CATABASIS PHARMACEUTICALS INC
Form 10-Q
November 10, 2016
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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, 2016

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** (Address of Principal Executive Offices)	**02139** (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 and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). **Yes** x **No** o

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

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

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, 2016, there were 18,652,303 shares of the registrant s Common Stock, par value $0.001 per share, outstanding.

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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 plans to identify, develop and commercialize novel therapeutics based on our SMART linker technology platform;

- ongoing and planned clinical trials for our 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 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.

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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, 2016	December 31, 2015
Assets		
Current assets:		
Cash and cash equivalents	$ 26,464	$ 62,780
Available-for-sale securities	20,803	
Prepaid expenses and other current assets	1,032	772
Total current assets	48,299	63,552
Property and equipment, net	641	504
Restricted cash		113
Total assets	$ 48,940	$ 64,169
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 1,721	$ 1,328
Accrued expenses	3,595	3,278
Current portion of notes payable, net of discount	3,225	3,173
Total current liabilities	8,541	7,779
Deferred rent, net of current portion		26
Notes payable, net of current portion and discount	3,296	5,720
Other liability	242	151
Total liabilities	12,079	13,676
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, 150,000,000 shares authorized; 18,639,285 and 15,313,297 shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively	19	15
Additional paid-in capital	172,137	158,488
Accumulated other comprehensive loss	(3)	
Accumulated deficit	(135,292)	(108,010)
Total stockholders equity	36,861	50,493
Total liabilities and stockholders equity	$ 48,940	$ 64,169

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

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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,	
	2016	**2015**	**2016**	**2015**
Operating expenses:				
Research and development	$ 5,936	$ 5,813	$ 19,190	$ 16,360
General and administrative	2,347	2,388	7,695	5,966
Total operating expenses	8,283	8,201	26,885	22,326
Loss from operations	(8,283)	(8,201)	(26,885)	(22,326)
Other (expense) income:				
Interest expense	(199)	(282)	(662)	(709)
Interest and investment income	50		183	
Other income, net	13	(2)	82	11
Total other expense, net	(136)	(284)	(397)	(698)
Net loss	$ (8,419)	$ (8,485)	$ (27,282)	$ (23,024)
Net loss per share - basic and diluted	$ (0.54)	$ (0.55)	$ (1.77)	$ (4.11)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	15,512,608	15,297,794	15,407,747	5,596,412

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

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Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Statements of Comprehensive Loss

(In thousands)

(Unaudited)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Net Loss	$ (8,419)	$ (8,485)	$ (27,282)	$ (23,024)
Other comprehensive loss:				
Unrealized losses on available-for-sale securities	(12)		(3)	
Total other comprehensive loss:	(12)		(3)	
Comprehensive loss	$ (8,431)	$ (8,485)	$ (27,285)	$ (23,024)

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

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Catabasis Pharmaceuticals, Inc.

Condensed Consolidated Statements of Cash Flows

(In thousands)

(Unaudited)

	Nine Months Ended September 30,	
	2016	**2015**
Operating activities		
Net loss	$ (27,282)	$ (23,024)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	300	130
Stock-based compensation expense	1,575	1,143
Accretion of discount/premium on investment securities	107	
Non-cash interest expense	218	209
Gain on the sale of fixed assets	(49)	
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(147)	(237)
Other assets		2
Accounts payable	178	619
Accrued expenses	252	112
Deferred rent	(26)	(4)
Net cash used in operating activities	(24,874)	(21,050)
Investing activities		
Purchases of available-for-sale securities	(41,497)	
Sales and maturities of available-for-sale securities	20,584	
Purchases of property and equipment	(436)	(60)
Sale of property and equipment	49	
Net cash used in investing activities	(21,300)	(60)
Financing activities		
Proceeds from initial public offering, net of issuance costs		61,776
Proceeds from issuance of preferred stock, net of issuance costs		12,331
Proceeds from registered direct offering, net of issuance costs	10,730	
Proceeds from at-the-market offering, net of issuance costs	1,483	
Proceeds from exercise of common stock options and warrants	145	51
Proceeds from borrowing		5,000
Payments on borrowing	(2,500)	
Debt issuance costs		(7)
Net cash provided by financing activities	9,858	79,151
Net (decrease) increase in cash and cash equivalents	(36,316)	58,041
Cash and cash equivalents, beginning of period	62,780	14,668
Cash and cash equivalents, end of period	$ 26,464	$ 72,709
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 661	$ 500
Non-cash financing activities		
At-the-market offering costs in accounts payable and accrued liabilities	$ 157	$
Registered direct offering costs in accounts payable and accrued liabilities	$ 123	$
Warrants for the purchase of series B preferred stock issued in conjunction with credit facility	$	$ 107

Initial public offering costs in accounts payable and accrued liabilities	$	$ 32
Reclassification of deferred IPO costs from non-current assets to additional paid-in capital	$	$ 1,787
Reclassification of warrant liability to additional paid-in capital	$	$ 206

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

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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 focused on the discovery, development and commercialization of novel therapeutics based on the Company s proprietary Safely Metabolized And Rationally Targeted, or SMART linker drug discovery platform. The Company s SMART linker technology platform enables the Company to engineer product candidates that can simultaneously modulate multiple targets in a disease. The Company s proprietary product candidates impact pathways that are central to diseases where efficacy may be optimized by a multiple target approach. The Company s primary focus is on treatments for rare diseases. The Company has applied its SMART linker drug discovery platform to build an internal pipeline of product candidates for rare diseases and plans to pursue partnerships to develop additional product candidates. The Company was incorporated in the State of Delaware on June 26, 2008.

Liquidity

In June 2015, the Company completed its initial public offering (the IPO). All of the shares issued and sold in the IPO were registered pursuant to a registration statement on Form S-1, as amended. An aggregate of 5,750,000 shares of the Company s common stock (Common Stock) registered pursuant to the registration statement were sold at a price to the public of $12.00 per share (including 750,000 shares of Common Stock sold pursuant to the exercise of an overallotment option granted to the Company s underwriters in connection with the IPO). Net proceeds of the IPO were $61.7 million, after deducting underwriting discounts, commissions and offering-related expenses payable by the Company of approximately $7.3 million. In connection with the IPO, all shares of the Company s convertible preferred stock (Preferred Stock) were automatically converted into an aggregate of 9,029,549 shares of Common Stock and outstanding warrants to purchase 315,688 shares of Preferred Stock were automatically converted into warrants to purchase 24,566 shares of Common Stock.

In August 2016, the Company entered into a sales agreement with Cowen and Company LLC (Cowen), pursuant to which the Company may issue and sell shares of Common Stock for an aggregate maximum offering amount of $10.0 million under an at-the-market (ATM) offering program. Cowen is not required to sell any specific amount, but acts as the Company s sales agent using commercially reasonable efforts consistent with its normal trading and sales practices. Shares sold pursuant to the sales agreement have been sold pursuant to a shelf registration statement, which became effective on July 19, 2016 (the Shelf Registration Statement). The Company pays Cowen 3% of the gross proceeds from any Common Stock sold through the sales agreement.

During the three months ended September 30, 2016, the Company sold an aggregate of 368,015 shares of Common Stock pursuant to the ATM program, at an average price of $4.35 per share, for gross proceeds of $1.6 million, resulting in net proceeds of $1.3 million after deducting sales commissions and offering expenses of approximately $0.3 million in the aggregate. On September 22, 2016, the Company reduced the amount

of Common Stock that it was offering pursuant to the sales agreement, such that it was only offering $1.4 million of Common Stock in addition to the $1.6 million of Common Stock it had sold under the ATM program as of that date. As of September 30, 2016, $1.4 million of common stock remained available for sale under the ATM program. On November 10, 2016, the Company increased the amount of common stock being offered pursuant to the sales agreement, such that the Company is offering an additional $8.4 million of common stock for sale under the sales agreement from and after such date.

In September 2016, the Company closed an underwritten registered direct offering, in which it sold 2,875,000 shares of Common Stock (including 375,000 shares of Common Stock sold pursuant to the exercise of an option by the underwriter to purchase additional shares) at an offering price of $4.00 per share. The shares sold in the offering were sold pursuant to the Shelf Registration Statement. The Company received aggregate gross proceeds from the offering of $11.5 million, resulting in net proceeds of $10.6 million after deducting underwriting discounts and commissions and offering expenses of approximately $0.9 million in the aggregate.

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 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.

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The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since inception.

As of September 30, 2016, the Company had an accumulated deficit of $135.3 million. The Company has relied on its ability to fund its operations through private and public equity and debt financings. As the Company continues to incur losses, transition to profitability is dependent upon the successful development, approval, and commercialization of its products and product candidates and the achievement of a level of revenues adequate to support its cost structure. The Company may never achieve profitability, and unless and until it does, the Company will continue to need to raise additional capital. However, the Company may be unable to raise additional funds or enter into such other arrangements when needed on favorable terms or at all.

At September 30, 2016, the Company believes that it has cash, cash equivalents and available-for-sale investments to fund its current operating plan through at least September 30, 2017. For more information, refer to the section titled Liquidity and Capital Resources in Item 2, Management s Discussion and Analysis of Financial Condition and Results of Operations.

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, 2015 and notes thereto, included in the Company s annual report on Form 10-K filed with the SEC on March 15, 2016 (the 2015 Annual Report on Form 10-K).

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, 2016, the results of its operations for the three and nine months ended September 30, 2016 and 2015 and its cash flows for the nine months ended September 30, 2016 and 2015. Such adjustments are of a normal and recurring nature. The results for the three and nine months ended September 30, 2016 are not necessarily indicative of the results for the year ending December 31, 2016, 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. These condensed consolidated financial statements have been prepared in accordance with U.S. GAAP and include all adjustments necessary for the fair presentation of the Company s financial position for the periods presented.

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.

Prior to completion of the IPO, the Company utilized significant estimates and assumptions in determining the fair value of its Common Stock. The board of directors determined the estimated fair value of the Common Stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which the Company sold shares of Preferred Stock, the achievement of research and development milestones, the superior rights and preferences of securities senior to the Common Stock at the time and the likelihood of achieving a liquidity event, such as an initial public offering or sale of the Company.

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The Company utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants (AICPA), Audit and Accounting Practice Aid Series: Valuation of Privately Held Company Equity Securities Issued as Compensation (AICPA Practice Aid), to estimate the fair value of its Common Stock. The methodologies included the Option Pricing Method utilizing the Back-solve Method (a form of the market approach defined in the AICPA Practice Aid) and the Probability-Weighted Expected Return Method based upon the probability of occurrence of certain future liquidity events such as an initial public offering or sale of the Company. Each valuation methodology included estimates and assumptions that required the Company s judgment. Significant changes to the key assumptions used in the valuations could result in different fair values of the Common Stock at each valuation date.

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, 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 life 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, net of estimated forfeitures, equal to the grant date fair value of stock options on a straight-line basis over the requisite service period.

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, 2016 and 2015, 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):

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
Research and development	$ 182	$ 173	$ 533	$ 505
General and administrative	328	315	1,042	638
Total	$ 510	$ 488	$ 1,575	$ 1,143

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

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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,	
	2016	2015	2016	2015
Stock options	2,486,409	1,712,011	2,486,409	1,712,011
Common stock warrants	24,566	59,405	24,566	59,405
	2,510,975	1,771,416	2,510,975	1,771,416

Deferred Financing Costs

On April 7, 2015, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) No. 2015-03, *Simplifying the Presentation of Debt Issuance Costs* (ASU 2015-03). ASU 2015-03 requires debt issuance costs to be presented in an entity s balance sheet as a direct deduction from the associated debt liability. The standard is retrospectively effective for annual reporting periods beginning after December 15, 2015.

The Company adopted the standard in the three months ended March 31, 2016, which resulted in a balance sheet reclassification of issuance costs in connection with its notes payable of approximately $32 thousand recorded in prepaid expenses and other current assets and approximately $22 thousand recorded in other assets to a reduction in current portion of notes payable, net of discount and in notes payable, net of current portion and discount, respectively. The Company s adoption of this standard did not have any impact on its results of operations or cash flows for the nine months ended September 30, 2016.

Recent Accounting Pronouncements

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 August 2014, the FASB issued ASU No. 2014-15, *Disclosure of Uncertainties about an Entity s Ability to Continue as a Going Concern* (ASU No. 2014-15), which provides guidance on determining when and how to disclose going concern uncertainties in financial statements. The new standard requires management to perform interim and annual assessments of an entity s ability to continue as a going concern. ASU No. 2014-15 is effective for an entity s annual period ending after December 15, 2016, and for annual periods and interim periods thereafter. Early application is permitted. Had the Company adopted this guidance in the quarter ended September 30, 2016, additional disclosure in the Company s financial statements would have resulted. The Company faces certain risks and uncertainties, as further described in Note 1, that

could have affected this analysis. The Company will continue to evaluate the potential impact that ASU No. 2014-15 may have in the future.

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, including the income tax consequences, classification of awards as either equity or liabilities, an option to recognize gross stock compensation expense with actual forfeitures recognized as they occur, as well as certain classifications on the statement of cash flows. The amendments are effective for annual reporting periods beginning after December 15, 2018, but early adoption is permitted. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

In March 2016, the FASB issued ASU 2016-09, *Improvements to Employee Share-Based Payment Accounting*. This standard amends the existing guidance in an attempt to simplify several aspects of accounting for employee share-based payment transactions. It is effective for annual reporting periods beginning after December 15, 2016, but early adoption is permitted. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

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Summary of Significant Accounting Policies

The Company s significant accounting policies are described in Note 2, Summary of Significant Accounting Policies, in the 2015 Annual Report on Form 10-K, and there were no significant changes to such policies in the nine months ended September 30, 2016.

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, 2016 and December 31, 2015 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 in 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, 2016 or 2015.

The Company s investment portfolio includes 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. In addition, model processes are used to assess interest rate impact and develop prepayment scenarios. These models take into consideration relevant credit information, perceived market movements, sector news and economic events. The inputs into these models may include benchmark yields, reported trades, broker-dealer quotes, issuer spreads and other relevant data. 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 determines the fair value of available-for-sale securities (Note 4) using Level 2 inputs. Below is a summary of assets measured at fair value on a recurring basis (in thousands):

	As of September 30, 2016			
	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	$ 23,396	$	$	$ 23,396
U.S. government-sponsored securities		820		820
Available-for-sale securities:				
Corporate debt securities		18,743		18,743
U.S. government-sponsored securities		2,060		2,060
Total assets	$ 23,396	$ 21,623	$	$ 45,019

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	As of December 31, 2015			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Money market funds	$ 62,004	$	$	$ 62,004
Total assets	$ 62,004	$	$	$ 62,004

At September 30, 2016 and December 31, 2015, the Company s cash equivalents consisted principally of money market funds, which approximated their fair value due to their short-term nature.

At September 30, 2016 and December 31, 2015, the carrying value of the Company s debt approximated fair value, which was determined using Level 3 inputs, including a quoted interest rate.

4. Available-for-Sale Securities

As of December 31, 2015, the Company held no available-for-sale securities. The following table summarizes the available-for-sale securities held at September 30, 2016 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
September 30, 2016				
Corporate debt securities	$ 18,746	$	$ (3)	$ 18,743
U.S. government-sponsored securities	2,060			2,060
Total	$ 20,806	$	$ (3)	$ 20,803

The contractual maturities of all securities held at September 30, 2016 were one year or less. There were seventeen available-for-sale securities in an unrealized loss position at September 30, 2016, none of which had been in an unrealized loss position for more than 12 months. The aggregate fair value of these securities at September 30, 2016 was approximately $15.7 million. The Company reviews its investments for other-than-temporary impairment whenever the fair value of an investment is less than amortized cost and evidence indicates that an investment s carrying amount is not recoverable within a reasonable period of time. To determine whether an impairment is other-than-temporary, the Company considers whether it has the ability and intent to hold the investment until a market price recovery and considers whether evidence indicating the cost of the investment is recoverable outweighs evidence to the contrary. The Company does not intend to sell the investments that were in unrealized loss positions at September 30, 2016, and it is not more likely than not that the Company will be required to sell the investments before recovery of their amortized cost bases, which may be at maturity. Accordingly, the Company concluded that it did not hold any securities with other-than-temporary impairment at September 30, 2016.

Gross realized gains and losses on the sales of available-for-sale securities 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 securities 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. During the three and nine-month periods ended September 30, 2016 the Company received $0 and $7.8 million in proceeds from sales of available-for-sale securities, respectively, the gains on which were not material to the Company s condensed consolidated results of operations.

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5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	September 30, 2016	December 31, 2015
Accrued compensation	$ 1,004	$ 1,181
Accrued contracted research costs	2,011	1,261
Accrued professional fees	248	181
Accrued other	332	655
Total	$ 3,595	$ 3,278

6. Notes Payable

On August 27, 2014, the Company entered into a credit facility with MidCap Financial Trust, Flexpoint MCLS Holdings, LLC and Square 1 Bank, which was subsequently amended in March and December 2015 (as amended, the Credit Facility). The Credit Facility provided for initial borrowings of $5.0 million under a term loan (Term Loan A) and additional borrowings of up to $20.0 million under other term loans, for a maximum of $25.0 million. On August 27, 2014, the Company received proceeds of $5.0 million from the issuance of promissory notes under Term Loan A. On March 31, 2015, the Company received proceeds of $5.0 million from the issuance of promissory notes under another term loan (Term Loan B). The remaining amounts available for borrowing under this arrangement expired unused as of July 31, 2015, leaving total borrowings under the Credit Facility at $10.0 million. All amounts outstanding under the Credit Facility are due on October 1, 2018 and are collateralized by substantially all of the Company s personal property, other than its intellectual property.

Interest-only payments were due monthly on amounts outstanding under the Credit Facility until September 1, 2015 and, thereafter, interest and principal payments are due in 36 equal monthly installments from October 1, 2015 through September 1, 2018. Amounts due under the Credit Facility bear interest at an annual rate of 7.49%. In addition, a final payment equal to 3.48% of any amounts drawn under the Credit Facility is 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 is being accrued as additional interest expense using the effective-interest method from the date of issuance through the maturity date, and is recorded within other long-term liabilities. In the event of prepayment, the Company is obligated to pay 1% to 3% of the amount of the outstanding principal depending upon the timing of the prepayment. The effective interest rate as of September 30, 2016 was 11.2%.

In conjunction with Term Loan A, the Company issued warrants (the 2014 Warrants) to purchase 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders. In conjunction with Term Loan B, the Company issued warrants (the 2015 Warrants) to purchase an additional 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders. Upon the closing of the Company s IPO on June 30, 2015, the 2014 Warrants and 2015 Warrants were automatically converted into warrants to purchase an aggregate 24,566 shares of Common Stock at an exercise price of $12.2114 per share. The 2014 Warrants and 2015 Warrants were exercisable immediately and have seven-year lives. The 2014 Warrants and 2015 Warrants were initially valued at $0.1 million and $0.1 million, respectively, using the Black-Scholes option-pricing model. The Company recorded debt discounts of $0.1 million and $0.1 million upon issuance of the 2014 Warrants and 2015 Warrants, respectively, which are being accreted as interest expense using the effective-interest method over the remaining term of the loan.

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There are no financial covenants associated with the Credit Facility; however, there are negative covenants restricting the Company s activities, including limitations on asset dispositions, mergers or acquisitions; encumbering or granting a security interest in its intellectual property; incurring indebtedness or liens; paying dividends; making certain investments; and entering into certain other business transactions.

Upon the occurrence and continuation of an event of default, the lenders have the right to exercise certain remedies against the Company and the collateral securing the loans under the Credit Facility, including cash. Events of default include, among other things, failure to pay amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, which includes a material adverse change in the business, operations or conditions (financial or otherwise) of the Company or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against the Company in an amount greater than $250,000. The occurrence of a material adverse event could result in acceleration of the payment of the debt. At September 30, 2016 and December 31, 2015, the Company concluded that the likelihood of the acceleration of the debt was remote, as a material adverse event had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments. Following the occurrence and during the continuance of an event of default, borrowings under the Credit Facility shall bear interest at a rate per annum, which is five hundred basis points, or 5.00%, above the rate that is otherwise applicable.

The Company assessed all terms and features of the Credit Facility in order to identify any potential embedded features that would require bifurcation or any beneficial conversion features. As part of this analysis, the Company assessed the economic characteristics and risks of the Credit Facility, including put and call features. The Company determined that all features of the Credit Facility were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability, or the fair value of the feature was immaterial to the Company s financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting.

Estimated future principal payments at September 30, 2016 are as follows (in thousands):

Year Ending December 31,		**Amount**
Remainder 2016	$	834
2017		3,333
2018		2,500
Total	$	6,667
Less: discount for warrants and costs paid to lender		(146)
Less: current portion		(3,225)
Note payable, net of current portion and discount	$	3,296

During the three months ended September 30, 2016 and 2015, the Company recognized $0.2 million and $0.3 million of interest expense related to the Credit Facility, respectively. During the nine months ended September 30, 2016 and 2015, the Company recognized $0.7 million and $0.7 million of interest expense related to the Credit Facility, respectively.

7. Commitments

In November 2010, the Company entered into a five-year, non-cancelable operating lease for office and laboratory space that provided for a five-year extension upon the completion of the lease term. In December 2011, the Company signed a lease amendment (the 2011 Lease

Amendment) that expanded the leased premises beginning in the second quarter of 2012. The 2011 Lease Amendment also extended the term of the existing lease through June 30, 2017. The 2011 Lease Amendment includes a free rent period for the expansion premises and escalating rent payments. In July 2015, the Company signed another lease amendment (the 2015 Lease Amendment) that expanded the leased premises beginning in the third quarter of 2015. The 2015 Lease Amendment includes escalating rent payments and is effective through June 30, 2017. The Company is recognizing rent expense on a straight-line basis over the lease term. The Company did not exercise its right to extend the term of the existing lease and subsequently entered in to an amendment that extended the term of the lease through June 2018. See Note 11.

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Future minimum payments required under the non-cancelable operating lease as of September 30, 2016 are summarized as follows (in thousands):

Period Ending December 31,	Amount
Remainder 2016	$ 234
2017	467
Total minimum lease payments	$ 701

Rent expense for the three months ended September 30, 2016 and 2015 was $0.2 million and $0.2 million, respectively. Rent expense for the nine months ended September 30, 2016 and 2015 was $0.7 million and $0.6 million, respectively.

8. Convertible Preferred Stock

On March 13, 2015, the Company s board of directors authorized the Company to increase the authorized number of shares of Series B Preferred Stock to 56,026,590 in connection with an anticipated Series B Preferred Stock financing. The Company subsequently issued 13,062,965 shares of Series B Preferred Stock at $0.9503 per share, and received net proceeds of $12.3 million.

Prior to the IPO, the holders of the Preferred Stock had certain voting and dividend rights, as well as liquidation preferences and conversion privileges. All rights, preferences, and privileges associated with the Preferred Stock were terminated at the time of the Company s IPO in conjunction with the conversion of all outstanding shares of Preferred Stock into shares of Common Stock.

Upon the closing of the Company s IPO on June 30, 2015, all outstanding shares of the Preferred Stock were automatically converted into 9,029,549 shares of Common Stock. As of September 30, 2016, 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.

9. Common Stock Reserved for Future Issuance

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

	September 30, 2016	December 31, 2015
Warrants for the purchase of Common Stock	24,566	59,405
Options to purchase Common Stock	3,118,255	2,557,456
Employee Stock Purchase Plan	335,484	182,352
Total	3,478,305	2,799,213

10. 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.

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The 2015 Plan provides for the grant of incentive stock options, non-statutory stock options, restricted stock awards, restricted stock units, stock appreciation rights and other stock-based awards. The Company s employees, officers, directors and consultants and advisors are eligible to receive awards under the 2015 Plan. The maximum number of shares of Common Stock that may be delivered in satisfaction of awards under the 2015 Plan is 1,068,287 shares, plus (1) 25,942 shares that were available for grant under the 2008 Plan immediately prior to the closing of the IPO, (2) the number of shares of Common Stock subject to outstanding awards under the 2008 Plan upon closing of the IPO that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by the Company at their original issuance price pursuant to a contractual repurchase right and (3) an annual increase, to be added the first day of each fiscal year, beginning with the fiscal year ending December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2025, equal to the lowest of 1,297,334 shares of Common Stock, 4% of the number of shares of Common Stock outstanding on the first day of the fiscal year and an amount determined by the Company s board of directors. The January 1, 2016 increase added 612,531 authorized shares to the 2015 Plan.

As of September 30, 2016, the Company had reserved 1,230,321 shares of Common Stock under the 2008 Plan, of which none remained available for future issuance. As of September 30, 2016, the Company had reserved 1,887,934 shares of Common Stock under the 2015 Plan, of which 631,846 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, 2016, 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, 2015	1,723,554	$ 6.66	7.92	$ 4,267
Granted	1,043,045	4.47		
Exercised	(51,732)	1.98		
Cancelled or forfeited	(228,458)	6.59		
Outstanding at September 30, 2016	2,486,409	$ 5.84	8.14	$ 4,037
Exercisable at September 30, 2016	978,785	$ 4.93	6.53	$ 2,327
Vested or expected to vest at September 30, 2016	2,378,562	$ 5.83	8.09	$ 3,902

The total intrinsic value of options exercised for the three months ended September 30, 2016 and 2015 was $30,000 and $0, respectively. The total intrinsic value of options exercised for the nine months ended September 30, 2016 and 2015 was $0.2 million and $0.2 million, respectively. The total fair value of employee and non-employee options vested for the three months ended September 30, 2016 and 2015 was $0.8 million and $0.3 million, respectively. The total fair value of employee and non-employee options vested for the nine months ended September 30, 2016 and 2015 was $1.9 million and $1.1 million, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the three months ended September 30, 2016 and 2015 was $2.84 and $9.13, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the nine months ended September 30, 2016 and 2015 was $2.88 and $7.77, respectively.

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At September 30, 2016, the total unrecognized compensation expense related to unvested stock option awards, including estimated forfeitures, was $5.4 million. The Company expects to recognize that cost over a weighted-average period of approximately 2.7 years.

Employee Stock Purchase Plan

In June 2015, the Company s board of directors adopted and the Company s stockholders approved the 2015 Employee Stock Purchase Plan (the 2015 ESPP) which became effective upon closing of the IPO. The 2015 ESPP initially authorizes the issuance of up to a total of 182,352 shares of Common Stock to participating eligible employees. The number of authorized shares increases each January 1, commencing on January 1, 2016 and ending on December 31, 2026, by an amount equal to the lesser of one percent of the Company s outstanding shares as of the first day of the applicable year, 364,705 shares and any lower amount determined by the Company s board of directors. The January 1, 2016 increase added 153,132 authorized shares to the plan. As of September 30, 2016, there had been no shares issued under the 2015 ESPP.

11. 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. There were no material recognized subsequent events recorded in the condensed consolidated financial statements as of and for the three and nine months ended September 30, 2016.

Operating Lease

On November 3, 2016, the Company entered into a Third Amendment of Lease (the Third Lease Amendment) with DWF IV One Kendall, LLC (the Landlord), which amended certain terms of the Company s existing lease with the Landlord. The Third Lease Amendment extended the term of the lease through June 30, 2018, and will increase the future minimum payments described in Note 7 from approximately $0.7 million to approximately $1.5 million.

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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 focused on the discovery, development and commercialization of novel therapeutics based on our proprietary Safely Metabolized And Rationally Targeted, or SMART, linker drug discovery platform. Our SMART linker drug discovery platform enables us to engineer product candidates that can simultaneously modulate multiple targets in a disease. Our proprietary product candidates impact pathways that are central to diseases where efficacy may be optimized by a multiple target approach. Our primary focus is on treatments for rare diseases. We have applied our SMART linker drug discovery platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates.

Our lead product candidate is edasalonexent, formerly known as CAT-1004, an oral small molecule. Based on its mechanism of action, the inhibition of NF- κB, or nuclear factor kappa-light-chain-enhancer of activated B cells, we believe it has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy, or DMD, regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. We are currently conducting the MoveDMD® Phase 1/2 trial of edasalonexent in boys with DMD between ages four and seven. The MoveDMD trial is a three-part clinical trial investigating the safety and efficacy of edasalonexent in DMD. We have reported positive results from Part A of the MoveDMD trial. We initiated Part B of the MoveDMD trial in April 2016 and initiated an open label extension, Part C, in July 2016. We completed enrollment for Part B at 31 patients in October 2016 and expect to report top-line Part B data in the first half of the first quarter of 2017, contingent on trial conduct. If the results from our MoveDMD clinical trial are positive and discussions with regulatory authorities regarding a pivotal trial are supportive, we intend to initiate a six-month Phase 3 placebo-controlled pivotal clinical trial of edasalonexent in ambulatory boys with DMD aged 4 to 7 and an additional clinical trial in non-ambulatory boys with DMD in 2017. 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. In addition to our work in DMD, we are considering other diseases where the inhibition of NF-κB may be beneficial as potential targets for further therapeutic applications of edasalonexent. There are a number of other rare diseases where NF-κB plays an important role, such as IgA Nephropathy and Becker Muscular Dystrophy.

We are developing a pipeline of product candidates using our SMART linker drug discovery platform as potential treatments for rare diseases including amyotrophic lateral sclerosis, or ALS, Friedreich s ataxia, or FA, and cystic fibrosis, or CF. Our pipeline includes CAT-4001 and CAT-5571, for which we are conducting preclinical activities. We are developing CAT-4001 as a potential treatment for neurodegenerative diseases such as FA and ALS, irrespective of mutation status. CAT-4001 is a small molecule that activates Nuclear factor (erythroid-derived 2)-like 2, or Nrf2, and inhibits NF-κB, two pathways that have been implicated in FA and ALS. In addition, we are developing CAT-5571 initially as a potential oral treatment for CF, with potential beneficial effects on both cystic fibrosis transmembrane conductance regulator, or CFTR, trafficking and function and on the clearance of

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Pseudomonas aeruginosa. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, which is known to be impaired in CF.

We also applied our SMART linker drug discovery platform to engineer our CAT-2000 series product candidates to inhibit the Sterol Regulatory Element Binding Protein, or SREBP, pathway. Inhibitors of SREBP have been proposed for the treatment of hyperlipidemias and nonalcoholic steatohepatitis, or NASH, based on the role of SREBP in lipid metabolism and known human polymorphisms associated with NASH disease progression. We have advanced two CAT-2000 molecules, CAT-2003 and CAT-2054, into clinical development and intend to pursue a partnership for further development of the CAT-2000 series in NASH.

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, 2016, we have raised an aggregate of $185.4 million, of which $92.9 million was from private placements of preferred stock, $69.0 million represented gross proceeds from our IPO, $11.5 million represented gross proceeds from our September 2016 registered direct offering, $10.0 million was from a secured debt financing, $1.6 million represented gross proceeds from our at-the-market, or ATM, offering program, and $0.4 million was from common stock option and warrant exercises.

In June 2015, we completed our IPO, in which we sold an aggregate of 5,750,000 shares of our common stock, including 750,000 shares of common stock sold pursuant to the underwriters exercise of their option to purchase additional shares of common stock, at a price to the public of $12.00 per share. Net proceeds from the IPO were $61.7 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $7.3 million.

In connection with our IPO, all shares of our preferred stock were automatically converted into an aggregate of 9,029,549 shares of our common stock and our outstanding warrants to purchase 315,688 shares of preferred stock were automatically converted into warrants to purchase 24,566 shares of common stock with an exercise price of $12.2114 per share.

In August 2016, we commenced a $10.0 million ATM offering program. During the three months ended September 30, 2016, we sold an aggregate of 368,015 shares of common stock pursuant to the ATM offering program, at an average price of $4.35 per share for gross proceeds of $1.6 million, resulting in net proceeds of $1.3 million after deducting sales commissions and offering expenses of approximately $0.3 million in the aggregate. On September 22, 2016, we reduced the amount of common stock that we were offering under the ATM program, such that we were only offering $1.4 million of common stock in addition to the $1.6 million of common stock we had sold as of that date. As of September 30, 2016, $1.4 million of common stock remained available for sale under the ATM program. On November 10, 2016, we increased the amount of common stock being offered pursuant to the sales agreement, such that we are offering an additional $8.4 million of common stock for sale under the sales agreement from and after such date.

In September 2016, we closed an underwritten registered direct offering, in which we sold 2,875,000 shares of our common stock (including 375,000 shares of common stock sold pursuant to the exercise of an option by the underwriter to purchase additional shares) at an offering price

of $4.00 per share. We received aggregate gross proceeds from the offering of $11.5 million, resulting in net proceeds of $10.6 million after deducting underwriting discounts and commissions and offering expenses of approximately $0.9 million in the aggregate.

We have not generated any revenue to date. We have incurred significant annual net operating losses in every year since our inception and expect to incur a net operating loss in 2016 and continue to incur net operating losses for the foreseeable future. As of September 30, 2016, we had an accumulated deficit of $135.3 million. We expect to continue to incur significant expenses and increasing operating losses for the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly if and as we continue to develop and conduct clinical trials with respect to edasalonexent and other product candidates; initiate and continue research, preclinical and clinical development efforts for

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our other product candidates and potential product candidates; maintain, expand and protect our intellectual property portfolio; establish a commercial infrastructure to support the marketing and sale of certain of our product candidates; hire additional personnel, such as clinical, regulatory, quality control and scientific personnel; and operate as a public company.

Financial Overview

Revenue

To date, we have not generated any revenue from product sales or any other source and do not expect to generate any revenue from the sale of products in the near future. In the future, we will seek to generate 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, or CROs, 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

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, a protein that is activated in DMD and that drives inflammation, fibrosis and muscle degeneration, and suppresses muscle regeneration. We have reported positive results from Part A of the MoveDMD trial, and results indicated that all three doses of edasalonexent studied were generally well tolerated with no safety signals observed. For the 67 mg/kg/day and 100 mg/kg/day dosing levels, pharmacokinetic results demonstrated edasalonexent plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-κB was observed. We reported top-line results for positive NF-κB biomarker data that demonstrated NF-κB target engagement via statistically significant reduction in NF-κB controlled gene expression for the 67 mg/kg/day and 100 mg/kg/day dosing levels. These are the doses we have advanced to Part B of the trial.

In April 2016, we initiated Part B of the MoveDMD trial, a 12 week, randomized, double-blind placebo-controlled trial, and, in July 2016, we initiated an open label extension, Part C, which is expected to provide additional safety and efficacy data on edasalonexent when administered for up to 48 weeks. We completed enrollment for Part B in October 2016 and expect to report top-line Part B data in the first half of the first quarter of 2017, contingent on trial conduct. Our primary endpoint for Part B is an assessment of the effects of edasalonexent on muscle inflammation as measured by magnetic resonance imaging. If the results from our MoveDMD clinical trial are positive and discussions with regulatory authorities regarding a pivotal trial are supportive, we intend to initiate a six-month Phase 3 placebo-controlled pivotal clinical trial of edasalonexent in ambulatory boys with DMD aged 4 to 7 and an additional clinical trial in non-ambulatory boys with DMD in 2017. If the results from

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the Phase 3 clinical trial are positive, we intend to seek marketing approval for edasalonexent. We hold rights to edasalonexent throughout the world.

In September 2016, we announced a pre-clinical joint research collaboration with Sarepta Therapeutics, Inc., a commercial stage developer of RNA targeted therapeutics, to explore a combination drug treatment approach for DMD.

CAT-4001

CAT-4001 is a conjugate that we designed to combine the potentially beneficial activities of monomethyl fumarate and DHA on the Nrf2 and NF-κB pathways. Nrf2 is a protein that works inside cells to control the expression of genes that controls the body s response to cellular stress and oxidative damage. We are developing CAT-4001 initially for the treatment of neurodegenerative diseases in which the Nrf2 and NF-κB pathways have been implicated, such as FA and ALS, irrespective of mutation status. We are conducting preclinical activities with CAT-4001. We have been granted U.S. patents covering CAT-4001 and related compounds and their use and we have pending similar patent applications in various countries in North America, South America, Europe, and Asia.

CAT-5571

CAT-5571 is a SMART linker conjugate that contains cysteamine and DHA. We are developing CAT-5571 initially as a potential oral treatment for CF with potential effects on both the CFTR and on the bacterial clearance of *Pseudomonas aeruginosa*. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, which is known to be impaired in CF. We have shown in ex vivo preclinical studies that CAT-5571, in combination with lumacaftor/ivacaftor, enhances cell-surface trafficking and function of CFTR with the F508del mutation, which is the most frequent CFTR mutation and is present in more than 85% of patients included in the Cystic Fibrosis Foundation United States Patient Registry. We have also shown that CAT-5571 enhances the clearance of *Pseudomonas aeruginosa* infection in preclinical models of CF, irrespective of CFTR mutation status. We are conducting preclinical activities with CAT-5571. We have a pending international patent application with claims covering CAT-5571 and related compounds and their use.

CAT-2000 Series

Our CAT-2000 compounds are SMART linker conjugates of nicotinic acid and the long chain fatty acid, eicosapentaenoic acid, or EPA. The linkers for our CAT-2000 series compounds are cleaved through intracellular enzymatic hydrolysis, to release the component bioactives to inhibit SREBP. By using different linkers, we have produced product candidates within the CAT-2000 series that possess different hydrolysis rates, resulting in distinct pharmacokinetics, biodistribution and pharmacology. We have been able to demonstrate enzymatic hydrolysis and inhibition of SREBP in in vitro studies with CAT-2000 molecules. In addition, in vivo, CAT-2000 molecules have demonstrated efficacy in

multiple preclinical models of hyperlipidemias and NASH. We believe that the portfolio of CAT-2000 molecules, which includes the clinical-stage molecules CAT-2003 and CAT-2054 and other discovery-stage molecules with intermediate rates of hydrolysis, provide an opportunity to develop a therapy for NASH We intend to pursue a partnership for further development of the CAT-2000 series in NASH. We hold rights to the CAT-2000 series throughout the world.

Other Programs

Other research and development programs include activities related to pathway biology validation and SMART linker conjugate design and optimization. Our focus in these efforts is on rare diseases.

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):

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	Nine Months Ended September 30,	
	2016	2015
Edasalonexent	$ 6,481	$ 4,200
CAT-2054	3,448	3,354
CAT-2003	158	927
Other research and platform programs	2,278	1,654
Costs not directly allocated to programs:		
Employee expenses including cash compensation, benefits and stock-based compensation	4,742	4,651
Facilities	708	611
Consultants and professional expenses, including stock-based compensation	805	609
Other	570	354
Total costs not directly allocated to programs	6,825	6,225
Total research and development expenses	$ 19,190	$ 16,360

Since inception, the total direct expenses to support the edasalonexent program have been $21.0 million. Since we began separately tracking CAT-2054 in 2013, the direct expenses to support that program have totaled $12.7 million. Since inception, the total direct expenses to support the CAT-2003 program have been $15.5 million.

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 the numerous risks and uncertainties associated with developing medicines, including the uncertainties of:

- establishing an appropriate safety profile with investigational new drug application-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 research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, 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

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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 our general and administrative expenses will increase in the future to support our continued operations, potential commercialization of our product candidates and costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company including expenses related to services associated with maintaining compliance with exchange listing and Securities and Exchange Commission, or SEC, requirements, insurance costs and investor relations costs.

Other (Expense) Income

Other (expense) income, net consists of interest expense incurred on debt instruments, amortized deferred financing costs and amortized debt discount, net amortization expense on available-for-sale securities and changes in the fair value of warrant liability, as offset by any interest income earned on our cash and cash equivalents. Upon completion of our IPO in June 2015, warrants to purchase preferred stock were converted to warrants to purchase common stock and as a result, we no longer record a fair value adjustment for warrants.

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, 2016, there were no material changes to our critical accounting policies as reported in our Annual Report on Form 10-K for the year ended December 31, 2015, which was filed with the SEC on March 15, 2016, which we refer to as our 2015 Annual Report on Form 10-K.

Results of Operations

Comparison of the Three Months Ended September 30, 2016 and 2015

The following table summarizes our results of operations for the three months ended September 30, 2016 and 2015, together with the dollar change in those items (in thousands):

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	Three Months Ended September 30, 2016	2015	Period-to-Period Change
Operating expenses:			
Research and development	$ 5,936	$ 5,813	$ 123
General and administrative	2,347	2,388	(41)
Total operating expenses	8,283	8,201	82
Loss from operations	(8,283)	(8,201)	(82)
Other expense, net	(136)	(284)	148
Net loss	$ (8,419)	$ (8,485)	$ 66

Research and Development Expenses

Research and development expenses increased by $0.1 million to $5.9 million for the three months ended September 30, 2016 from $5.8 million for the three months ended September 30, 2015, an increase of 2%. The increase in research and development expenses was attributable to a net increase of $0.1 million in direct program costs, which included a $1.5 million increase in costs to support our edasalonexent program that was largely offset by decreases in other programs.

General and Administrative Expenses

General and administrative expenses decreased by $0.1 million to $2.3 million for three months ended September 30, 2016 from $2.4 million for the three months ended September 30, 2015, a decrease of 4%. The decrease in general and administrative expenses was attributable to decreases in employee compensation and the general and administrative share of facilities expense, largely offset by increases in professional fees and Delaware franchise tax.

Other Expense, Net

Other expense net decreased by $0.2 million to $0.1 million for the three months ended September 30, 2016 from $0.3 million for the three months ended September 30, 2015, primarily due to $0.1 million in interest income and a $0.1 million decrease in interest expense due to the principal payments made on our credit facility.

Comparison of the Nine Months Ended September 30, 2016 and 2015

The following table summarizes our results of operations for the nine months ended September 30, 2016 and 2015, together with the dollar change in those items (in thousands):

	Nine Months Ended September 30,	Period-to-

	2016	2015	Period Change
Operating expenses:			
Research and development	$ 19,190	$ 16,360	$ 2,830
General and administrative	7,695	5,966	1,729
Total operating expenses	26,885	22,326	4,559
Loss from operations	(26,885)	(22,326)	(4,559)
Other expense, net	(397)	(698)	301
Net loss	$ (27,282)	$ (23,024)	$ (4,258)

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Research and Development Expenses

Research and development expenses increased by $2.8 million to $19.2 million for the nine months ended September 30, 2016 from $16.4 million for the nine months ended September 30, 2015, an increase of 17%. The increase in research and development expenses was primarily attributable to a net increase of $2.2 million in direct program costs, reflecting an increase of $2.3 million in costs related to edasalonexent, primarily related to the MoveDMD Phase 1/2 clinical trial, and a net decrease of $0.1 million in costs related to our other programs. In addition, costs related to internal research and development increased by $0.6 million, primarily related to employee and consultant compensation costs.

General and Administrative Expenses

General and administrative expenses increased by $1.7 million to $7.7 million for nine months ended September 30, 2016 from $6 million for the nine months ended September 30, 2015, an increase of 28%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $0.9 million primarily associated with salaries, benefits, and stock-based compensation expenses for new hires; increased consulting and professional fees of $0.5 million, driven by the costs of becoming and operating as a public company; increased insurance expense of $0.2 million due to our public company directors and officers insurance policy; and increased general office expense of $0.1 million.

Other Expense, Net

Other expense, net decreased by $0.3 million to $0.4 million for the nine months ended September 30, 2016 from $0.7 million for the nine months ended September 30, 2015. The decrease in other expense, net was primarily due to $0.2 million in interest and investment income, gains of $0.1 million from insurance settlements relating to water damage in our offices and a decrease in interest expense due to the amortization of principal on our credit facility.

Liquidity and Capital Resources

From our inception through September 30, 2016, we raised an aggregate of $185.4 million, of which $92.9 million was from private placements of preferred stock, $69.0 million represented gross proceeds from our IPO, $11.5 million represented gross proceeds from our registered direct common stock offering, $10.0 million was from a secured debt financing, $1.6 million represented gross proceeds from our ATM offering program, and $0.4 million was from common stock option and warrant exercises. As of September 30, 2016, we had $26.5 million in cash and cash equivalents and $20.8 million in available-for-sale securities.

Initial Public Offering

In June 2015, we completed the sale of an aggregate of 5,750,000 shares of our common stock, including 750,000 shares of common stock sold pursuant to the underwriters exercise of their option to purchase additional shares of common stock, in our IPO, at a price to the public of $12.00 per share. Net proceeds from the IPO were $61.7 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $7.3 million.

At-the-Market Offering

In August 2016, we entered into a sales agreement with Cowen and Company LLC, or Cowen, pursuant to which we may issue and sell shares of our common stock for an aggregate maximum offering amount of $10.0 million under an ATM offering program. Cowen is not required to sell any specific amount, but acts as our sales agent using commercially reasonable efforts consistent with its normal trading and sales practices. Shares sold pursuant to the sales agreement have been sold pursuant to a shelf registration statement, which became effective on July 19, 2016, or the Shelf Registration Statement. We pay Cowen 3% of the gross proceeds from any common stock sold through the sales agreement.

During the three months ended September 30, 2016, we sold an aggregate of 368,015 shares of common stock pursuant to the ATM program, at an average price of $4.35 per share, for gross proceeds of $1.6 million, resulting in net proceeds of $1.3 million

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after deducting sales commissions and offering expenses of approximately $0.3 million in the aggregate. On September 22, 2016, we reduced the amount of common stock that we were offering pursuant to the sales agreement, such that we were only offering $1.4 million of common stock in addition to the $1.6 million of common stock we had sold under the ATM program as of that date. As of September 30, 2016, $1.4 million of common stock remained available for sale under the ATM program. On November 10, 2016, we increased the amount of common stock being offered pursuant to the sales agreement, such that we are offering an additional $8.4 million of common stock for sale under the sales agreement from and after such date.

Registered Direct Offering

In September 2016, we closed an underwritten registered direct offering, in which we sold 2,875,000 shares of our common stock (including 375,000 shares of common stock sold pursuant to the exercise of an option by the underwriter to purchase additional shares) at an offering price of $4.00 per share. The shares sold in the offering were sold pursuant to the Shelf Registration Statement. We received aggregate gross proceeds from the offering of $11.5 million, resulting in net proceeds of $10.6 million after deducting underwriting discounts and commissions and offering expenses of approximately $0.9 million in the aggregate.

Credit Facility

On August 27, 2014, we entered into a loan and security agreement with MidCap Financial Trust, Flexpoint MCLS Holdings, LLC and Square 1 Bank, or the Credit Facility. In March and December 2015, we entered into amendments to the Credit Facility, or the March 2015 Amendment and the December 2015 Amendment, respectively. As amended, the Credit Facility provided for initial borrowings of $5.0 million and additional borrowings of up to $20.0 million. Concurrently with entering into the Credit Facility in August 2014, we borrowed $5.0 million under a term loan under the Credit Facility and we issued to the lenders warrants to purchase an aggregate of 157,844 shares of our series B preferred stock at an exercise price of $0.9503 per share. Concurrently with the March 2015 Amendment, we drew down an additional $5.0 million under our term loan under the Credit Facility and we issued to the lenders warrants to purchase an aggregate of 157,844 shares of our series B preferred stock at an exercise price of $0.9503 per share. The remaining amounts available for borrowing under this arrangement expired unused as of July 31, 2015. All borrowings under the Credit Facility are due on October 1, 2018 and are collateralized by substantially all of our personal property, other than our intellectual property. The December 2015 Amendment revised terms to allow for the creation of a wholly owned subsidiary entity.

There are no financial covenants associated with the Credit Facility; however, there are negative covenants that prohibit us from transferring any of our material assets except to our subsidiary, exclusively licensing our intellectual property (subject to certain exceptions), merging with or acquiring another entity, entering into a transaction that would result in a change of control, incurring additional indebtedness, creating any lien on our property, making investments in third parties or redeeming stock or paying dividends.

The Credit Facility also includes events of default, the occurrence and continuation of any of which provides the lenders the right to exercise remedies against us and the collateral securing the loans under the Credit Facility, including cash. These events of default include, among other things, failure to pay amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, which includes a material adverse change in our business, operations or conditions (financial or otherwise) or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against us in an amount greater than $250,000. The occurrence of a material adverse event could result in acceleration of payment of the debt. At September 30, 2016 and December 31, 2015, we concluded that the likelihood of the acceleration of the debt was remote, as a material adverse event had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments.

We were obligated to make monthly interest-only payments on any term loans borrowed under the Credit Facility until September 1, 2015 and we are obligated to pay 36 consecutive, equal monthly installments of principal and interest from October 1, 2015 through September 1, 2018. Term loans under the Credit Facility bear interest at an annual rate of 7.49%. Following the occurrence and during the continuance of an event of default, borrowings under the Credit Facility will bear interest at an annual rate that is 5.00% above the rate that is otherwise applicable. In addition, a final payment equal to 3.48% of any amounts drawn under the Credit Facility is due upon the earlier of the maturity date, acceleration of the term loans or prepayment of all or part of the term loans.

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Preferred Stock Financing

In March 2015, we raised $12.4 million in gross proceeds from the sale of 13,062,965 shares of our series B preferred stock at a price per share of $0.9503.

Cash Flows

Comparison of the Nine Months Ended September 30, 2016 and 2015

The following table provides information regarding our cash flows for the nine months ended September 30, 2016 and 2015 (in thousands):

	Nine Months Ended September 30,	
	2016	2015
Net cash used in operating activities	$ (24,874)	$ (21,050)
Net cash used in investing activities	(21,300)	(60)
Net cash provided by financing activities	9,858	79,151
Net (decrease) increase in cash and cash equivalents	$ (36,316)	$ 58,041

Net Cash Used in Operating Activities

Net cash used in operating activities was $24.9 million for the nine months ended September 30, 2016 and consisted primarily of a net loss of $27.3 million adjusted for non-cash items, including stock-based compensation expense of $1.6 million, depreciation and amortization expense of $0.3 million, non-cash interest expense of $0.2 million, accretion of discount/premium on investment securities of $0.1 million, and a net decrease in operating assets of $0.2 million, which resulted primarily from an increase in accounts payable and accrued expenses.

Net cash used in operating activities was $21.1 million for the nine months ended September 30, 2015 and consisted primarily of a net loss of $23.0 million adjusted for non-cash items (including stock-based compensation expense of $1.1 million, non-cash interest expense of $0.2 million and depreciation and amortization expense of $0.1 million), along with an increase in accounts payable of $0.6 million and an increase in accrued expenses of $0.1 million, partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $21.3 million during the nine months ended September 30, 2016, which was primarily attributable to net purchases of available-for-sale securities of $20.9 million and $0.4 million in net laboratory equipment purchases. Net cash used in investing activities was $0.1 million during the nine months ended September 30, 2015, which was attributable to laboratory equipment purchases.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $9.9 million during the nine months ended September 30, 2016 compared to $79.2 million during the nine months ended September 30, 2015. The cash provided by financing activities in the nine months ended September 30, 2016 was attributable to net proceeds of $10.7 million from the registered direct offering, net proceeds of $1.5 million from our ATM offering, and $0.2 million from exercises of common stock options and warrants. These proceeds were partially offset by $2.5 million in repayment of principal on the Credit Facility. The cash provided by financing activities for the nine months ended September 30, 2015 primarily consisted of net proceeds received from our IPO of $61.8 million, net proceeds of $12.3 million from

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the issuance of 13,062,965 shares of our series B preferred stock in March 2015, and $5.0 million from our borrowings under the Credit Facility in March 2015.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, and conduct clinical trials and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that our cash, cash equivalents and marketable securities at September 30, 2016 will enable us to fund our operating expenses and capital expenditure requirements through at least September 30, 2017. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of our current and potential product candidates, and because the extent to which we may enter into collaborations with third parties for the development of these product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

- the scope, progress, results and costs of drug discovery, preclinical development, laboratory testing and clinical trials for our product candidates;
- the success of any future collaborations;
- the extent to which we acquire or in-license other medicines and technologies;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- our ability to establish and maintain collaborations on favorable terms, if at all.

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

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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 SEC rules.

Contractual Obligations

On November 3, 2016, we entered into a Third Amendment of Lease, or the Third Lease Amendment, with DWF IV One Kendall, LLC, or the Landlord, which amended certain terms of our existing lease with the Landlord. The Third Lease Amendment extended the term of the lease through June 30, 2018, and will increase the future minimum payments for our headquarters under the lease following September 30, 2016 from approximately $0.7 million to approximately $1.5 million.

There were no other material changes to our contractual obligations and commitments described under Management s Discussion and Analysis of Financial Condition and Results of Operations in the 2015 Annual Report on Form 10-K.

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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, 2016, we had cash and cash equivalents and available-for-sale securities of $47.3 million and, as of December 31, 2015, we had cash and cash equivalents of $62.8 million. Our cash and cash equivalents at each date consisted primarily of money market funds and our available-for-sale securities at September 30, 2016 consisted primarily of corporate debt securities and U.S. government-sponsored securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in the general level of U.S. interest rates. Our available-for-sale securities are subject to interest rate risk and could fall in value if market interest rates increase. 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 the ability to hold our available-for-sale securities until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

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.

As of September 30, 2016, 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. 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, 2016, 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, 2016, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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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 have incurred significant losses since inception and expect to incur significant and increasing 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 and increasing operating losses for at least the next several years. Our net losses were $32.6 million, $21.9 million and $18.1 million for the years ended December 31, 2015, 2014 and 2013, respectively, and $27.3 million for the nine months ended September 30, 2016. As of September 30, 2016, we had an accumulated deficit of $135.3 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, as well as 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 as we:

- continue to develop and conduct clinical trials with respect to our product candidates, including the current MoveDMD Phase 1/2 clinical trial and contemplated future clinical trials of edasalonexent for the treatment of Duchenne muscular dystrophy, or DMD, including any potential Phase 3 trial;

- initiate and continue research and preclinical and clinical development efforts for our other product candidates;

- seek to identify and develop additional product candidates;

- potentially in-license third party 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

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- 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 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 collaborators 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 you to lose all or part of your investment.

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 early-stage 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, you 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.

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 expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and preclinical development efforts for and seek marketing approval for, our product candidates. 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. 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 substantial additional funding in connection with our continuing operations. 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.

We will be required to expend significant funds in order to advance the development of edasalonexent, as well as our other product candidates. In addition, while we may seek one or more collaborators for future development of our product candidates, we may not be able to enter into a collaboration for any of our product candidates on suitable terms or at all. In any event, our existing cash and cash equivalents 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 further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources to fund the completion of development of any of our product candidates. We do not have any committed external source of funds.

Adequate additional financing may not be available to us on acceptable terms, or at all. Further, our ability to obtain additional debt financing may be limited by covenants we have made under our loan and security agreement with MidCap Financial Trust, or MidCap, Flexpoint MCLS SPV LLC, or Flexpoint, and Square 1 Bank, or Square 1, including our negative pledge with respect to intellectual property in favor of Flexpoint and Square 1, as well as our pledge to MidCap, Flexpoint and Square 1 of substantially all of our assets, other than our intellectual property, as collateral. Our failure to raise capital as and when needed would have a negative impact on our financial condition and our ability to pursue our business strategy.

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We believe that our existing cash, cash equivalents and marketable securities as of September 30, 2016 will enable us to fund our operating expenses, debt service and capital expenditure requirements through at least September 30, 2017. Our estimate as to how long we expect our cash and cash equivalents 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, both short-term and long-term, will depend on many factors, including:

- 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 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.

We expect that 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. 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. For example, our credit facility with MidCap, Flexpoint and Square 1 contains restrictive covenants that, among other things and subject to certain exceptions, prohibit us from transferring any of our material assets, exclusively licensing our intellectual property (subject to certain exceptions), merging with or acquiring another entity, entering into a transaction that would result in a change of control, incurring additional indebtedness, creating any lien on our property, making investments in third parties or redeeming stock or paying dividends. Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. 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.

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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.

Our existing and any future indebtedness could adversely affect our ability to operate our business.

As of September 30, 2016, we had $6.7 million of outstanding borrowings under our credit facility with MidCap, Flexpoint and Square 1. We are required to repay principal and interest on these borrowings in monthly installments through October 2018. Subject to the restrictions in this existing credit facility, we could in the future incur additional indebtedness beyond our borrowings from MidCap, Flexpoint and Square 1.

Our outstanding indebtedness, including any additional indebtedness beyond our borrowings from MidCap, Flexpoint and Square 1, 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.

We intend to satisfy our current and future debt service obligations with our existing cash and cash equivalents. However, we may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing debt instruments. Failure to

make payments or comply with other covenants under our existing debt instruments could result in an event of default and acceleration of amounts due. Under our loan and security agreement with MidCap, Flexpoint and Square 1, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lenders accelerate the amounts due, we may not be able to make accelerated payments, and the lenders could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all of our assets other than our intellectual property. In addition, the covenants under our credit facility, the pledge of our assets as collateral and the negative pledge with respect to our intellectual property could limit our ability to obtain additional debt financing.

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 linker 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 therapeutics 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 later stage clinical trials or in obtaining marketing approval thereafter. For example, although we have discovered and evaluated numerous compounds using our SMART linker drug discovery platform, we have not yet advanced a compound into Phase 3 clinical development and no product created using the SMART linker drug discovery platform has ever been approved for sale.

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We currently only have one clinical-stage product, edasalonexent, that we are actively developing and we are dependent on its success. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize edasalonexent, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently have no products approved for sale and are investing a significant portion 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.

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

- successful completion of our ongoing Phase 1/2 clinical trial;
- initiation and successful enrollment and completion of additional clinical trials;
- 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 scope of any future collaborations and 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 could 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 are conducting 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

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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;

- 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 application is insufficient to obtain marketing approval of our product candidates. If the FDA does not accept or approve our NDAs for either of our most advanced product candidates, 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, in a Phase 1 clinical trial for CAT-2054, we observed decreases in LDL-C of up to 20%, which were statistically significant compared to baseline for all dose levels, after 14 days of dosing and seven days of follow-up, which were not borne out in a Phase 2a clinical trial of CAT-2054 in patients with hypercholesterolemia on high intensity statins,

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where we did not see reductions in LDL-C. 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 process for product candidates that target rare diseases, including DMD, Friedreich s ataxia and amyotrophic lateral sclerosis, is uncertain.

Due to the lack of extensive 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 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, or INDs, and NDAs for our product candidates, in a timely manner, or at all. For example, there is currently only one approved therapy for DMD in the United States. Sarepta Therapeutics drug eteplirsen was approved by the FDA for the treatment of DMD under the accelerated approval pathway in September 2016. Outside of the United States, PTC Therapeutics drug ataluren has been approved within the European Union Member States, Iceland, Liechtenstein, Norway, Israel and South Korea for the treatment of nonsense mutation DMD. Other than these two drugs, there has been limited historical clinical trial experience for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, is subject to increased risk. In particular, regulatory authorities in the United States have not issued definitive guidance as to how to measure and demonstrate efficacy. We may experience difficulties in seeking approval of edasalonexent for the treatment of DMD, and 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. The regulatory approval process is also uncertain for other rare diseases with only one or no approved therapy in the United States, such as amyotrophic lateral sclerosis and Friedreich s ataxia. The design and conduct of clinical trials for rare diseases with such limited historical clinical trial guidance, likewise, is subject to increased risk due to the inherent uncertainty from the extremely limited or completely lacking regulatory approval process precedent. 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. 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

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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. For example, our IND for CAT-2003 was placed on partial clinical hold by the FDA in November 2012 because of the need for additional nonclinical work to support potential expansion of dosing and duration of our proposed Phase 1 multiple ascending dose trial. Although the partial clinical hold was removed in July 2013, it is possible that any of our development programs may be placed on full or partial clinical hold by regulatory authorities at any point, which would delay and possibly prevent further development of our product candidates. 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. For example, in a Phase 2 clinical trial with CAT-2054, we did not see reductions in LDL-C that we observed in a Phase 1 clinical trial, causing us to deprioritize or stop further development of this molecule for hypercholesterolemia. 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 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 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.

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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 our clinical trials of CAT-2003 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 Phase 2 clinical trials for CAT-2003, in which we observed gastrointestinal adverse events at clinically meaningful doses, and CAT-2054, in which we did not see reductions in LDL-C, the primary end point of the trial, causing us to deprioritize or stop further development of these molecules for the diseases we were targeting;

- 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;

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- 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, such as the delay we experienced in one of our Phase 2 clinical trials of CAT-2003 while we reformulated CAT-2003 in a coated capsule and evaluated its tolerability;

- 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 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;

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- 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 or to complete our clinical trials for edasalonexent in a timely and cost-effective manner.

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, we anticipate that a Phase 3 clinical trial of edasalonexent would enroll only ambulatory boys between ages four and seven who have not used steroids for at least six months prior to the trial. Similarly, if we conduct an additional trial of edasalonexent for non-ambulatory boys, we would intend to enroll non-ambulatory boys in an older age range who have not used steroids for at least six months prior to the trial. These inclusion criteria could present challenges to enrollment because steroid therapy for DMD is often initiated or continued in each of the age ranges that we expect to use for our future trials.

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;

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- 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;

- 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

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- 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.

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 intend to collaborate with third parties for commercialization of some of our product candidates in one or more geographies 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 indications of our most advanced programs, including DMD.

We are initially developing edasalonexent for the treatment of DMD. The FDA has approved Sarepta Therapeutics drug eteplirsen for the treatment of DMD in the United States and corticosteroid therapy is often prescribed to treat the inflammation underlying DMD and to delay loss of ambulation. In addition, a number of companies are developing other therapies to treat DMD, one of which is already on the market in Europe and others are in the process of registration or late stage clinical development, including Marathon Pharmaceuticals, PTC Therapeutics, Santhera Pharmaceuticals and Sarepta Therapeutics.

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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.

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

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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 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;

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- 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. Likely collaborators may include large and mid-size pharmaceutical, biotechnology and health care 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, our loan and security agreement with MidCap, Flexpoint and Square 1 contains, and any 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.

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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;

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