2017, cash equivalents approximated their fair value due to their short-term nature.

At December 31, 2017, the carrying value of the Company s debt approximated fair value, which was determined using Level 3 inputs, including a quoted interest rate. As of September 30, 2018, the Company had paid the principal balance of the term loan.

4. Short-Term Investments

As of December 31, 2017, the Company held no short-term investments. The following table summarizes the short-term investments securities held at September 30, 2018 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
September 30, 2018				
Corporate debt securities	$ 10,368	$	$ (5)	$ 10,363
U.S reverse repurchase agreements	13,000			13,000
Total	$ 23,368	$	$ (5)	$ 23,363

The contractual maturities of all short-term investments held at September 30, 2018 were one year or less. There were ten short-term investments in an unrealized loss position at September 30, 2018, none of which had been in an unrealized loss position for more than 12 months. The aggregate fair value of these investments at September 30, 2018 was approximately $9.4 million. The Company did not hold any investments with other-than-temporary impairment at September 30, 2018.

Table of Contents

Gross realized gains and losses on the sales of short-term investments are included in other income, net. Unrealized holding gains or losses for the period that have been included in accumulated other comprehensive income, as well as gains and losses reclassified out of accumulated other comprehensive income into other income, net were not material to the Company s condensed consolidated results of operations. The cost of investments sold or the amount reclassified out of the accumulated other comprehensive income into other income, net is based on the specific identification method for purposes of recording realized gains and losses. There were no proceeds from sales of short-term investments in the three and nine-month periods ended September 30, 2018 and 2017. All proceeds in the three and nine-month periods ended September 30, 2018 and the nine-month period ended September 30, 2017 related to maturities of underlying investments. The gains on proceeds from maturities of short-term investments were not material to the Company s condensed consolidated results of operations for the three or nine months periods ended September 30, 2018 and 2017.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2018	December 31, 2017
Accrued compensation	$ 1,072	$ 632
Accrued contracted research costs	1,059	1,357
Accrued professional fees	193	298
Accrued severence	204	
Accrued other	202	145
Total	$ 2,730	$ 2,432

6. Notes Payable

On August 27, 2014, the Company entered into a credit facility with MidCap Financial Trust, Flexpoint MCLS SPV LLC and Square 1 Bank, which was subsequently amended in March and December 2015 (as amended, the Credit Facility). The Credit Facility provided for maximum borrowings of $25.0 million. The Company received total proceeds of $10.0 million under term loans and the remaining amounts available for borrowing under this arrangement expired unused as of July 31, 2015. All amounts outstanding under the Credit Facility were due on October 1, 2018 and were collateralized by substantially all of the Company s personal property, other than its intellectual property.

As of September 30, 2018, principal and interest due under the Credit Facility had been paid. A final payment equal to 3.48% of the amounts drawn under the Credit Facility was due upon the earlier of the maturity date, acceleration of the term loans or prepayment of all or part of the term loans. The final payment was accrued as additional interest expense using the effective-interest method from the date of issuance through the maturity date and recorded within other current liabilities. The final payment was made in October 2018.

The Company recognized $10 thousand and $0.1 million of interest expense related to the Credit Facility in the three-month periods ending September 30, 2018 and 2017, respectively. The Company recognized $0.1 million and $0.4 million of interest expense related to the Credit Facility in the nine-month periods ending September 30, 2018 and 2017, respectively.

7. Commitments

In November 2010, the Company entered into an operating lease agreement for office and laboratory space, which has been amended multiple times. Based on the latest amendment, the lease agreement includes escalating rent payments and is effective through June 30, 2020. The Company is recognizing rent expense on a straight-line basis over the lease term.

Future minimum payments required under the non-cancelable operating lease as of September 30, 2018 are summarized as follows (in thousands):

Table of Contents

Period Ending December 31,	Amount
2018	350
2019	1,421
2020	721
Total minimum lease payments	$ 2,492

During the three months ended September 30, 2018 and 2017, the Company recognized $0.3 million and $0.4 million in rent expense, respectively. The Company recognized $1.0 million in rent expense for each of the nine months ended September 30, 2018 and 2017.

The Company is party to a sublease with Inzen Therapeutics, Inc. (Izen), which became effective on October 15, 2018. See further discussion in Note 13.

8. Revenue Recognition

In August 2017, the Company entered into an option agreement (Option Agreement) with an unaffiliated party (Recipient), which is within the scope of ASC Topic 606: *Revenue from Contracts with Customers*. Under the terms of the Option Agreement, the Company agreed to provide compound material for certain of its product candidates and the right for the Recipient to use the material to perform research during the term of the Option Agreement. The Option Agreement included a non-refundable up-front payment to the Company of $250 thousand. The term of the Option Agreement commenced in August 2017 and could be extended upon additional payments of $250 thousand made by the Recipient, at its option, at the beginning of each of the next two quarterly periods. In October 2017, the Company received an additional $250 thousand payment to extend the term for an additional quarterly period. In December 2017, the Recipient terminated the agreement. In the future, the Company will seek to generate additional revenue primarily from a combination of product sales and collaborations with strategic partners.

During the three and nine months ended September 30, 2017, the Company recognized $250 thousand in revenue related to the Option Agreement, respectively. The Company did not recognize revenue in the three and nine months ended September 30, 2018.

9. Stockholders Equity

Preferred Stock

As of September 30, 2018, the Company had 5,000,000 shares of preferred stock authorized for issuance, $0.001 par value per share, with none issued or outstanding. Preferred stock may be issued from time to time in one or more series, each series to have such terms as stated or expressed in the resolutions providing for the issue of such series adopted by the board of directors of the Company. Preferred stock which may be redeemed, purchased or acquired by the Company may be reissued except as otherwise provided by law.

Common Stock Warrants

June 2018 Warrants

In the June 2018 Financing, the Company issued warrants to purchase 42,000,000 shares of common stock at an exercise price of $1.20 per share, which were immediately exercisable upon issuance, and will expire five years from the date of issuance.

The terms of the warrants include certain provisions related to fundamental transactions, a cashless exercise provision in the event registered shares are not available and do not include any mandatory redemption provisions. Therefore, the warrants have been classified in stockholders equity. Any changes to fair value of the warrants will not be recognized so long as the warrants continue to be equity classified.

As of September 30, 2018, all warrants related to this transaction were outstanding with a contractual life of 4.73 years.

Warrants Associated with the Term Loan

In conjunction with the term loans (Note 6), the Company issued warrants to purchase 315,688 shares of series B convertible preferred stock. Upon the closing of the Company s initial public offering (the IPO) in June 2015, the warrants were automatically converted into warrants to purchase an aggregate of 24,566 shares of Common Stock at an exercise price of $12.21 per share. As of September 30, 2018, all warrants related to this transaction were outstanding with a weighted average contractual life of 3.20 years.

Table of Contents

10. Common Stock Reserved for Future Issuance

The Company has reserved for future issuance the following shares of Common Stock:

	As of September 30,	
	2018	2017
Warrants for the purchase of Common Stock	42,024,566	24,566
Options to purchase Common Stock	4,612,684	3,669,791
Employee Stock Purchase Plan	760,111	523,659
Total	47,397,361	4,218,016

11. Stock Incentive Plans

Prior to the IPO, the Company granted awards to eligible participants under its 2008 Equity Incentive Plan (2008 Plan). In May 2015, the Company s board of directors adopted and, in June 2015, the Company s stockholders approved the 2015 Stock Incentive Plan (2015 Plan), which became effective immediately prior to the effectiveness of the IPO. Subsequent to the IPO, option grants are awarded to eligible participants only under the 2015 Plan.

As of September 30, 2018, the Company had reserved 735,635 shares of Common Stock under the 2008 Plan, of which none remained available for future issuance. As of September 30, 2018, the Company had reserved 3,753,822 shares of Common Stock under the 2015 Plan, of which 123,227 shares remained available for future issuance. Under the 2015 Plan, stock options may not be granted with exercise prices at less than fair value on the date of the grant.

Terms of stock option agreements, including vesting requirements, are determined by the Company s board of directors, subject to the provisions of the applicable stock incentive plan. Options granted by the Company generally vest ratably over four years, with a one-year cliff, and options are exercisable from the date of grant for a period of ten years. For options granted through September 30, 2018 the exercise price or purchase price, as applicable, equaled the estimated fair value of the Common Stock as determined by the Company s board of directors on the date of grant.

A summary of the Company s stock option activity and related information for employees and nonemployees follows:

	Shares	Weighted-Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2017	2,845,626	$ 4.53	7.75	$ 216
Granted	2,133,845	$ 0.98		
Exercised	(2,917)	$ 1.24		

Cancelled or forfeited	(487,097)	$	2.93			
Outstanding at September 30, 2018	4,489,457	$	3.02	8.19	$	102
Vested and Exercisable at September 30, 2018	1,734,044	$	5.24	6.54	$	

There were no options exercised in the three months ended September 30, 2018 and 2017. The total intrinsic value of options exercised for the nine months ended September 30, 2018 and 2017 was $1 thousand and $41 thousand, respectively. The total fair value of employee and non-employee options vested for the three months ended September 30, 2018 and 2017 was $0.3 million and $0.6 million, respectively. The total fair value of employee and non-employee options vested for the nine months ended September 30, 2018 and 2017 was $1.4 million and $1.8 million, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the three months ended September 30, 2018 and 2017 was $0.46 and $0.86, respectively. The weighted-average

Table of Contents

grant date fair value of options granted to employees and non-employees for the nine months ended September 30, 2018 and 2017 was $0.66 and $0.85, respectively.

At September 30, 2018, the total unrecognized compensation expense related to unvested stock option awards was $2.7 million. The Company expects to recognize that cost over a weighted-average period of approximately 1.8 years.

12. Restructuring Costs

In April 2018, the Company announced a strategic shift to focus resources on its lead program, edasalonexent. Consequently, the Company reduced its workforce by 40% during the quarter ended June 30, 2018. Pursuant to ASC 420, *Exit or Disposal Cost Obligations*, charges for employee severance, employee benefits, consolidation of facilities and contract terminations were recorded in the nine months ended September 30, 2018.

The following table summarizes the impact of the April 2018 restructuring activities for the nine months ended September 30, 2018 along with the current liability recorded in the balance sheet as of September 30, 2018 (in thousands):

	Charges Incurred during the Nine Months Ended September 30, 2018	Amount Paid through September 30, 2018	Remaining Liability at September 30, 2018
Employee severance, benefits and related costs	$ 786	$ 582	$ 204
Consolidation of facilities	98	98	
Contract terminations	16	16	
	$ 900	$ 696	$ 204

There were no expenses related to the April 2018 restructuring recognized in the three months ended September 31, 2018. Of the expenses recognized in the nine months ending September 30, 2018, $0.4 million was recorded in the general and administrative section and $0.5 million was recorded in the research and development section of the accompanying condensed consolidated statement of operations.

The Company also recorded a net gain of $0.3 million in connection with the sale or exchange of assets disposed of during the consolidation and relocation of facilities. This net gain has been recorded as a component of other income, (net), in the accompanying consolidated statement of operations.

13. Subsequent Events

The Company considers events or transactions that occur after the balance sheet date but prior to the issuance of the financial statements to provide additional evidence for certain estimates and to identify matters that require additional disclosure. Subsequent events have been

evaluated as required.

The Company is party to a sublease with Inzen, as subtenant, to sub-lease 14,817 square feet of the Company s facility (the Sublease). The Sublease became effective on October 15, 2018, and the term of the Sublease is from October 15, 2018 through June 30, 2020. Under the terms of the sublease, Inzen is obligated to pay the Company approximately $1.8 million in base rent and an additional $0.5 million in operating expenses.

Table of Contents

Item 2. Management s Discussion and Analysis of Financial Condition and Results of Operations

You should read the following discussion and analysis of our financial condition and results of operations together with the unaudited condensed consolidated financial statements and the related notes appearing elsewhere in this Quarterly Report on Form 10-Q. 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.

Overview

We are a clinical-stage biopharmaceutical company. Our lead program is edasalonexent, formerly known as CAT-1004, an oral small molecule designed to inhibit NF-κB, or nuclear factor kappa-light-chain-enhancer of activated B cells, in development for the treatment of Duchenne muscular dystrophy, or DMD. We believe edasalonexent has the potential to be a foundational therapy for all patients affected by DMD, regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. The United States Food and Drug Administration, or FDA, has granted orphan drug, fast track and rare pediatric disease designations to edasalonexent for the treatment of DMD. The European Commission, or EC, has granted orphan medicinal product designation to edasalonexent for the treatment of DMD.

We initiated a global Phase 3 trial for the treatment of DMD in September 2018, which we refer to as the PolarisDMD trial. The PolarisDMD trial is designed to evaluate the efficacy and safety of edasalonexent for registration purpose, with top-line results expected in the second quarter of 2020. PolarisDMD enrollment in the United States across approximately 25 sites is expected to run through the remainder of this year and into 2019. Additional sites in Australia, Canada, Europe and Israel are also expected to open for enrollment early next year. In total, the PolarisDMD trial is expected to include approximately 40 clinical trial sites globally. The trial design was informed by discussions with the FDA, as well as input from treating physicians, families of boys affected by DMD and patient advocacy organizations.

The PolarisDMD trial is a randomized, double-blind, placebo-controlled trial, and we anticipate enrolling approximately 125 patients between the ages of four and seven (up to eighth birthday), regardless of mutation type, who have not been on steroids for at least six months. Boys may be eligible to enroll in the trial if they are on a stable dose of EXONDYS 51®, also known as eteplirsen, Sarepta Therapeutics, Inc. s exon skipping therapy and one of two therapies approved for the treatment of DMD in the United States. The primary efficacy endpoint is change in North Star Ambulatory Assessment, or NSAA, score after 12 months of treatment with edasalonexent compared to placebo. Key secondary endpoints are the age-appropriate timed function tests: time to stand, 4-stair climb and 10-meter walk/run. Assessments of growth, cardiac and bone health are also planned. Enrolled boys are being randomized in a 2:1 ratio with two boys receiving edasalonexent for every boy that receives placebo, and we expect that after the initial 12-month treatment period all boys will be offered the opportunity to receive edasalonexent in an open-label extension.

Our MoveDMD® Phase 1/2 trial enrolled ambulatory boys four to seven years old with a genetically confirmed diagnosis of DMD who were steroid naive or had not used steroids for at least six months prior to the trial. Boys enrolled in the trial were not limited to any specific dystrophin mutations and the 31 boys in the trial had 26 different dystrophin mutations. The MoveDMD trial was designed to be conducted in three sequential parts, Phase 1 and Phase 2, both of which are completed, and an open-label extension, which is on-going. In Phase 1 of the MoveDMD trial, we assessed the safety, tolerability and pharmacokinetics of edasalonexent in 17 patients, following seven days of dosing, and we reported in January 2016 that all three doses of edasalonexent tested were generally well tolerated with no safety signals observed and there were no serious adverse events and no drug discontinuations. In the Phase 2 portion of the trial, we assessed the effects of edasalonexent using magnetic resonance imaging, or MRI, T2 as an early biomarker at 12 weeks, and announced in January 2017 that the primary efficacy endpoint of average change from baseline to week 12 in the MRI T2 composite measure of lower leg muscles for the pooled edasalonexent treatment groups compared to placebo was not met, although we observed directionally positive results in the 100/mg/kg/day edasalonexent treatment group that were not statistically significant.

We have completed key efficacy and safety assessments from the MoveDMD trial. In the open label extension of the MoveDMD trial through 72 weeks of oral 100 mg/kg/day edasalonexent treatment, we observed preserved muscle function and consistent improvements in all four assessments of muscle function: NSAA score, time to stand, 4-stair climb and 10-meter walk/run, compared to the rates of change in the control period for boys prior to receiving edasalonexent treatment. Additionally, supportive changes in non-effort-based measures of muscle health were seen, supporting the durability of edasalonexent treatment effects. Specifically, we observed statistically significant improvement in the rate of change in lower leg composite MRI T2

Table of Contents

through 12, 24, 36 and 48 weeks on 100 mg/kg of edasalonexent treatment compared to the off-treatment control period. MRI T2 is closely associated with functional outcomes in DMD supported by data from ImagingDMD, the largest natural history database of MRI assessments in boys with DMD.

The relative proportion of fat in muscle, which is referred to as fat fraction and is correlated with functional ability, can also be determined by magnetic resonance spectroscopy, or MRS. Improvements in the MRS fat fraction rate of change through 48 weeks of edasalonexent treatment compared to the off-treatment control period were observed in both soleus and vastus lateralis leg muscles, which are strongly correlated with ambulatory function. Additionally, boys with DMD in the age range enrolled in the trial typically have resting tachycardia, a heart rate that exceeds the normal resting rate, and we observed that the heart rate of the boys treated with edasalonexent significantly decreased toward age-normative values over a year and a half period of edasalonexent treatment. Significant decreases in muscle enzymes through 72 weeks were also seen in boys treated with edasalonexent, which is consistent with a positive impact on muscle health and supportive of a positive impact from the treatment with edasalonexent.

Through 72 weeks of treatment, edasalonexent continued to be well tolerated with no safety signals observed in the MoveDMD trial. Boys treated with edasalonexent continued to follow age-appropriate growth curves with age-appropriate increases in weight and height and overall body mass index trended down to age-normative values.

We also are evaluating edasalonexent for the potential treatment of Becker muscular dystrophy, or BMD, a related disease where edasalonexent therapy may be beneficial. BMD is a rare disease, and patients with BMD express low levels of dystrophin due to mutations in the dystrophin gene. Dystrophin production is reduced through the NF-κB-mediated induction of microRNAs that inhibit dystrophin translation. Inhibition of NF-κB in BMD directly enhances dystrophin production. We are currently investigating potential approaches for clinical trials in BMD.

On November 13, 2018, we announced a collaboration with University of Texas Southwestern, or UT Southwestern, to explore the potential of edasalonexent to improve cardiac function in DMD and BMD. This is a one-year preclinical collaboration with Pradeep Mammen, MD, FACC, FAHA, founder and Medical Director of the Neuromuscular Cardiomyopathy Clinic at UT Southwestern Medical Center as well as Co-Director of the National Institute of Health Sponsored UT Southwestern Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center. Cardiomyopathy is the leading cause of mortality in DMD and BMD. Preclinical and clinical data support the potential for cardiac benefits with edasalonexent in DMD and BMD.

In addition to edasalonexent, we have developed additional product candidates for rare diseases, including CAT-5571, a potential treatment for cystic fibrosis, or CF. CAT-5571 is a small molecule that is designed to activate autophagy, a mechanism for recycling cellular components and digesting pathogens, which is important for host defenses and is depressed in CF. We have completed investigational new drug, or IND, application-enabling activities for CAT-5571.

As of September 30, 2018, we owned six issued U.S. patents with composition of matter and method of use claims directed to edasalonexent and four issued U.S. patents with composition of matter and method of use claims directed to CAT-5571. These patents are expected to expire between 2029 and 2030, without taking into account potential patent term extensions. In addition, our patent portfolio includes over 70 issued foreign patents, four pending U.S. patent applications, three PCT applications, and 19 pending foreign patent applications. This patent portfolio does not include a number of patents and patent applications related to the development of certain product candidates other than those directed to edasalonexent and CAT-5571, because we have elected to abandon those patents or patent applications as part of our recent restructuring in April 2018.

Since our inception in June 2008, we have devoted substantially all of our resources to developing our proprietary platform technology, identifying potential product candidates, undertaking preclinical studies and conducting clinical trials for three clinical-stage compounds, building our intellectual property portfolio, organizing and staffing our company, business planning, raising capital, and providing general and administrative support for these operations. To date, we have primarily financed our operations through private placements of our preferred stock, registered offerings of our common stock, including our initial public offering, or IPO, as well as a secured debt financing. From our inception through September 30, 2018, we raised an aggregate of $245.0 million, of which $92.9 million was from private placements of preferred stock, $69.0 million represented gross proceeds from our IPO, $42.0 million represented gross proceeds from an underwritten public offering of common stock and warrants to purchase common stock in June 2018, or the June 2018 Financing, $11.5 million represented gross proceeds from a registered direct common stock offering, $10.0 million was from a secured debt financing, $18.8 million represented gross proceeds from at-the-market, or ATM, offerings, and $0.8 million was from common stock option and warrant exercises.

Financial Overview

Revenue

As of September 30, 2018, we have not generated any revenue from product sales.

Table of Contents

In August 2017, we entered into an option agreement, or the Option Agreement, with an unaffiliated party, or the Recipient, which is within the scope of Accounting Standards Codification, or ASC, Revenue from Contracts with Customers, or ASC 606. Under the terms of the Option Agreement, we agreed to provide compound material for certain of our product candidates and the right for the Recipient to use the material to perform research during the term of the Option Agreement. The Option Agreement included a non-refundable up-front payment to us of $250 thousand. The term of the Option Agreement commenced in August 2017 and could be extended upon additional payments of $250 thousand made by the Recipient, at its option, at the beginning of each of the next two quarterly periods. In October 2017, we received an additional $250 thousand payment to extend the term for an additional quarterly period. In December 2017, the Recipient terminated the agreement. In the future, we will seek to generate additional revenue primarily from a combination of product sales and collaborations with strategic partners.

Research and Development Expenses

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

- employee-related expenses including salaries, benefits and stock-based compensation expense;
- expenses incurred under agreements with third parties, including contract research organizations that conduct clinical trials and research and development and preclinical activities on our behalf;
- the cost of consultants;
- the cost of lab supplies and acquiring, developing and manufacturing preclinical study materials; and
- facilities and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

Research and development costs are expensed as incurred. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred and capitalized. The capitalized amounts are expensed as the related goods are delivered or the services are performed.

The following summarizes our most advanced current research and development programs:

- ***Edasalonexent for the treatment of DMD -*** Edasalonexent is a SMART Linker conjugate of salicylic acid and the omega-3 fatty acid docosahexaenoic acid, or DHA, a naturally occurring unsaturated fatty acid with anti-inflammatory properties. We designed edasalonexent to inhibit NF-κB, the key link between loss of dystrophin and disease pathology that plays a fundamental role in the initiation and progression of skeletal and cardiac muscle disease in DMD. We reported results from the Phase 1 portion of the MoveDMD trial in January 2016 and reported top-line safety and efficacy results for the 12-week placebo-controlled Phase 2 portion of the trial in January 2017. In July 2016, we initiated an open-label extension of the MoveDMD trial, which has provided safety and efficacy data through 24, 36, 48, 60 and 72 weeks of edasalonexent treatment, and we reported efficacy and safety results from the open-label extension in October 2017, February 2018, April 2018 and October 2018. We initiated the global Phase 3 PolarisDMD trial of edasalonexent for the treatment of DMD, regardless of mutation type, in the second half of 2018 with top-line results expected in the second quarter of 2020. The PolarisDMD trial is designed to evaluate the efficacy and safety of edasalonexent in patients with DMD and is intended to support an application for commercial

registration of edasalonexent.

- ***Edasalonexent for the treatment of BMD*** We are evaluating the potential benefits of edasalonexent treatment in BMD adults and investigating potential approaches for clinical trials in BMD.

- ***CAT-5571 -*** CAT-5571 is a SMART Linker conjugate that contains cysteamine, a naturally occurring molecule that is a degradation product of the amino acid cysteine, and DHA. CAT-5571 is a potential oral therapy to treat CF. CAT-5571 is a small molecule designed to restore host defense by activating autophagy, which is depressed in CF, to reestablish host defense to enhance the clearance of pathogens. People with CF suffer from persistent lung infections with opportunistic pathogens causing chronic infections that are difficult to eradicate and lead to respiratory failure. We have completed IND-enabling activities for CAT-5571.

We typically use our employee, consultant and infrastructure resources across our development programs. We track outsourced development costs by product candidate or development program, but we do not allocate personnel costs, other internal costs or external consultant costs to specific product candidates or development programs. We record our research and development expenses net of any research and development tax incentives we are entitled to receive from government authorities.

The following table summarizes our research and development expenses by program (in thousands):

Table of Contents

	Nine Months Ended September 30,	
	2018	2017
Edasalonexent	$ 5,720	$ 4,703
CAT-5571	526	1,814
Other research and platform programs	544	1,013
Costs not directly allocated to programs:		
Employee expenses including cash compensation, benefits and stock-based compensation	5,246	5,565
Facilities	810	944
Consultants and professional expenses, including stock-based compensation	240	125
Other	297	529
Total costs not directly allocated to programs	6,593	7,163
Total research and development expenses	$ 13,383	$ 14,693

Since inception of the edasalonexent and the CAT-5571 programs, total direct expenses to support the programs have been $35.1 million and $4.2 million, respectively.

The successful development of our product candidates is highly uncertain. Accordingly, at this time, we cannot reasonably estimate the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of these product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from edasalonexent or any of our other current or potential product candidates. This is due to our need to raise additional capital to fund further clinical trials of our product candidates and the numerous risks and uncertainties associated with developing medicines, including the uncertainties of:

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

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 and timing associated with the development of that product candidate.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect to incur significant research and development costs for the foreseeable future. We expect that our research and

development expenses will increase significantly in the near term in connection with the substantial activities required to conduct our PolarisDMD trial and prepare for registration and commercialization of edasalonexent for the treatment of DMD. We do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. 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 and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters, and fees for accounting and consulting services.

We anticipate that in the near term our general and administrative expenses will remain relatively consistent with their current levels. As we approach the anticipated read out of top-line results from our PolarisDMD trial in the second quarter of 2020, we may increase our general and administrative expenditures to hire personnel to support potential commercialization of edasalonexent, dependent on our available capital resources and our prospects for obtaining additional financing.

Table of Contents

Restructuring

In April 2018, we announced a strategic shift to focus resources on our lead program edasalonexent. Consequently, we reduced our workforce by 40% during the quarter ended June 30, 2018. Charges for employee severance, employee benefits, consolidation of facilities and contract terminations of $0.9 million were recorded in the nine months ended September 30, 2018, of which $0.7 million was paid in the period. Of these costs, $0.4 million was recorded in the general and administrative section and $0.5 million was recorded in the research and development section of the accompanying condensed consolidated statement of operations. We also recorded a net gain in other income, net of $0.3 million in connection with the sale or exchange of assets disposed of during the consolidation and relocation of facilities.

Other Income (Expense)

Other income (expense), net consists of gains and losses on sale and disposal of property and equipment, interest income earned on our cash, cash equivalents, and short-term investments, interest expense incurred on debt instruments, amortized deferred financing costs and amortized debt discount and net amortization expense on short-term investments.

Critical Accounting Policies and Significant Judgments and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with United States generally accepted accounting principles. We believe that several accounting policies are important to understanding our historical and future performance. We refer to these policies as critical because these specific areas generally require us to make judgments and estimates about matters that are uncertain at the time we make the estimate, and different estimates which also would have been reasonable could have been used. On an ongoing basis, we evaluate our estimates and judgments. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

During the nine months ended September 30, 2018, there were no material changes to our critical accounting policies as reported in our 2017 Annual Report on Form 10-K.

Results of Operations

Comparison of the Three Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations for the three months ended September 30, 2018 and 2017, together with the dollar change in those items (in thousands):

	Three Months Ended September 30, 2018	2017	Period-to-Period Change
Revenue	$	$ 250	(250)
Operating expenses:			
Research and development	3,897	4,776	(879)
General and administrative	2,111	2,426	(315)
Total operating expenses	6,008	7,202	(1,194)
Loss from operations	(6,008)	(6,952)	944
Other income (expense), net	329	(65)	394
Net loss	$ (5,679)	$ (7,017)	$ 1,338

Table of Contents

Revenue

During the three months ended September 30, 2017, we recognized revenue of $250 thousand related to the Option Agreement. We did not recognize any revenue in the three months ended September 30, 2018.

Research and Development Expenses

Research and development expenses decreased by $0.9 million to $3.9 million for the three months ended September 30, 2018 from $4.8 million for the three months ended September 30, 2017, a decrease of 18%. The decrease in research and development expenses was attributable to a $0.6 million decrease in costs not directly allocated to programs, and a net decrease of $0.3 million in direct program costs. The $0.6 million decrease in costs not directly allocated to programs was attributable to a $0.4 million decrease in employee compensation due to a reduction in our workforce, a $0.1 million decrease in the research and development portion of facilities costs and a $0.1 million decrease in professional services expenses. The decrease in direct program expenses included a $0.9 million decrease in costs to support our CAT-5571 program and a $0.4 million decrease in costs to support other research and platform programs. These decreases were partially offset by a $1.0 million increase in costs to support our edasalonexent program due to activities associated with the initiation of the PolarisDMD trial.

General and Administrative Expenses

General and administrative expenses decreased by $0.3 million to $2.1 million for three months ended September 30, 2018 from $2.4 million for the three months ended September 30, 2017, a decrease of 13%. The decrease in general and administrative expenses was attributable to a $0.3 million decrease in professional services expense due to our strategic shift and focus on streamlining resources.

Other Income (Expense), Net

Other income (expense), net increased by $0.4 million in the three months ended September 30, 2018 compared to the three months ended September 30, 2017, primarily due to an increase in other income, net of $0.2 million due to the net gain realized on assets sold in consolidation and relocation of our facilities, a decrease in interest expense of $0.1 million due to principal payments made on our credit facility, and an increase of $0.1 million in interest and investment income due to an increase in our interest-bearing assets.

Comparison of the Nine Months Ended September 30, 2018 and 2017

The following table summarizes our results of operations for the nine months ended September 30, 2018 and 2017, together with the dollar change in those items (in thousands):

	Nine Months Ended September 30, 2018	2017	Period-to-Period Change
Revenue	$	$ 250	(250)
Operating expenses:			
Research and development	13,383	14,693	(1,310)
General and administrative	6,900	7,189	(289)
Total operating expenses	20,283	21,882	(1,599)
Loss from operations	(20,283)	(21,632)	1,349
Other income (expense), net	473	(235)	708
Net loss	$ (19,810)	$ (21,867)	$ 2,057

Revenue

During the nine months ended September 30, 2017, we recognized revenue of $250 thousand related to the Option Agreement. We did not recognize any revenue in the nine months ended September 30, 2018.

Research and Development Expenses

Research and development expenses decreased by $1.3 million to $13.4 million for the nine months ended September 30, 2018 from $14.7 million for the nine months ended September 30, 2017, a decrease of 9%. The decrease in research and development

Table of Contents

expenses was attributable to a net decrease of $0.7 million in direct program costs and a $0.6 million decrease in costs not directly allocated to programs. This decrease in direct program expenses included a $1.3 million decrease in costs to support our CAT-5571 program, and a $0.4 million decrease in costs to support other research and platform programs. These decreases were partially offset by a $1.0 million increase in costs to support edasalonexent associated with the initiation of the POLARIS DMD trial. The $0.6 million decrease in costs not directly allocated to programs was attributable to a $0.3 million decrease in employee compensation due to a reduction in our workforce, a $0.2 million decrease in the research and development portion of facilities expense and a $0.2 million decrease in the research and development portion of general office expenses. These decreases were partially offset by a $0.1 million increase in professional services expense due to increased use of research and development external contractors.

General and Administrative Expenses

General and administrative expenses decreased by $0.3 million to $6.9 million for nine months ended September 30, 2018 from $7.2 million for the nine months ended September 30, 2017, a decrease of 4%. The decrease in general and administrative expenses was attributable to a $0.7 million decrease in professional services expense due to our strategic shift and focus on streamlining resources. This decrease was partially offset by a $0.4 million increase in general and administrative employee costs, due to merit increases and bonuses.

Other Income (Expense), Net

Other income (expense), net increased by $0.7 million in the nine months ended September 30, 2018 compared to the nine months ended September 30, 2017, primarily due to an increase in other income, net of $0.3 million due to the net gain realized on assets sold in consolidation and relocation of our facilities, a decrease in interest expense of $0.3 million due to principal payments made on our credit facility, and an increase of $0.1 million in interest and investment income due to an increase in our interest-bearing assets.

Liquidity and Capital Resources

From our inception through September 30, 2018, we raised an aggregate of $245.0 million, of which $92.9 million was from private placements of preferred stock, $69.0 million represented gross proceeds from our IPO, $42.0 million represented gross proceeds from the June 2018 Financing, $11.5 million represented gross proceeds from a registered direct common stock offering, $10.0 million was from a secured debt financing, $18.8 million represented gross proceeds from our ATM offerings, and $0.8 million was from common stock option and warrant exercises. As of September 30, 2018, we had $43.2 million in cash, cash equivalents and short-term investments.

June 2018 Financing

On June 20, 2018, we entered into an underwriting agreement with Oppenheimer & Co. Inc. relating to an underwritten public offering, of 42,000,000 shares of our common stock, and accompanying warrants to purchase up to 42,000,000 shares of common stock, at a combined price to the public of $1.00 per share, for gross proceeds of $42.0 million, resulting in net proceeds of $38.9 million. The warrants were immediately exercisable at an exercise price of $1.20 per share and will expire five years from the date of issuance.

At-the-Market Offering

During the nine months ended September 30, 2018, we sold an aggregate of 5,390,255 shares of common stock pursuant to our ATM offering, at an average price of $1.59 per share, for gross proceeds of $8.6 million, resulting in net proceeds of $8.3 million after deducting sales commissions and offering expenses. The ATM offering was fully utilized as of February 2018.

Credit Facility

On August 27, 2014, we entered into a credit facility with MidCap Financial Trust, Flexpoint MCLS SPV LLC and Square 1 Bank, which was subsequently amended in March and December 2015 (as amended, the Credit Facility). The Credit Facility provided for maximum borrowings of $25.0 million. We received total proceeds of $10.0 million under term loans and the remaining amounts available for borrowing under this arrangement expired unused as of July 31, 2015. In connection with the drawdowns under the Credit Facility, we issued warrants to purchase shares of convertible preferred stock to the lenders, which upon the closing of our IPO were automatically converted into warrants to purchase an aggregate of 24,566 shares of our common stock at an exercise price of $12.21 per share. The warrants were exercisable immediately and expire after seven years.

Table of Contents

As of September 30, 2018, all outstanding principal and interest due under the Credit Facility had been paid. A final payment equal to 3.48% of the amounts drawn under the Credit Facility was paid in October 2018. The Credit Facility has expired.

Cash Flows

Comparison of the Nine Months Ended September 30, 2018 and 2017

The following table provides information regarding our cash flows for the nine months ended September 30, 2018 and 2017 (in thousands):

	Nine Months Ended September 30,	
	2018	**2017**
Net cash used in operating activities	$ (18,197)	$ (21,199)
Net cash (used in) provided by investing activities	(22,999)	14,883
Net cash provided by financing activities	44,703	4,433
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 3,507	$ (1,883)

Net Cash Used in Operating Activities

Net cash used in operating activities was $18.2 million for the nine months ended September 30, 2018 and consisted primarily of a net loss of $19.8 million adjusted for non-cash items, including stock-based compensation expense of $1.3 million, depreciation and amortization expense of $0.1 million, a gain on disposal of property and equipment of $0.3 million, and a net decrease in operating assets of $0.5 million, which resulted primarily from increases in accounts payable and accrued expenses.

Net cash used in operating activities was $21.2 million for the nine months ended September 30, 2017 and consisted primarily of a net loss of $21.9 million adjusted for non-cash items, including stock-based compensation expense of $1.5 million, depreciation and amortization expense of $0.2 million, non-cash interest expense of $0.1 million, and a net increase in operating assets of $1.1 million, which resulted primarily from decreases in accrued expenses and accounts payable, partially offset by an increase in prepaid expenses and other current assets.

Net Cash (Used In) Provided by Investing Activities

Net cash used in investing activities was $23.0 million for the nine months ended September 30, 2018 and consisted of purchases of short-term investments of $39.4 million partially offset by proceeds from maturities of short-term investments of $16.0 million and sales of property and equipment of $0.4 million. Net cash provided by investing activities was $14.9 million during the nine months ended September 30, 2017, which was primarily attributable to maturities of short-term investments.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $44.7 million during the nine months ended September 30, 2018, which was primarily attributable to net proceeds of $38.9 million from the June 2018 Financing and net proceeds of $8.3 million from our ATM offering, partially offset by $2.5 million in repayment of principal on the Credit Facility. Net cash provided by financing activities was $4.4 million during the nine months ended September 30, 2017, which was primarily attributable to net proceeds of $6.9 million from our ATM offering, partially offset by $2.5 million in repayment of principal on the Credit Facility.

Funding Requirements

Our primary uses of capital are for compensation and related expenses, manufacturing costs for pre-clinical and clinical materials, third party clinical trial research and development services, clinical costs, legal and other regulatory expenses, and general overhead.

As of September 30, 2018, we had an accumulated deficit of $191.2 million. We have been primarily involved with research and development activities and have incurred operating losses and negative cash flows from operations since our inception.

Table of Contents

As of September 30, 2018, we had available cash, cash equivalents and short-term investments of $43.2 million. We expect that our existing cash, cash equivalents and short-term investments are sufficient to support our operating expenses into the second quarter of 2020, and therefore the substantial doubt about our ability to continue as a going concern disclosed in the 2017 Annual Report on Form 10-K has been alleviated.

Our estimate as to how long we expect our cash, cash equivalents and short-term investments to be able to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements will depend on many factors, including:

- the pace of clinical site initiation and patient enrollment in our PolarisDMD Phase 3 trial and any unanticipated costs or expenses related to this trial, including costs and expenses for any additional research or preclinical or clinical development efforts related to this trial;
- the progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, our product candidates and potential product candidates, including current and future clinical trials;
- our ability to enter into and the terms and timing of any additional collaborations, licensing or other arrangements that we may establish;
- the number and characteristics of future product candidates that we pursue and their development requirements;
- the outcome, timing and costs of seeking regulatory approvals;
- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;
- subject to receipt of marketing approval, revenue, if any, received from commercial sales of our product candidates;
- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;
- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and
- the costs of operating as a public company.

Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain 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 medicines 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. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders rights. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

If we raise funds through 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.

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.

Table of Contents

Contractual Obligations

As of September 30, 2018, other than the payment in full of amounts due under our Credit Facility, there had been no material changes to our contractual obligations and commitments disclosed under Management s Discussion and Analysis of Financial Condition and Results of Operations in the 2017 Annual Report on Form 10-K.

Table of Contents

Item 3. Qualitative and Quantitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of September 30, 2018, we had cash, cash equivalents and short-term investments of $43.2 million and, as of December 31, 2017, we had cash and cash equivalents of $16.4 million. As of September 30, 2018, our cash equivalents consisted of money market funds, U.S. reverse repurchase agreements, and corporate debt securities. As of September 30, 2018, short-term investments consisted of corporate debt securities and U.S reverse repurchase agreements. As of December 31, 2017, our cash equivalents consisted of money market funds. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio.

We have no significant operations outside the United States and we do not expect to be impacted significantly by foreign currency fluctuations.

Item 4. Controls and Procedures

Management s Evaluation of our 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, 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.

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 Quarterly Report on Form 10-Q. 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, 2018, 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, 2018, there were 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 Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Table of Contents

PART II OTHER INFORMATION

Item 1A. Risk Factors

We operate in a dynamic and rapidly changing business environment that involves risks and substantial uncertainty. The following discussion addresses risks and uncertainties that could cause, or contribute to causing, actual results to differ from expectations in material ways. In evaluating our business, investors should pay particular attention to the risks and uncertainties described below and in other sections of this Quarterly Report on Form 10-Q and in our subsequent filings with the Securities and Exchange Commission, or SEC. These risks and uncertainties, or other events that we do not currently anticipate or that we currently deem immaterial also may affect our results of operations, cash flows and financial condition. The trading price of our common stock could also decline due to any of these risks, and you could lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

We will need substantial additional funding. If we are unable to raise capital when needed, we could be forced to delay, reduce or eliminate our product development programs or commercialization efforts.

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We recently initiated our Phase 3 clinical trial of edasalonexent and expect that our expenses will increase substantially as we conduct that trial. In addition, we may in the future initiate new research, preclinical and clinical development efforts for and seek marketing approval for, other product candidates, and would expect our expenses to increase in connection with each of these activities. In addition, if we obtain marketing approval for any of our product candidates, we may incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future collaborator, and these activities would require substantial additional funding. Furthermore, we have incurred and will continue to incur significant additional costs associated with operating as a public company.

Accordingly, we will need to obtain additional funding in connection with our continuing operations and for costs related to seeking regulatory approvals and commercialization activities for edasalonexent in Duchenne muscular dystrophy, or DMD, and for any of our other product candidates that have successful clinical trials. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts. For example, in April 2018 we announced a strategic shift to focus resources on our lead program edasalonexent and reduced our workforce by 40%. In connection with this restructuring, we suspended our other research and development programs until a collaboration or funding is obtained. Any additional funding may not be available to us on acceptable terms, on a timely basis or at all. In the event that we are unable to obtain such funding on acceptable terms and in a timely manner, we may not be able to complete the regulatory approval or commercialization of edasalonexent or the clinical development, regulatory approval or commercialization of any other product candidate.

In addition, while we may seek one or more collaborators for future development of our product candidates or programs or for our platform technology, we may not be able to enter into a collaboration for any of our product candidates or programs or for our platform technology on suitable terms or at all. In any event, our existing cash, cash equivalents and short-term investments will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain substantial additional funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or

other sources. We do not have any committed external source of funds.

Adequate additional funding may not be available to us on acceptable terms, on a timely basis or at all, impacting our ability to execute on our strategic plans. Our failure to raise capital on acceptable terms as and when needed would have a material adverse effect on our business, results of operations, financial condition and ability to pursue our business strategy.

We believe that our existing cash, cash equivalents and short-term investments as of September 30, 2018 will enable us to fund our operating expenses and debt service and capital expenditure requirements based on our current operating plan into the second quarter of 2020, and therefore the substantial doubt about our ability to continue as a going concern disclosed in the 2017 Annual Report on Form 10-K has been alleviated. Our estimate as to how long we expect our cash, cash equivalents and short-term investments securities to be able to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements will depend on many factors, including:

Table of Contents

- the pace of clinical site initiation and patient enrollment in our PolarisDMD Phase 3 trial and any unanticipated costs or expenses related to this trial, including costs and expenses for any additional research or preclinical or clinical development efforts related to this trial;

- the progress, timing, costs and results of clinical trials of, and research and preclinical development efforts for, our product candidates and potential product candidates, including current and future clinical trials;

- our ability to enter into and the terms and timing of any additional collaborations, licensing or other arrangements that we may establish;

- the number and characteristics of future product candidates that we pursue and their development requirements;

- the outcome, timing and costs of seeking regulatory approvals;

- the costs of commercialization activities for any of our product candidates that receive marketing approval to the extent such costs are not the responsibility of any future collaborators, including the costs and timing of establishing product sales, marketing, distribution and manufacturing capabilities;

- subject to receipt of marketing approval, revenue, if any, received from commercial sales of our product candidates;

- our headcount growth and associated costs as we expand our research and development and establish a commercial infrastructure;

- the costs of preparing, filing and prosecuting patent applications, maintaining and protecting our intellectual property rights and defending against intellectual property related claims; and

- the costs of operating as a public company.

Raising additional capital may cause dilution to our stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.

Significant additional capital will be needed in the future to continue our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, our existing stockholders ownership interest may be substantially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. For example, our June 2018 registered offering of common stock and common stock warrants was highly dilutive to existing stockholders ownership interests. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. In addition, securing additional financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management s ability to oversee the development of our product candidates.

If we raise additional funds through collaborations or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds 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.

Any future indebtedness could adversely affect our ability to operate our business.

Any future indebtedness that we may incur, combined with our other financial obligations and contractual commitments could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product development and other general corporate purposes;
- increasing our vulnerability to adverse changes in general economic, industry and market conditions;
- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;
- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and
- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

Failure to make payments or comply with other covenants under any debt instruments could result in an event of default and acceleration of amounts due.

Table of Contents

We have incurred significant losses since inception and expect to incur significant losses for at least the next several years. We may never achieve or maintain profitability.

We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur significant operating losses for at least the next several years. Our net losses were $27.4 million and $36.1 million for the years ended December 31, 2017 and 2016, respectively. For the three and nine months ended September 30, 2018, our net losses were $5.7 million and $19.8 million, respectively. As of September 30, 2018, we had an accumulated deficit of $191.2 million. We have not generated any revenues from product sales, have not completed the development of any product candidate and may never have a product candidate approved for commercialization. We have financed our operations to date primarily through private placements of our preferred stock, registered offerings of our common stock, including our initial public offering, or IPO, our June 2018 registered offering of common stock and common stock warrants, our at-the-market program, and a secured debt financing, and have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical development programs. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders equity and working capital.

We anticipate that our expenses will increase substantially if and to the extent we:

- conduct our Phase 3 clinical trial of edasalonexent in DMD;
- initiate and continue research and preclinical and clinical development efforts for our other product candidates;
- seek to identify and develop additional product candidates;
- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;
- establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any;
- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;
- maintain, expand and protect our intellectual property portfolio;
- hire and retain additional personnel, such as clinical, quality control and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and
- add equipment and physical infrastructure to support our research and development programs.

Our ability to become and remain profitable depends on our ability to generate revenue. We do not expect to generate significant revenue unless and until we are, or any future collaborator is, able to obtain marketing approval for, and successfully commercialize, one or more of our product candidates. This will require our, or any of our future collaborators , success in a range of challenging activities, including obtaining funding to

conduct clinical trials of our product candidates, completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of increased expenses, and if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborator does, we may never generate revenues that are large enough for us to achieve profitability. Even 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, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our company could cause our investors to lose all or part of their investments.

We have a limited operating history and no history of commercializing pharmaceutical products, which may make it difficult to evaluate the prospects for our future viability.

We began operations in 2008. Our operations to date have been limited to financing and staffing our company and developing our technology and conducting preclinical research and clinical trials for our product candidates. We have not yet demonstrated an ability to successfully conduct pivotal clinical trials, obtain marketing approvals, manufacture a commercial scale product, or arrange for a third party to do so on our behalf, or conduct sales and marketing activities necessary for successful product commercialization. Accordingly, our investors should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early stages of development, especially clinical-stage biopharmaceutical companies such as ours. Predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

Table of Contents

Risks Related to the Discovery, Development and Commercialization of Our Product Candidates

Our approach to the discovery and development of product candidates based on our SMART LinkerSM drug discovery platform is unproven, and we do not know whether we will be able to develop any products of commercial value.

We are focused on discovering and developing novel small molecule drugs by applying our Safely Metabolized And Rationally Targeted, or SMART, linker drug discovery platform. We have not yet succeeded and may never succeed in demonstrating efficacy and safety for any of our product candidates in a Phase 3 clinical trial or in obtaining marketing approval thereafter. For example, although we have discovered and evaluated numerous compounds using our SMART Linker drug discovery platform, no product created using the SMART Linker drug discovery platform has ever been approved for sale.

We are dependent on the success of our product candidate edasalonexent. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize this product candidate, either alone or with a collaborator, or if we experience significant delays in doing so, our business would be substantially harmed.

We currently have no products approved for sale and are investing substantially all of our efforts and financial resources in the development of edasalonexent for the treatment of DMD. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize edasalonexent. Because our business is almost entirely dependent upon this one product candidate, any setback in obtaining regulatory approval for edasalonexent would have a material adverse effect on our business and prospects.

The success of edasalonexent will depend on several factors, including the following:

- successful enrollment and completion of our Phase 3 clinical trial of edasalonexent, as well as any additional clinical trials of edasalonexent, including the ongoing open-label extension of our MoveDMD clinical trial;
- safety, tolerability and efficacy profiles that are satisfactory to the U.S. Food and Drug Administration, or FDA, or any comparable foreign regulatory authority for marketing approval;
- timely receipt of marketing approvals from applicable regulatory authorities;
- the performance of our future collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials suppliers and manufacturers;
- establishment of arrangements with third-party manufacturers to obtain finished drug products that are appropriately packaged for sale;

- obtaining and maintaining patent, trade secret protection and regulatory exclusivity, both in the United States and internationally;

- protection of our rights in our intellectual property portfolio;

- successful launch of commercial sales following any marketing approval;

- a continued acceptable safety profile following any marketing approval;

- commercial acceptance by patients, the medical community and third-party payors following any marketing approval; and

- our ability to compete with other therapies, including therapies targeting dystrophin, utrophin, myostatin and inflammatory mediators.

Many of these factors are beyond our control, including the outcome of clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize edasalonexent, on our own or with any future collaborator, or experience delays as a result of any of these or other factors, our business would be substantially harmed.

Our SMART Linker drug discovery platform may fail to help us discover and develop additional potential product candidates.

A significant portion of the research that we have conducted and may in the future conduct, involves the development of new compounds using our SMART Linker drug discovery platform. The drug discovery that we are conducting using our SMART Linker drug discovery platform may not be successful in creating compounds that have commercial value or therapeutic utility. Our SMART Linker drug discovery platform may initially show promise in identifying potential product candidates, yet fail to yield viable product candidates for clinical development or commercialization for a number of reasons, including:

- compounds created through our SMART Linker drug discovery platform may not demonstrate improved efficacy, safety or tolerability;

Table of Contents

- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;

- competitors may develop alternative therapies that render our potential product candidates non-competitive or less attractive; or

- a potential product candidate may not be capable of being produced at an acceptable cost.

Our research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

We have never obtained marketing approval for a product candidate and we may be unable to obtain, or may be delayed in obtaining, marketing approval for any of our product candidates.

We have never obtained marketing approval for a product candidate. It is possible that the FDA may refuse to accept for substantive review any new drug applications, or NDAs, that we submit for our product candidates or may conclude after review of our data that our applications are insufficient to obtain marketing approval of our product candidates. If the FDA does not accept or approve any NDAs we submit, it may require that we conduct additional clinical, nonclinical or manufacturing validation studies and submit that data before it will reconsider our applications. Depending on the extent of these or any other FDA-required studies, approval of any NDA or application that we submit may be delayed by several years, or may require us to expend more resources than we have available. It is also possible that additional studies, if performed and completed, may not be considered sufficient by the FDA to approve our NDAs.

Any delay in obtaining, or an inability to obtain, marketing approvals would prevent us from commercializing our product candidates, generating revenues and achieving and sustaining profitability. If any of these outcomes occur, we may be forced to abandon our development efforts for our product candidates, which could significantly harm our business.

Results of preclinical studies and early clinical trials may not be predictive of results of future clinical trials.

The outcome of preclinical studies and early clinical trials may not be predictive of the success of later clinical trials, and interim results of clinical trials do not necessarily predict success in future clinical trials. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials after achieving positive results in earlier development, and we cannot be certain that we will not face similar setbacks. The design of a clinical trial can determine whether its results will support approval of a product and flaws in the design of a clinical trial may not become apparent until the clinical trial is well advanced. We have limited experience in designing clinical trials and may be unable to design and execute a clinical trial to support marketing approval. In addition, preclinical and clinical data are often susceptible to varying interpretations and analyses. Many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain marketing approval for the product candidates. Even if we, or any future collaborators, believe that the results of clinical trials for our product candidates warrant marketing approval, the FDA or comparable foreign regulatory authorities may disagree and may not grant marketing approval of our product candidates.

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. For example, while we observed positive NF-kB biomarker data in the Phase 1 portion of our MoveDMD Phase 1/2 clinical trial of edasalonexent for the treatment of DMD that demonstrated NF-kB target engagement via statistically significant reduction in NF-kB controlled gene expression for the 67 mg/kg/day and 100 mg/kg/day dosing levels, the primary efficacy endpoint in the Phase 2 portion of the trial for the same dosing levels was not met. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted.

The regulatory approval processes for product candidates that target rare diseases, including DMD and cystic fibrosis, are uncertain.

Due to the lack of precedent, broad discretion of regulatory authorities, and a multitude of unique factors that impact the regulatory approval process, the likelihood of the approval of any of our product candidates that target rare diseases, such as DMD, or cystic fibrosis, is uncertain, and we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned investigational new drug applications and NDAs for our product candidates, in a timely

Table of Contents

manner, or at all. For example, DMD is a rare disease for which there are only two FDA approved therapeutics. Further, the FDA may determine, after evaluation of our data and analyses, that such data and analyses do not support an NDA submission, filing or approval. Due to this lack of predictability, we may not have the resources necessary to meet regulatory requirements and successfully complete a potentially protracted, expensive and wide-ranging approval process for commercialization of product candidates for rare diseases.

We may expend our limited resources to pursue a particular product candidate or indication and fail to capitalize on product candidates or indications that may be more profitable or for which there is a greater likelihood of success.

Because we have limited financial and managerial resources, we intend to focus on developing product candidates for specific indications that we identify as most likely to succeed, in terms of both their potential for marketing approval and commercialization. As a result, we may forego or delay pursuit of opportunities with other product candidates or for other indications that may prove to have greater commercial potential.

Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs and product candidates for specific indications may not yield any commercially viable product candidates. If we do not accurately evaluate the commercial potential or target market for a particular product candidate, we may relinquish valuable rights to that product candidate through collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to the product candidate.

Clinical drug development involves a lengthy and expensive process with an uncertain outcome.

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. Further, the clinical development of our product candidates is susceptible to the risk of failure at any stage of drug development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in our clinical trials we may fail to detect toxicity of or intolerability caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case.

In addition to the risk of failure inherent in drug development, certain of the compounds that we are developing and may develop in the future using our SMART Linker drug discovery platform may be particularly susceptible to failure to the extent they are based on compounds that others have previously studied or tested, but did not progress in development due to safety, tolerability or efficacy concerns or otherwise. Our failure to successfully complete clinical trials of our product candidates and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of our product candidates would significantly harm our business.

If clinical trials of our product candidates fail to satisfactorily demonstrate safety and efficacy to the FDA and other comparable foreign regulators, we, or any future collaborators, may incur additional costs or experience delays in completing, or ultimately be unable to complete, the development and commercialization of these product candidates.

We, and any future collaborators, are not permitted to commercialize, market, promote or sell any product candidate in the United States without obtaining marketing approval from the FDA. Comparable foreign regulatory authorities, such as the European Medicines Agency, or the EMA, impose similar restrictions. We, and any future collaborators, may never receive such approvals. We, and any future collaborators, must complete extensive preclinical development and clinical trials to demonstrate the safety and efficacy of our product candidates in humans before we, or they, will be able to obtain these approvals.

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. Any inability to complete preclinical and clinical development successfully could result in additional costs to us, or any future collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if (1) we, or any future collaborators, are required to modify our trial designs, such as required modifications with respect to patient populations, endpoints, comparators or trial duration, (2) we, or any future collaborators, are required to conduct additional clinical trials or other testing of our product candidates beyond the trials and testing that we, or they contemplate, (3) we, or any future collaborators, are unable to successfully complete clinical trials of our product

Table of Contents

candidates or other testing, (4) the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or (5) there are unacceptable safety concerns associated with our product candidates, we, or any future collaborators, may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval 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 significant safety warnings, including boxed warnings;
- be subject to additional post-marketing testing or other requirements; or
- be required to remove the product from the market after obtaining marketing approval.

Adverse events or undesirable side effects caused by, or other unexpected properties of, any of our product candidates may be identified during development that could delay or prevent their marketing approval or limit their use.

Adverse events or undesirable side effects caused by, or other unexpected properties of, our product candidates could cause us, any future collaborators, an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. For example, in 2014, in our clinical trials of our CAT-2003 compound we observed gastrointestinal tolerability issues, including nausea, diarrhea and vomiting, and in some cases these adverse events led to dose reductions or discontinuations. If any of our product candidates is associated with adverse events or undesirable side effects or has properties that are unexpected, we, or any future collaborators, may need to abandon development or limit development of that product candidate to certain uses or subpopulations in which the undesirable side effects or other characteristics are less prevalent, less severe or more acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound.

If we, or any future collaborators, experience any of a number of possible unforeseen events in connection with clinical trials of our product candidates, potential marketing approval or commercialization of our product candidates could be delayed or prevented.

We, or any future collaborators, may experience numerous unforeseen events during, or as a result of, clinical trials that could delay or prevent marketing approval or commercialization of our product candidates, including:

- clinical trials of our product candidates may produce unfavorable or inconclusive results, such as occurred in our MoveDMD Phase 1/2 clinical trial of edasalonexent for the treatment of DMD, where the primary efficacy endpoint was not met;

- we, or any future collaborators, may decide, or regulators may require us or them, to conduct additional clinical trials or abandon product development programs;

- the number of patients required for clinical trials of our product candidates may be larger than we, or any future collaborators, anticipate, patient enrollment in these clinical trials may be slower than we, or any future collaborators, anticipate or participants may drop out of these clinical trials at a higher rate than we, or any future collaborators, anticipate;

- the cost of planned clinical trials of our product candidates may be greater than we anticipate;

- our third-party contractors or those of any future collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of any future collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or any future collaborators in a timely manner or at all;

- regulators or institutional review boards may not authorize us, any future collaborators or our or their investigators to commence a clinical trial or conduct a clinical trial at a prospective trial site;

- we, or any future collaborators, may have delays in reaching or fail to reach agreement on acceptable clinical trial contracts or clinical trial protocols with prospective trial sites;

- patients that enroll in a clinical trial may misrepresent their eligibility to do so or may otherwise not comply with the clinical trial protocol, resulting in the need to drop the patients from the clinical trial, increase the needed enrollment size for the clinical trial or extend the clinical trial s duration;

- we, or any future collaborators, may have to delay, suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate;

- regulators or institutional review boards may require that we, or any future collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects

Table of Contents

or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar drug or drug candidate;

- the FDA or comparable foreign regulatory authorities may disagree with our, or any future collaborators , clinical trial designs or our or their interpretation of data from preclinical studies and clinical trials;

- the FDA or comparable foreign regulatory authorities may fail to approve or subsequently find fault with the manufacturing processes or facilities of third-party manufacturers with which we, or any future collaborators, enter into agreements for clinical and commercial supplies;

- the supply or quality of raw materials or manufactured product candidates or other materials necessary to conduct clinical trials of our product candidates may be insufficient, inadequate or not available at an acceptable cost, or we may experience interruptions in supply; and

- the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient to obtain marketing approval.

Product development costs for us, or any future collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we, or any future collaborators, may have the exclusive right to commercialize our product candidates or allow our competitors, or the competitors of any future collaborators, to bring products to market before we, or any future collaborators, do and impair our ability, or the ability of any future collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

If we, or any future collaborators, experience delays or difficulties in the enrollment of patients in clinical trials, our or their receipt of necessary regulatory approvals could be delayed or prevented.

We, or any future collaborators, may not be able to initiate or continue clinical trials for any of our product candidates if we, or they, are unable to locate and enroll a sufficient number of eligible patients to participate in clinical trials as required by the FDA or comparable foreign regulatory authorities, such as the EMA. Patient enrollment is a significant factor in the timing of clinical trials, and is affected by many factors, including:

- the size and nature of the patient population;

- the severity of the disease under investigation;

- the proximity of patients to clinical sites;

- the eligibility criteria for the trial;

- the design of the clinical trial;

- efforts to facilitate timely enrollment;
- competing clinical trials; and
- clinicians and patients perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

In particular, the successful completion of our clinical development program for edasalonexent for the treatment of DMD is dependent upon our ability to enroll a sufficient number of patients with DMD. DMD is a rare disease with a small patient population. Further, there are only a limited number of specialist physicians that regularly treat patients with DMD and major clinical centers that support DMD treatment are concentrated in a few geographic regions. In addition, other companies are conducting clinical trials and have announced plans for future clinical trials that are seeking, or are likely to seek, to enroll patients with DMD and patients are generally only able to enroll in a single trial at a time. The small population of patients, competition for these patients and the limited trial sites may make it difficult for us to enroll enough patients to complete our clinical trials for edasalonexent in a timely and cost-effective manner or at all. Furthermore, we intend to enroll a significantly greater number of patients in our Phase 3 clinical trial of edasalonexent in DMD than were enrolled in our previous trials of edasalonexent. As a result, we may be subject to greater risks with respect to patient enrollment.

The clinical trials that we conduct may also have inclusion criteria that further limit the population of patients that we are able to enroll. For example, further clinical trials for edasalonexent may require that the enrolled boys be between certain ages and not on certain other medications. These inclusion criteria, or other inclusion criteria that are not yet defined, could further limit the available patient pool and present challenges to clinical trial enrollment.

Table of Contents

Our inability, or the inability of any future collaborators, to enroll a sufficient number of patients for our, or their, clinical trials could result in significant delays or may require us or them to abandon one or more clinical trials altogether. Enrollment delays in our, or their, clinical trials may result in increased development costs for our product candidates, delay or halt the development of and approval processes for our product candidates and jeopardize our, or any future collaborators , ability to commence sales of and generate revenues from our product candidates, which could cause the value of our company to decline.

If any of our product candidates receives marketing approval and we, or others, later discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, our ability, or that of any future collaborators, to market the drug could be compromised.

Clinical trials of our product candidates are conducted in carefully defined subsets of patients who have agreed to enter into clinical trials. Consequently, it is possible that our clinical trials, or those of any future collaborator, may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any, or alternatively fail to identify undesirable side effects. If, following approval of a product candidate, we, or others, discover that the drug is less effective than previously believed or causes undesirable side effects that were not previously identified, any of the following adverse events could occur:

- regulatory authorities may withdraw their approval of the drug or seize the drug;
- we, or any future collaborators, may be required to recall the drug, change the way the drug is administered or conduct additional clinical trials;
- additional restrictions may be imposed on the marketing of, or the manufacturing processes for, the particular drug;
- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a black box warning or a contraindication;
- we, or any future collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or any future collaborators, could be sued and held liable for harm caused to patients;
- the drug may become less competitive; and
- our reputation may suffer.

Any of these events could have a material and adverse effect on our operations and business and could adversely impact our stock price.

Even if one of our product candidates receives marketing approval, it may fail to achieve the degree of market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success and the market opportunity for the product candidate may be smaller than we estimate.

We have never commercialized a product. Even if one of our product candidates is approved by the appropriate regulatory authorities for marketing and sale, it may nonetheless fail to gain sufficient market acceptance by physicians, patients, third-party payors and others in the medical community. For example, physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective or convenient treatments enter the market. Further, patients often acclimate to the therapy that they are currently taking and do not want to switch unless their physicians recommend switching products or they are required to switch therapies due to lack of reimbursement for existing therapies.

Efforts to educate the medical community and third-party payors on the benefits of our product candidates may require significant resources and may not be successful. If any of our product candidates is approved but does not achieve an adequate level of market acceptance, we may not generate significant revenues and we may not become profitable. The degree of market acceptance of our product candidates, if approved for commercial sale, will depend on a number of factors, including:

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;
- whether the product is designated under physician treatment guidelines as a first-line therapy or as a second- or third-line therapy;
- limitations or warnings, including distribution or use restrictions, contained in the product s approved labeling;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product s convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the strength of sales, marketing and distribution support;

Table of Contents

- the approval of other new products for the same indications;
- changes in the standard of care for the targeted indications for the product;
- the timing of market introduction of our approved products as well as competitive products;
- availability and amount of reimbursement from government payors, managed care plans and other third-party payors;
- adverse publicity about the product or favorable publicity about competitive products; and
- potential product liability claims.

The potential market opportunities for our product candidates are difficult to estimate precisely. Our estimates of the potential market opportunities are predicated on many assumptions, including industry knowledge and publications, third-party research reports and other surveys. While we believe that our internal assumptions are reasonable, these assumptions involve the exercise of significant judgment on the part of our management, are inherently uncertain and the reasonableness of these assumptions has not been assessed by an independent source. If any of the assumptions proves to be inaccurate, the actual markets for our product candidates could be smaller than our estimates of the potential market opportunities.

If we are unable to establish sales, marketing and distribution capabilities or enter into sales, marketing and distribution arrangements with third parties, we may not be successful in commercializing any product candidates that we develop if and when those product candidates are approved.

We do not have a sales, marketing or distribution infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product, we must either develop a sales and marketing organization or outsource these functions to third parties. We plan to use a combination of focused in-house sales and marketing capabilities and third-party collaboration, licensing and distribution arrangements to sell any of our products that receive marketing approval.

We generally plan to seek to retain full commercialization rights for products that we can commercialize with a specialized sales force and to retain co-promotion or similar rights when feasible in indications requiring a larger commercial infrastructure. The development of sales, marketing and distribution capabilities will require substantial resources, will be time-consuming and could delay any product launch. If the commercial launch of a product for which we recruit a sales force and establish marketing and distribution capabilities is delayed or does not occur for any reason, we could have prematurely or unnecessarily incurred these commercialization costs. This may be costly, and our investment could be lost if we cannot retain or reposition our sales and marketing personnel. In addition, we may not be able to hire or retain a sales force that is sufficient in size or has adequate expertise in the medical markets that we plan to target. If we are unable to establish or retain a sales force and marketing and distribution capabilities, our operating results may be adversely affected. If a potential partner has development or commercialization expertise that we believe is particularly relevant to one of our products, then we may seek to collaborate with that potential partner even if we believe we could otherwise develop and commercialize the product independently.

We may collaborate with third parties for commercialization of any products that require a large sales, marketing and product distribution infrastructure. We intend to potentially commercialize our product candidates through collaboration, licensing and distribution arrangements with third parties. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product

revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

If we do not establish sales, marketing and distribution capabilities, either on our own or in collaboration with third parties, we will not be successful in commercializing any of our product candidates that receive marketing approval.

We face substantial competition from other pharmaceutical and biotechnology companies, and our operating results may suffer if we fail to compete effectively.

The development and commercialization of new drug products is highly competitive. We expect that we, and any future collaborators, will face significant competition from major pharmaceutical companies, specialty pharmaceutical companies and biotechnology companies worldwide with respect to any of our product candidates that we, or they, may seek to develop or commercialize in the future. Specifically, there are a number of large pharmaceutical and biotechnology companies that currently market and sell products or are pursuing the development of product candidates for the treatment of the key indication of our most advanced program, DMD.

Table of Contents

There are currently two therapies approved for the treatment of DMD in the United States, Sarepta Therapeutics drug EXONDYS 51®, also known as eteplirsen, and PTC Therapeutics EMFLAZA®, also known as deflazacort, a corticosteroid. Additionally, corticosteroid therapy, including prednisone, is often prescribed off-label to treat the inflammation underlying DMD and to delay loss of ambulation. PTC Therapeutics Translarna has conditional marketing authorization in the European Union for the treatment of DMD caused by a nonsense mutation. A number of companies are developing additional therapies to treat DMD and are in the process of registration or in late stage clinical development, including Hoffman-La Roche Ltd., Italfarmaco S.p.A., Pfizer, Inc., PTC Therapeutics, Santhera Pharmaceuticals and Sarepta Therapeutics.

Our competitors may succeed in developing, acquiring or licensing technologies and drug products that are more effective, have fewer or more tolerable side effects or are less costly than any product candidates that we are currently developing or that we may develop, which could render our product candidates obsolete and noncompetitive.

Our commercial opportunity could be reduced or eliminated if our 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, or any future collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or any future collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or any future collaborators, are able to enter the market.

Many of our existing and potential future competitors have significantly greater financial resources and expertise in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining marketing approvals and marketing approved products than we do. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a smaller number of our competitors. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical trial sites and patient registration for clinical trials, as well as in acquiring technologies complementary to, or necessary for, our programs.

If the FDA or comparable foreign regulatory authorities approve generic versions of any of our products that receive marketing approval, or such authorities do not grant our products appropriate periods of data exclusivity before approving generic versions of our products, the sales of our products could be adversely affected.

Once an NDA is approved, the product covered thereby becomes a reference-listed drug in the FDA s publication, Approved Drug Products with Therapeutic Equivalence Evaluations. Manufacturers may seek approval of generic versions of reference-listed drugs through submission of abbreviated new drug applications, or ANDAs, in the United States. In support of an ANDA, a generic manufacturer need not conduct clinical studies. Rather, the applicant generally must show that its product has the same active ingredient(s), dosage form, strength, route of administration and conditions of use or labeling as the reference-listed drug and that the generic version is bioequivalent to the reference-listed drug, meaning it is absorbed in the body at the same rate and to the same extent. Generic products may be significantly less costly to bring to market than the reference-listed drug and companies that produce generic products are generally able to offer them at lower prices. Thus, following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug may be typically lost to the generic product.

The FDA may not approve an ANDA for a generic product until any applicable period of non-patent exclusivity for the reference-listed drug has expired. The Federal Food, Drug, and Cosmetic Act, or FDCA, provides a period of five years of non-patent exclusivity for a new drug containing a new chemical entity, or NCE. Specifically, in cases where such exclusivity has been granted, an ANDA may not be filed with the FDA until the expiration of five years unless the submission is accompanied by a Paragraph IV certification that a patent covering the reference-listed drug is either invalid or will not be infringed by the generic product, in which case the applicant may submit its application four years following approval of the reference-listed drug. It is unclear whether the FDA will treat the active ingredients in our product candidates as NCEs and, therefore, afford them five years of NCE data exclusivity if they are approved. If any product we develop does not receive five years of NCE exclusivity, the FDA may approve generic versions of such product three years after its date of approval. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

Competition that our products may face from generic versions of our products could materially and adversely impact our future revenue, profitability and cash flows and substantially limit our ability to obtain a return on the investments we have made in those product candidates.

Table of Contents

Even if we, or any future collaborators, are able to commercialize any product candidate that we, or they, develop, the product may become subject to unfavorable pricing regulations, third-party payor reimbursement practices or healthcare reform initiatives that could harm our business.

The commercial success 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 third-party payors, including government health administration authorities and private health coverage insurers. If coverage and reimbursement is not available, or reimbursement is available only to limited levels, we, or any future collaborators, may not be able to successfully commercialize our product candidates. Even if coverage is provided, the approved reimbursement amount may not be high enough to allow us, or any future collaborators, to establish or maintain pricing sufficient to realize a sufficient return on our or their investments. In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors and coverage and reimbursement for products can differ significantly from payor to payor.

There is significant uncertainty related to third-party payor coverage and reimbursement of newly approved drugs. Marketing approvals, pricing and reimbursement for new drug products vary widely from country to country. Some countries require approval of the sale price of a drug before it can be marketed. In many countries, the pricing review period begins after marketing or product licensing approval is granted. In some foreign markets, prescription pharmaceutical pricing remains subject to continuing governmental control even after initial approval is granted. As a result, we, or any future collaborators, might obtain marketing approval for a product in a particular country, but then be subject to price regulations that delay commercial launch of the product, possibly for lengthy time periods, which may negatively impact the revenues we are able to generate from the sale of the product in that country. Adverse pricing limitations may hinder our ability or the ability of any future collaborators to recoup our or their investment in one or more product candidates, even if our product candidates obtain marketing approval.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any future collaborators, to commercialize any of our product candidates will depend in part on the extent to which coverage and reimbursement for these products and related treatments will be available from third-party payors. Third-party payors decide which medications they will cover and establish reimbursement levels. The healthcare industry is acutely focused on cost containment, both in the United States and elsewhere. Government authorities and other third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications, which could affect our ability or that of any future collaborators to sell our product candidates profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of any future collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or any future collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third-party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer.

There may also be delays in obtaining coverage and reimbursement for newly approved drugs, and coverage may be more limited than the indications for which the drug is approved by the FDA or comparable foreign regulatory authorities. Moreover, eligibility for reimbursement does not imply that any drug will be paid for in all cases or at a rate that covers our costs, including research, development, manufacture, sale and distribution. Reimbursement rates may vary, by way of example, according to the use of the drug and the clinical setting in which it is used. Reimbursement rates may also be based on reimbursement levels already set for lower cost drugs or may be incorporated into existing payments for other services.

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any future collaborator, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be

sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any of our product candidates for which we, or any future collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

Product liability lawsuits against us could divert our resources, cause us to incur substantial liabilities and limit commercialization of any products that we may develop.

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we or any future collaborators commercially sell any product that we may or they may develop. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability

Table of Contents

claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

- decreased demand for our product candidates or products that we may develop;
- injury to our reputation and significant negative media attention;
- withdrawal of clinical trial participants;
- significant costs to defend resulting litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain general liability insurance of $5.0 million in the aggregate and clinical trial liability insurance of $10.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could adversely affect our business, financial condition, results of operations and prospects.

Risks Related to Our Dependence on Third Parties

We expect to seek to establish collaborations and, if we are not able to establish them on commercially reasonable terms, we may have to alter our development and commercialization plans.

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We expect to seek one or more collaborators for the development and commercialization of one or more of our product candidates. For example, conducting clinical trials of CAT-5571 in patients with cystic fibrosis will likely involve significant cost, and we expect that we would conduct any clinical trial of CAT-5571 in patients with cystic fibrosis in collaboration with one or more partners. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies.

We face significant competition in seeking appropriate collaborators. Whether we reach a definitive agreement for a collaboration will depend, among other things, upon our assessment of the collaborator s resources and expertise, the terms and conditions of the proposed collaboration and the proposed collaborator s evaluation of a number of factors. Those factors may include the potential differentiation of our product candidate from competing product candidates, design or results of clinical trials, the likelihood of approval by the FDA or comparable foreign regulatory authorities and the regulatory pathway for any such approval, the potential market for the product candidate, the costs and complexities of manufacturing and delivering the product to patients and the potential of competing products. The collaborator may also consider alternative product candidates or technologies for similar indications that may be available for collaboration and whether such a collaboration could be more attractive than the one with us for our product candidate.

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. In addition, any loan and security agreements or collaboration agreements that we enter into in the future may contain, restrictions on our ability to enter into potential collaborations or to otherwise develop specified compounds.

We may not be able to negotiate collaborations on a timely basis, on acceptable terms, or at all. If we are unable to do so, we may have to curtail the development of the product candidate for which we are seeking to collaborate, reduce or delay its development program or one or more of our other development programs, delay its potential commercialization or reduce the scope of any sales or marketing activities, or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to increase our expenditures to fund development or commercialization activities on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms or at all. If we do not have sufficient funds, we may not be able to further develop our product candidates or bring them to market and generate product revenue.

Table of Contents

If we enter into collaborations with third parties for the development and commercialization of our product candidates, our prospects with respect to those product candidates will depend in significant part on the success of those collaborations.

We expect to enter into collaborations for the development and commercialization of certain of our product candidates. If we enter into such collaborations, we will have limited control over the amount and timing of resources that our collaborators will dedicate to the development or commercialization of our product candidates. Our ability to generate revenues from these arrangements will depend on any future collaborators abilities to successfully perform the functions assigned to them in these arrangements. In addition, any future collaborators may have the right to abandon research or development projects and terminate applicable agreements, including funding obligations, prior to or upon the expiration of the agreed upon terms.

Collaborations involving our product candidates pose a number of 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;
- collaborators may not pursue development and commercialization of our product candidates 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 a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;
- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;
- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might 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;
- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and
- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

Collaboration agreements may not lead to development or commercialization of product candidates in the most efficient manner or at all. If any future collaborator of ours is involved in a business combination, it could decide to delay, diminish or terminate the development or commercialization of any product candidate licensed to it by us.

We rely on third parties to conduct our clinical trials. If they do not perform satisfactorily, our business could be significantly harmed.

We do not independently conduct clinical trials of any of our product candidates. We rely on third parties, such as contract research organizations, clinical data management organizations, medical institutions and clinical investigators, to conduct these clinical trials and expect to rely on these third parties to conduct clinical trials of any other product candidate that we develop. Any of these third parties may terminate their engagements with us under certain circumstances. We may not be able to enter into alternative arrangements or do so on commercially reasonable terms. In addition, there is a natural transition period when a new contract research organization begins work. As a result, delays would likely occur, which could materially impact our ability to meet our expected clinical development timelines and harm our business, financial condition and prospects.

Further, our reliance on these third parties for clinical development activities limits our control over these activities, but we remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as current Good Clinical Practices, or cGCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these cGCPs through

Table of Contents

periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third-party contractors fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. We are also required to register clinical trials and post the results of completed clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Furthermore, the third parties conducting clinical trials on our behalf are not our employees, and except for remedies available to us under our agreements with such contractors, we cannot control whether or not they devote sufficient time, skill and resources to our ongoing development programs. These contractors 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, which could impede their ability to devote appropriate time to our clinical programs. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our clinical trials in accordance with regulatory requirements or our stated protocols, we may not be able to obtain, or may be delayed in obtaining, marketing approvals for our product candidates. If that occurs, we will not be able to, or may be delayed in our efforts to, successfully commercialize our product candidates. In such an event, our financial results and the commercial prospects for any product candidates that we seek to develop could be harmed, our costs could increase and our ability to generate revenues could be impaired.

We also rely on other third parties to store and distribute drug supplies for our clinical trials. Any performance failure on the part of our distributors could delay clinical development or marketing approval of our product candidates or commercialization of any resulting products, producing additional losses and depriving us of potential product revenue.

We contract with third parties for the manufacture and distribution of our product candidates for clinical trials and expect to continue to do so in connection with our future development and commercialization efforts. This reliance on third parties increases the risk that we will not have sufficient quantities of our product candidates or such quantities at an acceptable cost, which could delay, prevent or impair our development or commercialization efforts.

We currently have no manufacturing facilities and limited personnel with manufacturing experience. We rely on contract manufacturers to produce both drug substance and drug product required for our clinical trials. We plan to continue to rely upon contract manufacturers, and, potentially collaboration partners, to manufacture commercial quantities of our products, if approved. Reliance on such third-party contractors entails risks, including:

- manufacturing delays if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the possible termination or nonrenewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the possible breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements;

- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;

- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and

- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

We currently rely, and expect to continue to rely, on a small number of third-party contract manufacturers to supply the majority of our active pharmaceutical ingredient and required finished product for our preclinical studies and clinical trials. We do not have long-term agreements with any of these third parties. If any of our existing manufacturers should become unavailable to us for any reason, we may incur some delay in identifying or qualifying replacements.

Any manufacturing problem or the loss of a contract manufacturer could be disruptive to our operations, delay our clinical trials and, if our products are approved for sale, result in lost sales. Additionally, we rely on third parties to supply the raw materials needed to manufacture our product candidates. Any reliance on suppliers may involve several risks, including a potential inability to obtain critical materials and reduced control over production costs, delivery schedules, reliability and quality. Any unanticipated disruption to future contract manufacture caused by problems at suppliers could delay shipment of our product candidates, increase our cost of goods sold and result in lost sales.

If any of our product candidates are approved by any regulatory agency, we plan to enter into agreements with third-party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a

Table of Contents

contract manufacturer on satisfactory terms or in a timely manner. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under current good manufacturing practices, or cGMPs, that are capable of manufacturing our product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization efforts.

Third-party manufacturers are required to comply with cGMPs and similar regulatory requirements outside the United States. Facilities used by our third-party manufacturers must be approved by the FDA after we submit an NDA and before potential approval of the product candidate. Similar regulations apply to manufacturers of our product candidates for use or sale in foreign countries. We do not control the manufacturing process and are completely dependent on our third-party manufacturers for compliance with the applicable regulatory requirements for the manufacture of our product candidates. If our manufacturers cannot successfully manufacture material that conforms to our specifications or the strict regulatory requirements of the FDA and any applicable foreign regulatory authority, they will not be able to secure the applicable approval for their manufacturing facilities. If these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate.

In addition, our manufacturers are subject to ongoing periodic inspections by the FDA and corresponding state and foreign agencies for compliance with cGMPs and similar regulatory requirements both prior to and following the receipt of marketing approval for any of our product candidates. Some of these inspections may be unannounced. Failure by any of our manufacturers to comply with applicable cGMPs or other regulatory requirements could result in sanctions being imposed on us, including fines, injunctions, civil penalties, delays, suspensions or withdrawals of approvals, operating restrictions, interruptions in supply and criminal prosecutions, any of which could adversely affect supplies of our product candidates and significantly harm our business, financial condition and results of operations.

Our current and anticipated future dependence upon others for the manufacture of our product candidates may adversely affect our future profit margins and our ability to commercialize any products that receive marketing approval on a timely and competitive basis.

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to commercialize our product candidates successfully 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. If we do not adequately protect our intellectual property, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which 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 patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind 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 our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in post-grant review procedures, oppositions, derivations, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may

Table of Contents

result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, 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 products. In addition, 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.

Our pending and future patent applications may not result in patents being issued which protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive products. 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. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does.

Even if our patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our patents by developing similar or alternative technologies or products in a non-infringing manner. Our competitors may also seek approval to market their own products similar to or otherwise competitive with our products. Alternatively, our competitors may seek to market generic versions of any approved products by submitting ANDAs to the FDA in which they claim that patents owned or licensed by us are invalid, unenforceable or not infringed. In these circumstances, we may need to defend or assert our patents, or both, including by filing lawsuits alleging patent infringement. In any of these types of proceedings, a court or other agency with jurisdiction may find our patents invalid or unenforceable, or that our competitors are competing in a non-infringing manner. Thus, even if we have valid and enforceable patents, these patents still may not provide protection against competing products or processes sufficient to achieve our business objectives.

If we are unable to protect the confidentiality of our trade secrets, the value of our technology could be materially adversely affected and our business would be harmed.

While we have obtained composition of matter patents with respect to our most advanced product candidates, we also rely on trade secret protection for certain aspects of technology platform, including certain aspects of our SMART Linker drug discovery platform. We seek to protect these trade secrets, in part, by entering into non-disclosure and confidentiality agreements with parties who have access to them, such as our employees, consultants, independent contractors, advisors, contract manufacturers, suppliers and other third parties. We also enter into confidentiality and invention or patent assignment agreements with employees and certain consultants. Any party with whom we have executed such an agreement may breach that agreement and disclose our proprietary information, including our trade secrets, and we may not be able to obtain adequate remedies for such breaches. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, if any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent such third party, or those to whom they communicate such technology or information, from using that technology or information to compete with us. If any of our trade secrets were to be disclosed to or independently developed by a competitor, our business and competitive position could be harmed.

We may become involved in lawsuits to protect or enforce our patents or other intellectual property, which could be expensive, time consuming and unsuccessful.

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent

infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent s claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

Table of Contents

Even if we establish infringement, the court may decide not to grant an injunction against further infringing activity and instead award only monetary damages, which may or may not be an adequate remedy. Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during litigation. There could also be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a material adverse effect on the price of shares of our common stock. Moreover, there can be no assurance that we will have sufficient financial or other resources to file and pursue such infringement claims, which typically last for years before they are concluded. Even if we ultimately prevail in such claims, the monetary cost of such litigation and the diversion of the attention of our management and scientific personnel could outweigh any benefit we receive as a result of the proceedings.

If we are sued for infringing intellectual property rights of third parties, such litigation could be costly and time consuming and could prevent or delay us from developing or commercializing our product candidates.

Our commercial success depends, in part, on our ability to develop, manufacture, market and sell our product candidates and use our SMART Linker drug discovery platform without infringing the intellectual property and other proprietary rights of third parties. Third parties have U.S. and non-U.S. issued patents and pending patent applications relating to compounds and methods of use for the treatment of DMD, the key indication for our most advanced program. If any third-party patents or patent applications are found to cover our product candidates or their methods of use, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our product candidates, including interference proceedings before the USPTO. Third parties may assert infringement claims against us based on existing or future intellectual property rights. The outcome of intellectual property litigation is subject to uncertainties that cannot be adequately quantified in advance. The pharmaceutical and biotechnology industries have produced a significant number of patents, and it may not always be clear to industry participants, including us, which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we were sued for patent infringement, we would need to demonstrate that our product candidates, products or methods either do not infringe the patent claims of the relevant patent or that the patent claims are invalid or unenforceable, and we may not be able to do this. Proving invalidity is difficult. For example, in the United States, proving invalidity requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Even if we are successful in these proceedings, we may incur substantial costs and the time and attention of our management and scientific personnel could be diverted in pursuing these proceedings, which could significantly harm our business and operating results. In addition, we may not have sufficient resources to bring these actions to a successful conclusion.

If we are found to infringe a third party s intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. 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 access to the same technologies licensed to us. 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. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

Changes to the patent law in the United States and other jurisdictions could diminish the value of patents in general, thereby impairing our ability to protect our products.

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involves both technological and legal complexity and is therefore costly, time consuming and inherently uncertain. Recent patent reform legislation in the United States and other countries, including the Leahy-Smith America Invents Act, or the Leahy-Smith Act, signed into law in September 2011, could increase those uncertainties and costs. The Leahy-Smith Act includes a number of significant changes to U.S. patent law. These include provisions that affect the way patent applications are prosecuted, redefine prior art and provide more efficient and cost-effective avenues for competitors to challenge the validity of patents. In addition, the Leahy-Smith Act has transformed the U.S. patent system into a first to file system. The first-to-file provisions, however, only became effective in March 2013. Accordingly, it is not yet 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 make it more difficult to obtain patent protection for our inventions and increase the uncertainties and costs surrounding the prosecution of our or our

Table of Contents

collaboration partners patent applications and the enforcement or defense of our or our collaboration partners issued patents, all of which could harm our business, results of operations and financial condition.

The U.S. Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. Additionally, there have been recent proposals for additional changes to the patent laws of the United States and other countries that, if adopted, could impact our ability to enforce our proprietary technology. Depending on future actions by the U.S. Congress, the U.S. courts, the USPTO and the relevant law-making bodies in other countries, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future.

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance 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. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

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

Filing, prosecuting and defending patents on our product candidates in all countries throughout the world would be prohibitively expensive. The requirements for patentability may differ in certain countries, particularly in developing countries. 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 may obtain patent protection, but where patent enforcement is not as strong as that in the United States. These products may compete with our products in jurisdictions where we do not have any issued or licensed patents or where any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us.

Moreover, our ability to protect and enforce our intellectual property rights may be adversely affected by unforeseen changes in foreign intellectual property laws. Additionally, laws of some countries outside of the United States and Europe do not afford intellectual property protection to the same extent as the laws of the United States and Europe. Many companies have encountered significant problems in protecting and defending intellectual property rights in certain foreign jurisdictions. The legal systems of some countries, including India, China and other developing countries, do not favor the enforcement of patents and other intellectual property rights. This could make it difficult for us to stop the infringement of our patents or the misappropriation of our other intellectual property rights. For example, many foreign countries have compulsory licensing laws under which a patent owner must grant licenses to third parties. Consequently, we may not be able to prevent third parties from practicing our inventions in certain countries outside the United States and Europe. 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, if our ability to enforce our patents to stop infringing activities is inadequate. 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.

Proceedings to enforce our patent rights in foreign jurisdictions, whether or not successful, could result in substantial costs and divert our efforts and resources from other aspects of our business. Furthermore, while we intend to protect our intellectual property rights in major markets for our products, we cannot ensure that we will be able to initiate or maintain similar efforts in all jurisdictions in which we may wish to market our products. Accordingly, our efforts to protect our intellectual property rights in such countries may be inadequate.

Patent term may be inadequate to protect our competitive position on our products for an adequate amount of time.

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. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price

Table of Contents

Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the patent, which is limited to the approved indication (or any additional indications approved during the period of extension). However, the applicable authorities, including the FDA and the USPTO in the United States, and any equivalent regulatory authority in other countries, may not agree with our assessment of whether such extensions are available, and may refuse to grant extensions to our patents, or may grant more limited extensions than we request. If this occurs, our competitors may be able to take advantage of our investment in development and clinical trials by referencing our clinical and preclinical data and launch their product earlier than might otherwise be the case.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property, or claiming ownership of what we regard as our own intellectual property.

Many of our employees, including our senior management, were previously employed at universities or at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Some of these employees, including members of our senior management, executed proprietary rights, non-disclosure and non-competition agreements, or similar agreements, in connection with such previous employment. Although we try to ensure that our employees do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that we or these employees have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such third party. Litigation may be necessary to defend against such claims. If we fail in defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel or sustain damages. Such intellectual property rights could be awarded to a third party, and we could be required to obtain a license from such third party to commercialize our technology or products. Such a license may not be available on commercially reasonable terms or at all. 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 we typically require our employees, consultants and contractors who may be involved in the 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 develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

Even if we complete the necessary preclinical and clinical studies, the marketing approval process is expensive, time consuming and uncertain and may prevent us or any future collaborators from obtaining approvals for the commercialization of some or all of our product candidates. As a result, we cannot predict when or if, and in which territories, we, or any future collaborators, will obtain marketing approval to commercialize a product candidate.

The research, testing, manufacturing, labeling, approval, selling, marketing, promotion and distribution of drug products are subject to extensive regulation by the FDA and comparable foreign regulatory authorities, which regulations differ from country to country. We, and any future collaborators, are not permitted to market our product candidates in the United States or in other countries until we, or they, receive approval of an NDA from the FDA or marketing approval from applicable regulatory authorities outside the United States. Our product candidates are in various stages of development and are subject to the risks of failure inherent in drug development. We have not submitted an application for or received marketing approval for any of our product candidates in the United States or in any other jurisdiction. We have limited experience in conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of an NDA.

The process of obtaining marketing approvals, both in the United States and abroad, is lengthy, expensive and uncertain. It may take many years, if approval is obtained at all, and can vary substantially based upon a variety of factors, including the type, complexity and novelty of the product candidates involved.

In addition, changes in marketing approval policies during the development period, changes in or the enactment or promulgation of additional statutes, regulations or guidance or changes in regulatory review for each submitted product application, may cause delays in the approval or rejection of an application. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of the data obtained from preclinical and clinical testing could delay, limit or prevent marketing approval of a product candidate. Any marketing approval we, or any future collaborators, ultimately obtain may be limited or subject to restrictions or post-approval commitments that render the approved product not commercially viable.

Table of Contents

Any delay in obtaining or failure to obtain required approvals could materially adversely affect our ability or that of any future collaborators to generate revenue from the particular product candidate, which likely would result in significant harm to our financial position and adversely impact our stock price.

Failure to obtain marketing approval in foreign jurisdictions would prevent our product candidates from being marketed abroad.

In order to market and sell our products in the European Union and many other jurisdictions, we, and any future collaborators, must obtain separate marketing approvals and comply with numerous and varying regulatory requirements. The approval procedure varies among countries and can involve additional testing. The time required to obtain approval may differ substantially from that required to obtain FDA approval. The marketing approval process outside the United States generally includes all of the risks associated with obtaining FDA approval. In addition, in many countries outside the United States, it is required that the product be approved for reimbursement before the product can be approved for sale in that country. We, and any future collaborators, may not obtain approvals from regulatory authorities outside the United States on a timely basis, if at all. Approval by the FDA does not ensure approval by regulatory authorities in other countries or jurisdictions, and approval by one regulatory authority outside the United States does not ensure approval by regulatory authorities in other countries or jurisdictions or by the FDA.

Additionally, on June 23, 2016, the electorate in the United Kingdom voted in favor of leaving the European Union, commonly referred to as Brexit. On March 29, 2017, the country formally notified the European Union of its intention to withdraw pursuant to Article 50 of the Lisbon Treaty. Since a significant proportion of the regulatory framework in the United Kingdom is derived from European Union directives and regulations, the referendum could materially impact the regulatory regime with respect to the approval of our product candidates in the United Kingdom or the European Union. Any delay in obtaining, or an inability to obtain, any marketing approvals, as a result of Brexit or otherwise, would prevent us from commercializing our product candidates in the United Kingdom and/or the European Union and restrict our ability to generate revenue and achieve and sustain profitability. If any of these outcomes occur, we may be forced to restrict or delay efforts to seek regulatory approval in the United Kingdom and/or European Union for our product candidates, which could significantly and materially harm our business.

We, or any future collaborators, may not be able to obtain orphan drug designation or orphan drug exclusivity for our product candidates.

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. While we have obtained orphan drug designation from the FDA and orphan medicinal product designation from the European Commission for edasalonexent for the treatment of DMD, we, or any future collaborators, may seek orphan drug designations for other product candidates or in other jurisdictions and may be unable to obtain such designations.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective

or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because FDA has taken the position that, under certain circumstances, another drug with the same active moiety can be approved for the same condition. Specifically, the FDA s regulations provide that it can approve another drug with the same active moiety for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

In August 2017, the Congress passed the FDA Reauthorization Act of 2017 (FDARA). FDARA, among other things, codified the FDA s pre-existing regulatory interpretation, to require that a drug sponsor demonstrate the clinical superiority of an orphan drug that is otherwise the same as a previously approved drug for the same rare disease in order to receive orphan drug exclusivity. The new legislation reverses prior precedent holding that the Orphan Drug Act unambiguously requires that the FDA recognize the orphan exclusivity period regardless of a showing of clinical superiority. The FDA may further reevaluate the Orphan Drug Act and its

Table of Contents

regulations and policies. We do not know if, when, or how the FDA may change the orphan drug regulations and policies in the future, and it is uncertain how any changes might affect our business. Depending on what changes the FDA may make to its orphan drug regulations and policies, our business could be adversely impacted.

Even if we, or any future collaborators, obtain marketing approvals for our product candidates, the terms of approvals and ongoing regulation of our products may limit how we, or they, manufacture and market our products, which could materially impair our ability to generate revenue.

Once marketing approval has been granted, an approved product and its manufacturer and marketer are subject to ongoing review and extensive regulation. We, and any future collaborators, must therefore comply with requirements concerning advertising and promotion for any of our product candidates for which we or they obtain marketing approval. Promotional communications with respect to prescription drugs are subject to a variety of legal and regulatory restrictions and must be consistent with the information in the product s approved labeling. Thus, we and any future collaborators will not be able to promote any products we develop for indications or uses for which they are not approved.

In addition, manufacturers of approved products and those manufacturers facilities are required to comply with extensive FDA requirements, including ensuring that quality control and manufacturing procedures conform to cGMPs, which include requirements relating to quality control and quality assurance as well as the corresponding maintenance of records and documentation and reporting requirements. We, our contract manufacturers, any future collaborators and their contract manufacturers could be subject to periodic unannounced inspections by the FDA to monitor and ensure compliance with cGMPs.

Accordingly, assuming we, or any future collaborators, receive marketing approval for one or more of our product candidates, we, and any future collaborators, and our and their contract manufacturers will continue to expend time, money and effort in all areas of regulatory compliance, including manufacturing, production, product surveillance and quality control.

If we, and any future collaborators, are not able to comply with post-approval regulatory requirements, we, and any future collaborators, could have the marketing approvals for our products withdrawn by regulatory authorities and our, or any future collaborators , ability to market any future products could be limited, which could adversely affect our ability to achieve or sustain profitability. Further, the cost of compliance with post-approval regulations may have a negative effect on our operating results and financial condition.

Any of our product candidates for which we, or any future collaborators, obtain marketing approval in the future could be subject to post-marketing restrictions or withdrawal from the market and we, or any future collaborators, may be subject to substantial penalties if we, or they, fail to comply with regulatory requirements or if we, or they, experience unanticipated problems with our products following approval.

Any of our product candidates for which we, or any future collaborators, obtain marketing approval in the future, as well as the manufacturing processes, post-approval studies and measures, labeling, advertising and promotional activities for such product, among other things, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include submissions of safety and other post-marketing information and reports, registration and listing requirements, requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a Risk Evaluation and

Mitigation Strategy.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of a product. The FDA and other agencies, including the Department of Justice, closely regulate and monitor the post-approval marketing and promotion of products to ensure that they are manufactured, marketed and distributed only for the approved indications and in accordance with the provisions of the approved labeling. The FDA imposes stringent restrictions on manufacturers communications regarding off-label use and if we, or any future collaborators, do not market any of our product candidates for which we, or they, receive marketing approval for only their approved indications, we, or they, may be subject to warnings or enforcement action for off-label marketing. Violation of the FDCA and other statutes, including the False Claims Act, relating to the promotion and advertising of prescription drugs may lead to investigations or allegations of violations of federal and state health care fraud and abuse laws and state consumer protection laws.

In addition, later discovery of previously unknown adverse events or other problems with our products or their manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

Table of Contents

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;
- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;
- product seizure; or
- injunctions or the imposition of civil or criminal penalties.

Under the Cures Act and the Trump Administration s regulatory reform initiatives, the FDA s policies, regulations and guidance may be revised or revoked and that could prevent, limit or delay regulatory approval of our product candidates, which would impact our ability to generate revenue.

In December 2016, the 21st Century Cures Act, or Cures Act, was signed into law. The Cures Act, among other things, is intended to modernize the regulation of drugs and spur innovation, but its ultimate implementation is unclear. 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 adversely affect our business, prospects, financial condition and results of operations.

We also cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative or executive action, either in the United States or abroad. For example, certain policies of the Trump Administration may impact our business and industry. Namely, the Trump Administration has taken several executive actions, including the issuance of a number of executive orders, that could impose significant burdens on, or otherwise materially delay, the FDA s ability to engage in routine regulatory and oversight activities such as implementing statutes through rulemaking, issuance of guidance, and review and approval of marketing applications. An under-resourced FDA could result in delays in the FDA s responsiveness or in its ability to review submissions or applications, issue regulations or guidance, or

implement or enforce regulatory requirements in a timely fashion or at all. In January 2017, President Trump issued an executive order, applicable to all executive agencies including the FDA, which requires that for each notice of proposed rulemaking or final regulation to be issued in fiscal year 2017, the agency shall identify at least two existing regulations to be repealed, unless prohibited by law. These requirements are referred to as the two-for-one provisions. This executive order includes a budget neutrality provision that requires the total incremental cost of all new regulations in the 2017 fiscal year, including repealed regulations, to be no greater than zero, except in limited circumstances. For fiscal years 2018 and beyond, the executive order requires agencies to identify regulations to offset any incremental cost of a new regulation and approximate the total costs or savings associated with each new regulation or repealed regulation. In interim guidance issued by the Office of Information and Regulatory Affairs within OMB in February 2017, the administration indicates that the two-for-one provisions may apply not only to agency regulations, but also to significant agency guidance documents. In addition, on February 24, 2017, President Trump issued an executive order directing each affected agency to designate an agency official as a Regulatory Reform Officer and establish a Regulatory Reform Task Force to implement the two-for-one provisions and other previously issued executive orders relating to the review of federal regulations. It is difficult to predict how these various requirements will be implemented, and the extent to which they will impact the FDA s ability to exercise its regulatory authority. If these executive actions impose constraints on the FDA s ability to engage in oversight and implementation activities in the normal course, our business may be negatively impacted.

Recently enacted and future legislation may increase the difficulty and cost for us and any future collaborators to obtain marketing approval of and commercialize our product candidates and affect the prices we, or they, may obtain.

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures

Table of Contents

that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, or Medicare Modernization Act, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. Cost reduction initiatives and other provisions of this legislation could decrease the coverage and price that we receive for any approved products. While the Medicare Modernization Act applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates. Therefore, any reduction in reimbursement that results from the Medicare Modernization Act may result in a similar reduction in payments from private payors.

The Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively the Affordable Care Act, or ACA, became law in 2010 and includes the following provisions of potential importance to our product candidates:

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;
- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;
- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;
- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;
- extension of manufacturers Medicaid rebate liability;
- expansion of eligibility criteria for Medicaid programs;
- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;
- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and
- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation s automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional

Congressional action is taken. The American Taxpayer Relief Act of 2012, 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 and otherwise affect the prices we may obtain for any of our product candidates for which we may obtain regulatory approval or the frequency with which any such product candidate is prescribed or used.

We expect that these healthcare reforms, as well as other healthcare reform measures that may be adopted in the future, may result in additional reductions in Medicare and other healthcare funding, more rigorous coverage criteria, new payment methodologies and additional downward pressure on the price that we receive for any approved product and/or the level of reimbursement physicians receive for administering any approved product we might bring to market. Reductions in reimbursement levels may negatively impact the prices we receive or the frequency with which our products are prescribed or administered. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors.

Since enactment of the ACA, there have been numerous legal challenges and Congressional actions to repeal and replace provisions of the law. For example, with the December 2017 enactment of the Tax Cuts and Jobs Act of 2017, Congress repealed the individual mandate. The repeal of this provision, which requires most Americans to carry a minimal level of health insurance, will become effective in 2019. According to the Congressional Budget Office, the repeal of the individual mandate will cause 13 million fewer Americans to be insured in 2027 and premiums in insurance markets may rise. Additionally, on January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain ACA-mandated fees, including the so-called Cadillac tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain

Table of Contents

health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. Further, the Bipartisan Budget Act of 2018, among other things, amends the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and to close the coverage gap in most Medicare drug plans, commonly referred to as the donut hole. Further, each chamber of the Congress has put forth multiple bills designed to repeal or repeal and replace portions of the ACA. Although none of these measures has been enacted by Congress to date, Congress may consider other legislation to repeal and replace elements of the ACA. The Congress will likely consider other legislation to replace elements of the ACA during the next Congressional session.

The Trump Administration has also taken executive actions to undermine or delay implementation of the ACA. In January 2017, President Trump signed an executive order directing federal agencies with authorities and responsibilities under the ACA to waive, defer, grant exemptions from, or delay the implementation of any provision of the ACA that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. In October 2017, the President signed a second executive order allowing for the use of association health plans and short-term health insurance, which may provide fewer health benefits than the plans sold through the ACA exchanges. At the same time, the Trump Administration announced that it will discontinue the payment of cost-sharing reduction, or CSR, payments to insurance companies until Congress approves the appropriation of funds for such CSR payments. The loss of the CSR payments is expected to increase premiums on certain policies issued by qualified health plans under the ACA. A bipartisan bill to appropriate funds for CSR payments was introduced in the Senate, but the future of that bill is uncertain. Further, in July 2018 following a federal district court decision from New Mexico, the Administration announced that it would be freezing payments to insurers under the ACA to cover sicker patients until it or Congress can address the appropriate methodology for calculating and making such payments. It remains to be seen how this action will affect the implementation of the ACA.

We will continue to evaluate the effect that the ACA and its possible repeal and replacement could have on our business. It is possible that repeal and replacement initiatives, if enacted into law, could ultimately result in fewer individuals having health insurance coverage or in individuals having insurance coverage with less generous benefits. While the timing and scope of any potential future legislation to repeal and replace ACA provisions is uncertain in many respects, it is also possible that some of the ACA provisions that generally are not favorable for the research-based pharmaceutical industry could also be repealed along with ACA coverage expansion provisions. Accordingly, such reforms, if enacted, could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain marketing approval and may affect our overall financial condition and ability to develop commercialize product candidates.

Further, there have been several recent U.S. congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the costs of drugs under Medicare and reform government program reimbursement methodologies for drug products. At the federal level, the Trump administration s budget proposal for fiscal year 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. While any proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs.

In addition, on May 11, 2018, the Administration issued a plan to lower drug prices. Under this blueprint for action, the Administration indicated that the Department of Health and Human Services, or HHS, will: take steps to end the gaming of regulatory and patent processes by drug makers to unfairly protect monopolies; advance biosimilars and generics to boost price competition; evaluate the inclusion of prices in drug makers ads to enhance price competition; speed access to and lower the cost of new drugs by clarifying policies for sharing information between insurers and drug makers; avoid excessive pricing by relying more on value-based pricing by expanding outcome-based payments in Medicare and Medicaid; work to give Part D plan sponsors more negotiation power with drug makers; examine which Medicare Part B drugs could be negotiated for a lower price by Part D plans, and improving the design of the Part B Competitive Acquisition Program; update Medicare s drug-pricing dashboard to increase transparency; prohibit Part D contracts that include gag rules that prevent pharmacists from informing patients when they could pay less out-of-pocket by not using insurance; and require that Part D plan members be provided with an annual statement of plan payments, out-of-pocket spending, and drug price increases.

At the state level, individual states are increasingly aggressive in passing legislation and implementing regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing. In addition, regional health care authorities and individual hospitals are increasingly using bidding procedures to determine what pharmaceutical products and which suppliers will be included in their

Table of Contents

prescription drug and other health care programs. These measures could reduce the ultimate demand for our products, once approved, or put pressure on our product pricing.

Moreover, legislative and regulatory proposals have also been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical drugs. We cannot be sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on the marketing approvals of our drug candidates, if any, may be. In addition, increased scrutiny by the Congress of the FDA s approval process may significantly delay or prevent marketing approval, as well as subject us and any collaborators to more stringent drug labeling and post-marketing testing and other requirements.

Our relationships with customers and third-party payors, among others, will be subject to applicable anti-kickback, fraud and abuse and other healthcare laws and regulations, which could expose us to penalties, including criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.

Healthcare providers and third-party payors will play a primary role in the recommendation and prescription of any products for which we obtain marketing approval. Our arrangements with third-party payors and customers, if any, will subject us to broadly applicable fraud and abuse and other healthcare laws and regulations. The laws and regulations may constrain the business or financial arrangements and relationships through which we market, sell and distribute any products for which we obtain marketing approval. These include the following:

Anti-Kickback Statute. The federal healthcare Anti-Kickback Statute prohibits, among other things, persons and entities from knowingly and willfully soliciting, offering, receiving or providing remuneration, directly or indirectly, in cash or in kind, to induce or reward, or in return for, either the referral of an individual for, or the purchase, order or recommendation or arranging of, any good or service, for which payment may be made under a federal healthcare program such as Medicare and Medicaid;

False Claims Laws. The federal false claims laws impose criminal and civil penalties against individuals or entities for, among other things, knowingly presenting, or causing to be presented false or fraudulent claims for payment by a federal healthcare program or making a false statement or record material to payment of a false claim or avoiding, decreasing or concealing an obligation to pay money to the federal government, with potential liability including mandatory treble damages and significant per-claim penalties;

HIPAA. The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, imposes criminal and civil liability for, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters, and, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, also imposes obligations, including mandatory contractual terms and technical safeguards, with respect to maintaining the privacy, security and transmission of individually identifiable health information;

Transparency Requirements. Federal laws require applicable manufacturers of covered drugs, biologics, devices and supplies to report payments and other transfers of value to physicians and teaching hospitals and ownership and investment interests by physicians; and

Analogous State and Foreign Laws. Analogous state and foreign fraud and abuse laws and regulations, such as state anti-kickback and false claims laws, which may be broader in scope, can apply to our business activities, including sales or marketing arrangements, and claims involving healthcare items or services and are generally broad and are enforced by many different federal and state agencies as well as through private actions. Some state laws require pharmaceutical companies to comply with the pharmaceutical industry s voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government and 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 also govern the privacy and security of health information in some circumstances, many of which differ from each other in significant ways and often are not pre-empted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our business arrangements with third parties will comply with applicable healthcare laws and regulations will involve substantial costs. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, imprisonment, exclusion of products from government funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other healthcare providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

Table of Contents

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 also prohibited in the European Union. The provision of benefits or advantages to physicians is 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 publicly 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, use, disclosure, transfer, or other processing of personal data, including personal health data, of individuals in the European Union is governed by the General Data Protection Regulation (GDPR). The GDPR became effective on May 25, 2018. It imposes numerous requirements on companies that process personal data, including requirements relating to: processing health and other sensitive data; obtaining consent of individuals; providing notice to individuals regarding data processing activities; responding to data subject requests; taking certain measures when engaging third-party processors; notifying data subjects and regulators of data breaches; and implementing safeguards to protect the security and confidentiality of personal data. The GDPR imposes strict rules on the transfer of personal data to countries outside the European Union, including the United States. Failure to comply with the requirements of the GDPR may result in fines of up to 20 million Euros or four percent of annual global revenues, whichever is greater. The GDPR also confers a private right of action on data subjects and consumer associations to lodge complaints with supervisory authorities, seek judicial remedies, and obtain compensation for damages. The GDPR increases our responsibility and potential liability in relation to personal data that we process, and we may be required to change our business practices or put in place additional mechanisms ensuring compliance with the GDPR. This may be onerous and adversely affect our business, financial condition, results of operations, and prospects, and despite our efforts, there is a risk that we may be subject to fines, litigation, and reputational harm in connection with our European activities.

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 significantly harm our business.

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Although we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our 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 for failure to comply with such laws and regulations.

We maintain workers compensation insurance to cover us for costs and expenses we may incur due to injuries to our employees resulting from the use of hazardous materials, but this insurance may not provide adequate coverage against potential liabilities. However, we do not maintain insurance for environmental liability or toxic tort claims that may be asserted against us.

In addition, we may incur substantial costs in order to comply with current or future environmental, health and safety laws and regulations. Current or future environmental laws and regulations may impair our research, development or production efforts, which could adversely affect our business, financial condition, results of operations or prospects. In addition, failure to comply with these laws and regulations may result in

substantial fines, penalties or other sanctions.

Governments outside the United States tend to impose strict price controls, which may adversely affect our revenues, if any.

In some countries, such as the countries of the European Union, the pricing of prescription pharmaceuticals is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after the receipt of marketing approval for a product. To obtain reimbursement or pricing approval in some countries, we, or any future collaborators, may be required to conduct a clinical trial that compares the cost-effectiveness of our product to other available therapies. If reimbursement of our products is unavailable or limited in scope or amount, or if pricing is set at unsatisfactory levels, our business could be materially harmed.

Table of Contents

A fast track designation by the FDA may not actually lead to a faster development, regulatory review or approval process.

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet needs for this condition, the treatment sponsor may apply for FDA fast track designation. In July 2015, the FDA notified us that we obtained fast track designation for edasalonexent for the treatment of DMD. Fast track designation does not ensure that we will experience a faster development, regulatory review or approval process compared to conventional FDA procedures. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

A rare pediatric disease designation may not lead to the receipt of a Priority Review Voucher, even if edasalonexent is approved, due to the potential expiration of the FDA s Rare Pediatric Disease program.

The FDA has awarded rare pediatric disease Priority Review Vouchers to sponsors of drug candidates to treat rare pediatric disease products, if the treatment sponsors apply for this designation and meet certain criteria. Under this program, upon the approval of a qualifying NDA or biologics license application, or BLA, for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease Priority Review Voucher that can be used to obtain priority review for a subsequent NDA or BLA. The Priority Review Voucher may be sold or transferred an unlimited number of times. In September 2015, the FDA notified us that we obtained rare pediatric disease designation for edasalonexent for the treatment of DMD. With passage of the 21st Century Cures Act in December 2016, the Rare Pediatric Disease Priority Review Voucher program was reauthorized until 2020. In addition, if a product candidate is designated before October 1, 2020, as is the case with edasalonexent, it is eligible to receive a voucher if it is approved before October 2022. However, there is no guarantee that edasalonexent will be approved by that date and, therefore, we may not be in a position to obtain the Priority Review Voucher prior to expiration of the program.

We are subject to anti-corruption laws, as well as export control laws, customs laws, sanctions laws and other laws governing our operations. If we fail to comply with these laws, we could be subject to civil or criminal penalties, other remedial measures and legal expenses, which could adversely affect our business, results of operations and financial condition.

Our operations are subject to anti-corruption laws, including the U.K. Bribery Act 2010, or Bribery Act, the U.S. Foreign Corrupt Practices Act, or FCPA, and other anti-corruption laws that apply in countries where we do business and may do business in the future. The Bribery Act, FCPA and these other laws generally prohibit us, our officers, and our employees and intermediaries from bribing, being bribed or making other prohibited payments to government officials or other persons to obtain or retain business or gain some other business advantage. We may in the future operate in jurisdictions that pose a high risk of potential Bribery Act or FCPA violations, and we may participate in collaborations and relationships with third parties whose actions could potentially subject us to liability under the Bribery Act, FCPA or local anti-corruption laws. In addition, we cannot predict the nature, scope or effect of future regulatory requirements to which our international operations might be subject or the manner in which existing laws might be administered or interpreted.

We are also subject to other laws and regulations governing our international operations, including regulations administered by the governments of the United Kingdom and the United States, and authorities in the European Union, including applicable export control regulations, economic sanctions on countries and persons, customs requirements and currency exchange regulations, which we collectively refer to as the Trade Control Laws.

There is no assurance that we will be completely effective in ensuring our compliance with all applicable anti-corruption laws, including the Bribery Act, the FCPA or other legal requirements, including Trade Control Laws. If we are not in compliance with the Bribery Act, the FCPA and other anti-corruption laws or Trade Control Laws, we may be subject to criminal and civil penalties, disgorgement and other sanctions and remedial measures, and legal expenses, which could have an adverse impact on our business, financial condition, results of operations and liquidity. Likewise, any investigation of any potential violations of the Bribery Act, the FCPA, other anti-corruption laws or Trade Control Laws by U.K., U.S. or other authorities could also have an adverse impact on our reputation, our business, results of operations and financial condition.

Risks Related to Employee Matters, Managing Growth and Information Technology

Our future success depends on our ability to retain our senior management and to attract, retain and motivate qualified personnel.

We are highly dependent on members of our senior management, including Jill C. Milne, Ph.D., our President and Chief Executive Officer, Joanne Donovan, M.D., Ph.D., our Chief Medical Officer, and Andrew Nichols, Ph.D., our Chief Scientific Officer. The loss of the services of any of these persons could impede the achievement of our research, development and commercialization

Table of Contents

objectives. Recruiting and retaining qualified scientific, clinical, manufacturing, sales and marketing personnel will also be critical to our success. The loss of the services of our executive officers or other key employees and any difficulties in recruiting and retaining other critical personnel could impede the achievement of our research, development and commercialization objectives and seriously harm our ability to successfully implement our business strategy.

We undertook an organizational restructuring in April 2018 to reduce costs, in which we reduced our workforce by 40%. This restructuring increased our dependence on senior management and other key employees and could present significant additional risks including unintended consequences, such as attrition beyond our planned reduction in workforce and reduced employee morale, which may cause our remaining employees to seek alternate employment, fail to meet operational objectives as a result of decreased staffing, increased demands on our remaining employees and diversion of management s attention from ongoing business activities to implement and oversee the restructuring.

If we are unable to retain our executive officers or other key employees, replacing them may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

We expect to grow our organization significantly if our Phase 3 trial of edasalonexent for the treatment of DMD is successful, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

We expect to experience significant growth in the number of our employees and the scope of our operations, particularly in the areas of drug manufacturing, regulatory affairs and sales, marketing and distribution, if our Phase 3 trial of edasalonexent for the treatment of DMD is successful and we obtain FDA approval to market edasalonexent for the treatment of DMD in the United States. To manage these growth activities, we would need to implement and improve our managerial, operational and financial systems, expand our facilities and to recruit and train additional qualified personnel. Our management may need to devote a disproportionate amount of its attention to managing these growth activities. Due to our limited financial resources and the limited experience of our management team in managing a company with such anticipated growth, we may not be able to effectively manage the expansion of our operations or identify, recruit and train additional qualified personnel. Our inability to manage the expansion of our operations effectively may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could also require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If we are unable to effectively manage our expected growth, our expenses may increase more than expected, our ability to generate revenues could be reduced and we may not be able to implement our business strategy, including the successful commercialization of our product candidates.

Security breaches and other disruptions to our information technology systems could compromise our information, disrupt our business and expose us to liability, which would cause our business and reputation to suffer.

In the ordinary course of our business, we collect, process and store sensitive data, including intellectual property, as well as our proprietary business information and employee data. We also rely to a large extent on information technology systems to operate our business. We have outsourced elements of our confidential information processing and information technology structure, and as a result, we are managing independent vendor relationships with third parties who may or could have access to our confidential information. The secure maintenance of this information is important to our operations and business strategy. Despite our security measures, our information technology infrastructure, and that of our vendors and third-party providers, may be vulnerable to attacks by hackers or breached due to employee error, malfeasance or other disruptions. We, our vendors and third-party providers could be susceptible to third party attacks on our, and their, information security systems, which attacks are of ever-increasing levels of sophistication and are made by groups and individuals with a wide range of motives and expertise, including organized criminal groups, hacktivists, nation states and others. While we have invested in information technology security measures and the protection of confidential information, there can be no assurance that our efforts will prevent service interruptions or security breaches. Although we are not aware of any material information security incidents to date, we have detected common types of attempts to attack our information technology systems and data using means that have included phishing. Any service interruptions or security breaches of our information technology systems may substantially impair our

Table of Contents

ability to operate our business and could compromise our networks, or those of our vendors and third-party providers, and the information stored could be accessed, publicly disclosed, lost or stolen. Any such access, disclosure, or other loss of information could result in legal claims or proceedings, liability under laws that protect the privacy of personal information, disrupt our operations, and damage our reputation, any of which could adversely affect our business.

Risks Related to Our Common Stock

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

Our shares of common stock began trading on The Nasdaq Global Market in June 2015. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price for our common stock and thereby affect the ability of our stockholders to sell their shares. 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.

If we were to be delisted from The Nasdaq Stock Market, it could make trading in our stock more difficult.

There are various quantitative listing requirements for a company to remain listed on The Nasdaq Stock Market, or Nasdaq, including maintaining a minimum bid price of $1.00 per share. The closing price of our common stock from July 1, 2018 to October 31, 2018 ranged from a high of $0.91 per share to a low of $0.62 per share. On August 3, 2018, we received a deficiency letter from the Listing Qualifications Department of Nasdaq notifying us that, for the last 30 consecutive business days, the bid price for our common stock had closed below the minimum $1.00 per share requirement for continued inclusion on the Nasdaq Global Market. We have been provided an initial period of 180 calendar days, or until January 30, 2019, to regain compliance with this rule. If, at any time before January 30, 2019, the bid price for our common stock closes at $1.00 or more for a minimum of 10 consecutive business days, we may be eligible to regain compliance with the minimum bid requirement. Under certain circumstances, Nasdaq could require that the minimum bid price exceed $1.00 for more than ten consecutive business days before determining that we comply with Nasdaq s continued listing standards.

If we do not regain compliance within the allotted compliance period, including any extensions that may be granted by Nasdaq, Nasdaq would notify us that our common stock would be delisted, eliminating the only established trading market for our shares. We would then be entitled to appeal this determination to a Nasdaq Hearings Panel, which we may request to review the matter in a written or an oral hearing.

In the event we are delisted from Nasdaq, we would be forced to list our shares on the OTC Electronic Bulletin Board or another quotation medium, such as the pink sheets, depending on our ability to meet the specific listing requirements of those quotation systems. As a result, an investor might find it more difficult to trade, or to obtain accurate price quotations for, such shares. Delisting might also reduce the visibility, liquidity, and price of our common stock.

The price of our common stock is likely to be highly volatile, which could result in substantial losses for our stockholders.

Our stock price is likely to be highly volatile. The stock market in general and the market for smaller pharmaceutical and biotechnology companies in particular have experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our investors may lose some or all of their investments. The market price for our common stock may be influenced by many factors, including:

- the timing and results of clinical trials of edasalonexent and any of our other product candidates;
- commencement or termination of collaborations for our development programs;
- failure or discontinuation of any of our development programs;
- the success of existing or new competitive products or technologies;
- results of clinical trials of product candidates of our competitors;
- 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 develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;

Table of Contents

- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- 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 this Risk Factors section.

Additionally, in the past, securities class action litigation has often been brought against a company following a decline in the market price of its securities. This risk is especially relevant for us because smaller pharmaceutical and biotechnology companies have experienced significant stock price volatility in recent years. If we face such litigation, it could result in substantial costs and a diversion of management s attention and resources, which could harm our business.

We are an emerging growth company and a smaller reporting company, and the reduced disclosure requirements applicable to such companies may make our common stock less attractive to investors.

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company until the last day of our fiscal year following the fifth anniversary of our IPO, subject to specified conditions. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, 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. Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company, which would allow us to take advantage of many of the same exceptions from disclosure requirements, including not being required to comply with the auditor attestation requirements of SOX Section 404 and reduced disclosure obligations regarding executive compensation. Investors may find our common stock less attractive as a result of our reliance on these exemptions. 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.

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

As a public company, and particularly after we are no longer an emerging growth company or a smaller reporting company, we have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The Nasdaq Global Market and other

applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being a public company and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. We are currently evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404 we are required to furnish reports by our management on our internal control over financial reporting with our Annual Reports on Form 10-K with the SEC. However, while we remain an emerging growth company or a smaller reporting company, we will not be required to include attestation reports on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX 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, 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

Table of Contents

able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

A portion of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. As of September 30, 2018, we had outstanding 71,038,419 shares of common stock. The holders of an aggregate of 4,098,653 of these outstanding shares of common stock, along with the holders of warrants to purchase 24,556 shares of common stock, have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. Following registration, such shares could be freely sold in the public market, subject to volume limitations applicable to affiliates. In addition, as of September 30, 2018 we had outstanding warrants to purchase an additional 42,000,000 shares of common stock at an exercise price of $1.20 per share. These warrants are fully exercisable and we have registered the issuance of shares upon exercise of these warrants under a registration statement. As a result, the shares issuable upon exercise of these warrants can be freely sold in the public marked upon issuance, subject to volume limitations applicable to affiliates.

We have filed registration statements registering all of the shares of common stock that we may issue under our equity compensation plans. As of September 30, 2018, we had outstanding options to purchase an aggregate of approximately 4,489,457 shares of our common stock, of which options to purchase approximately 1,734,044 shares were vested. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates.

The number of shares of common stock underlying our outstanding warrants is significant in relation to our currently outstanding common stock, which could have a negative effect on the market price of our common stock and make it more difficult for us to raise funds through future equity offerings.

As part of our June 2018 equity financing we issued warrants to purchase an aggregate of 42,000,000 shares of common stock at an exercise price of $1.20 per share. As of September 30, 2018, all of these warrants remained outstanding and, upon exercise in full of these warrants, the shares issuable upon exercise would represent a significant portion of our outstanding common stock. The warrants were fully exercisable upon issuance and remain exercisable for five years from the date of issuance. We have registered the issuance of shares upon exercise of these warrants under a registration statement under the Securities Act of 1933, as amended, and, accordingly, such shares can be freely sold into the public market upon issuance, subject to volume limitations applicable to affiliates. Sales of these shares could cause the market price of our common stock to decline significantly. Furthermore, if our stock price rises, the holders of these warrants may be more likely to exercise their warrants and sell a large number of shares, which could negatively impact the market price of our common stock and reduce or eliminate any appreciation in our stock price that might otherwise occur.

We may also find it more difficult to raise additional equity capital while these warrants are outstanding. At any time during which these warrants are likely to be exercised, we may be unable to obtain additional equity capital on more favorable terms from other sources. In addition, the exercise of these warrants would result in a significant increase in the number of our outstanding shares of common stock, which could have the effect of significantly diluting the interest of our current stockholders, and following such exercise the former holders of such warrants could have significant influence over our company as a result of the shares of common stock they acquire upon such exercise.

We do not anticipate paying any cash dividends on our capital stock in the foreseeable future, accordingly, stockholders must rely on capital appreciation, if any, for any return on their investment.

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. Any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be investors sole source of gain for the foreseeable future.

Provisions in our corporate charter documents and under 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 corporate charter and our 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 our investors might otherwise receive a premium for their shares. These provisions could also limit the price that investors might be willing to pay in the future for shares of our common

Table of Contents

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 all members of the board are not 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 nominations for election to the board of directors or for proposing matters that can be acted on at stockholder meetings;
- 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 a special meeting of stockholder meetings;
- authorize our board of directors to issue preferred stock without stockholder approval, which could be used to institute a 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 charter or bylaws.

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, 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. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders.

Our certificate of incorporation designates the state courts in the State of Delaware or, if no state court located within the State of Delaware has jurisdiction, the federal court for the District of Delaware, as the sole and exclusive forum for certain types of actions and proceedings that may be initiated by our stockholders, which could discourage lawsuits against the company and our directors and officers.

Our certificate of incorporation provides that, unless we consent in writing to the selection of an alternative forum, the Court of Chancery of the State of Delaware (or, if the Court of Chancery does not have jurisdiction, the federal district court for the District of Delaware) will be the sole and exclusive forum for any derivative action or proceeding brought on our behalf, any action asserting a claim of breach of a fiduciary duty owed by any of our directors, officers or employees to our company or our stockholders, any action asserting a claim against us arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or any action asserting a claim against us governed by the internal affairs doctrine. This exclusive forum provision may limit the ability of our stockholders to bring a claim in a judicial forum that such stockholders find favorable for disputes with us or our directors or officers, which may discourage such

lawsuits against us and our directors and officers.

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

The trading market for our common stock will likely depend, in part, on the research and reports that securities or industry analysts publish about us or our business. We do not have any control over these analysts. There can be no assurance that analysts will cover us, or provide favorable coverage. If one or more analysts downgrade our stock or change their opinion of our stock, our share price would likely decline. In addition, if one or more analysts cease coverage of our company or fail to regularly publish reports on us, we could lose visibility in the financial markets, which could cause our share price or trading volume to decline.

Table of Contents

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibits Index below:

Table of Contents

Exhibit Number	Exhibit
10.1	Sublease Agreement, dated as of September 14, 2018, by and between Inzen Therapeutics, Inc. and Catabasis Pharmaceuticals, Inc. (incorporated by reference to Exhibit 99.1 to the Registrant s Current Report on Form 8-K (File No. 001-37467) filed with the Securities and Exchange Commission on October 16, 2018)
31.1	Certification of principal executive officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of principal financial officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certification pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of The Sarbanes-Oxley Act of 2002, by the Registrant s principal executive officer and principal financial officer
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Calculation Linkbase Document
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB	XBRL Taxonomy Label Linkbase Document
101.PRE	XBRL Taxonomy Presentation Linkbase Document

Table of Contents

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) 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.

Catabasis Pharmaceuticals, Inc.

Date: November 13, 2018

By: /s/ DEIRDRE A. CUNNANE, J.D.
Deirdre A. Cunnane, J.D.
Treasurer and Chief Legal Officer (Principal Financial Officer)