CATABASIS PHARMACEUTICALS INC
Form 10-Q
May 11, 2017
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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 March 31, 2017

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, smaller reporting company, or an emerging growth company. See the definitions of large accelerated filer, accelerated filer, smaller reporting company, and emerging growth company in Rule 12b-2 of the Exchange Act:

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

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

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

As of April 30, 2017, there were 22,470,062 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 drug discovery platform;
- our plans to continue to evaluate data from Part C of our MoveDMD® clinical trial of edasalonexent for the treatment of Duchenne muscular dystrophy;
- ongoing and planned clinical trials for edasalonexent and other product candidates, whether conducted by us or by any future collaborators, including the timing of initiation of these trials and of the anticipated results;
- our plans to enter into collaborations for the development and commercialization of product candidates;
- the potential benefits of any future collaboration;
- our ability to receive research and development funding and achieve anticipated milestones under any future collaborations;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our ability to identify additional products or product candidates with significant commercial potential;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- developments relating to our competitors and our industry; and

- the impact of government laws and regulations.

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

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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)

	March 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 31,795	$ 23,596
Available-for-sale securities		14,931
Prepaid expenses and other current assets	1,020	1,001
Total current assets	32,815	39,528
Property and equipment, net	494	568
Restricted cash	113	113
Total assets	$ 33,422	$ 40,209
Liabilities and stockholders equity		
Current liabilities:		
Accounts payable	$ 942	$ 1,405
Accrued expenses	3,294	3,677
Current portion of notes payable, net of discount	3,260	3,243
Total current liabilities	7,496	8,325
Deferred rent, net of current portion	26	53
Notes payable, net of current portion and discount	1,657	2,479
Other liability	288	266
Total liabilities	9,467	11,123
Commitments (Note 7)		
Stockholders equity:		
Preferred stock, $0.001 par value per share, 5,000,000 shares authorized and no shares issued and outstanding		
Common stock, $0.001 par value per share, 150,000,000 shares authorized; 19,997,197 and 18,817,572 shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively	20	19
Additional paid-in capital	175,881	173,141
Accumulated other comprehensive loss		(4)
Accumulated deficit	(151,946)	(144,070)
Total stockholders equity	23,955	29,086
Total liabilities and stockholders equity	$ 33,422	$ 40,209

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 March 31,	
	2017	**2016**
Operating expenses:		
Research and development	$ 5,398	$ 6,436
General and administrative	2,363	2,770
Total operating expenses	7,761	9,206
Loss from operations	(7,761)	(9,206)
Other (expense) income:		
Interest expense	(149)	(243)
Interest and investment income	39	53
Other expense, net	(5)	(22)
Total other expense, net	(115)	(212)
Net loss	$ (7,876)	$ (9,418)
Net loss per share - basic and diluted	$ (0.41)	$ (0.61)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	19,093,273	15,335,516

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 March 31,	
	2017	**2016**
Net Loss	$ (7,876)	$ (9,418)
Other comprehensive income:		
Unrealized gains on available-for-sale securities	4	14
Total other comprehensive income:	4	14
Comprehensive loss	$ (7,872)	$ (9,404)

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)

	Three Months Ended March 31,	
	2017	2016
Operating activities		
Net loss	$ (7,876)	$ (9,418)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	88	92
Stock-based compensation expense	505	548
Accretion of discount/premium on investment securities	25	38
Non-cash interest expense	50	79
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(19)	(38)
Other assets		(2)
Accounts payable	(468)	230
Accrued expenses	(454)	(663)
Deferred rent	44	(13)
Net cash used in operating activities	(8,105)	(9,147)
Investing activities		
Purchases of available-for-sale securities		(32,111)
Sales and maturities of available-for-sale securities	14,910	3,332
Purchases of property and equipment	(9)	(290)
Net cash provided by (used in) investing activities	14,901	(29,069)
Financing activities		
Proceeds from at-the-market offering, net of issuance costs	2,215	
Proceeds from exercise of common stock options and warrants	21	87
Payments on borrowing	(833)	(833)
Net cash provided by (used in) financing activities	1,403	(746)
Net increase (decrease) in cash and cash equivalents	8,199	(38,962)
Cash and cash equivalents, beginning of period	23,596	62,780
Cash and cash equivalents, end of period	$ 31,795	$ 23,818
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 104	$ 163
Non-cash financing activities		
Fixed asset purchases included in accounts payable	$ 5	$ 68

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 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 its 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 Company pays Cowen 3% of the gross proceeds from any Common Stock sold through the sales agreement.

During the three months ended March 31, 2017, the Company sold an aggregate of 1,168,419 shares of Common Stock pursuant to the ATM program, at an average price of $1.95 per share, for gross proceeds of $2.3 million, resulting in net proceeds of $2.2 million after deducting sales commissions and offering expenses. On March 16, 2017, the Company reduced the amount of Common Stock that it was offering pursuant to the sales agreement, such that it was only offering $4.9 million of Common Stock from and after such date in addition to the $3.9 million of Common Stock it had sold under the ATM program as of that date. As of March 31, 2017, $4.9 million of Common Stock remained available for sale under the ATM program.

As of March 31, 2017, the Company had an accumulated deficit of $151.9 million. The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since its inception. The Company is subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its drug candidates, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology and regulatory approval and market acceptance of the Company s products. The Company anticipates that it will continue to incur significant

operating losses for the next several years as it continues to develop its product candidates. The Company adopted Accounting Standards Update (ASU) No. 2014-15, *Presentation of Financial Statements Going Concern: Disclosure of Uncertainties about an Entity s Ability to Continue as a Going Concern* (ASU 2014-15) in connection with the issuance of its consolidated financial statements for the year ended December 31, 2016. The Company s current operating plan, together with proceeds received from the issuance of common shares under the Company s ATM program in April 2017 of $4.1 million, provides for cash to fund operations for at least one year from the date of this Quarterly Report on Form 10-Q.

The Company will require substantial additional capital to fund operations. The Company has not generated any product revenues and has financed its operations primarily through public offerings and private placements of its equity securities. There can be no assurance that the Company will be able to obtain additional debt or equity financing or generate product revenue or revenues from collaborative partners, on terms acceptable to the Company, on a timely basis or at all. The failure of the Company to obtain sufficient

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funds on acceptable terms when needed could have a material adverse effect on the Company s business, results of operations, and financial condition.

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, 2016 and notes thereto, included in the Company s annual report on Form 10-K filed with the Securities and Exchange Commission on March 16, 2017 (the 2016 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 March 31, 2017, the results of its operations for the three months ended March 31, 2017 and 2016 and its cash flows for the three months ended March 31, 2017 and 2016. Such adjustments are of a normal and recurring nature. The results for the three months ended March 31, 2017 are not necessarily indicative of the results for the year ending December 31, 2017, or for any future period.

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

Use of Estimates

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

The Company utilizes certain estimates to record expenses relating to research and development contracts. These contract estimates, which are primarily related to the length of service of each contract, 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 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,

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Equity. For equity instruments granted to non-employees, the Company recognizes stock-based compensation expense on a straight-line basis.

During the three months ended March 31, 2017 and 2016, 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 March 31,	
	2017	2016
Research and development	$ 200	$ 172
General and administrative	305	376
Total	$ 505	$ 548

Net Loss Per Share

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

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

	Three Months Ended March 31,	
	2017	2016
Stock options	2,903,497	2,197,030
Common stock warrants	24,566	36,084
	2,928,063	2,233,114

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 May 2014, the FASB issued ASU 2014-09, *Revenue from Contracts with Customers*, (ASU 2014-09). The standard will replace existing revenue recognition standards and significantly expand the disclosure requirements for revenue arrangements. It may be adopted either retrospectively or on a modified retrospective basis to new contracts and existing contracts with remaining performance obligations as of the effective date. The standard is effective for public business entities for annual reporting periods beginning after December 15, 2017, with earlier adoption permitted as of annual reporting periods beginning after December 15, 2016. At this time, the Company does not have and has never had any contracts that are within the scope of ASU 2014-09 or its predecessor guidance, ASC 605 *Revenue Recognition*. The Company early adopted the standard on January 1, 2017. This will impact the accounting for any future transactions.

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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. It is 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, including the accounting for income taxes, forfeitures, and statutory tax withholding requirements, as well as classification in the statement of cash flows. The standard is retrospectively effective for annual reporting periods beginning after December 15, 2016. The Company adopted the standard in the three months ended March 31, 2017. The amended guidance eliminates the requirement that excess tax benefits be realized as a reduction in current taxes payable before the associated tax benefit can be recognized in additional paid-in capital. This created approximately $0.2 million of deferred tax assets relating to federal and state net operating losses that were fully offset by a corresponding increase in the valuation allowance. As a result, there was no cumulative effect adjustment to accumulated deficit. The implementation of ASU 2016-09 did not have a material impact on the Company s stock-based compensation expense. As part of the adoption of ASU 2016-09, the Company elected to record forfeitures as they occur.

Summary of Significant Accounting Policies

The Company s significant accounting policies are described in Note 2, Summary of Significant Accounting Policies, in the 2016 Annual Report on Form 10-K, and there were no significant changes to such policies in the three months ended March 31, 2017.

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 March 31, 2017 and December 31, 2016 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 three months ended March 31, 2017 or 2016.

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 using Level 2 inputs. Below is a summary of assets measured at fair value on a recurring basis (in thousands):

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	As of March 31, 2017			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash and cash equivalents:				
Money market funds	$ 25,671	$	$	$ 25,671
Corporate debt securities		3,798		3,798
Total assets	$ 25,671	$ 3,798	$	$ 29,469

	As of December 31, 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	$ 22,423	$	$	$ 22,423
Available-for-sale securities:				
Corporate debt securities		13,930		13,930
U.S. government-sponsored securities		1,001		1,001
Total assets	$ 22,423	$ 14,931	$	$ 37,354

At March 31, 2017 and December 31, 2016, the Company s cash equivalents consisted principally of money market funds, which approximated their fair value due to their short-term nature.

At March 31, 2017 and December 31, 2016, 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 March 31, 2017, the Company held no available-for-sale securities. The following table summarizes the available-for-sale securities held at December 31, 2016 (in thousands):

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value
December 31, 2016				
Corporate debt securities	$ 13,934	$	$ (4)	$ 13,930
U.S. government-sponsored securities	1,001			1,001
Total	$ 14,935	$	$ (4)	$ 14,931

The contractual maturities of all available-for-sale securities held at December 31, 2016 were one year or less. There were fifteen available-for-sale securities in an unrealized loss position at December 31, 2016, none of which had been in an unrealized loss position for more than 12 months. The aggregate fair value of these securities at December 31, 2016 was approximately $13.0 million. The

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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 did not hold any securities with other-than-temporary impairment at December 31, 2016.

Gross realized gains and losses on the sales of available-for-sale securities are included in other (expense) 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 (expense) income 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 (expense) income is based on the specific identification method for purposes of recording realized gains and losses. During the three-month periods ended March 31, 2017 and 2016 the Company received $14.9 million and $3.3 million, respectively, in proceeds from sales and maturities of available-for-sale securities the gains on which were not material to the Company s condensed consolidated results of operations.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	March 31, 2017	December 31, 2016
Accrued compensation	$ 542	$ 1,252
Accrued contracted research costs	2,319	1,850
Accrued professional fees	136	215
Accrued other	297	360
Total	$ 3,294	$ 3,677

6. Notes Payable

On August 27, 2014, the Company entered into a credit facility with MidCap Financial Trust, Flexpoint MCLS SPV LLC and Square 1 Bank, which was subsequently amended in March and December 2015 (as amended, the Credit Facility). The Credit Facility provided for 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

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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 March 31, 2017 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. warrants to purchase shares of convertible preferred stock to the lenders, which upon the closing of the Company s IPO in June 2015 were automatically converted into warrants to purchase an aggregate of 24,566 shares of Common Stock at an exercise price of $12.2114 per share. The warrants were exercisable immediately and have seven-year lives. The warrants associated with Term Loan A were initially valued at $0.1 million and the warrants associated with Term Loan B at $0.1 million using the Black-Scholes option-pricing model. The Company recorded debt discounts of $0.2 million in aggregate upon issuance of warrants, which is being accreted as interest expense using the effective-interest method over the remaining term of the loan.

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 March 31, 2017 and December 31, 2016, 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.

Future principal payments at March 31, 2017 are as follows (in thousands):

Year Ending December 31,		**Amount**
Remainder 2017		2,500
2018		2,500
Total	$	5,000
Less: discount for warrants and costs paid to lenders		(83)
Less: current portion		(3,260)

Note payable, net of current portion and discount	$	1,657

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During the three months ended March 31, 2017 and 2016, the Company recognized $0.1 million and $0.2 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. In November 2016, the Company signed a third lease amendment (the 2016 Lease Amendment). The 2016 Lease Amendment includes escalating rent payments and is effective through June 30, 2018. The Company is recognizing rent expense on a straight-line basis over the lease term.

Future minimum payments required under the non-cancelable operating lease as of March 31, 2017 are summarized as follows (in thousands):

Period Ending December 31,		Amount
Remainder 2017	$	1,019
2018		680
Total minimum lease payments	$	1,699

Rent expense for the three months ended March 31, 2017 and 2016 was $0.3 million and $0.2 million, respectively.

8. Preferred Stock

As of March 31, 2017, 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:

	As of March 31,	
	2017	2016
Warrants for the purchase of Common Stock	24,566	36,084
Options to purchase Common Stock	3,681,464	3,141,868
Employee Stock Purchase Plan	523,659	335,484
Total	4,229,689	3,513,436

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, 2017 and 2016 increases to the 2015 Plan added 752,702 and 612,531 authorized shares, respectively.

As of March 31, 2017, the Company had reserved 919,107 shares of Common Stock under the 2008 Plan, of which none remained available for future issuance. As of March 31, 2017, the Company had reserved 2,762,357 shares of Common Stock under the 2015 Plan, of which 777,967 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 March 31, 2017, 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, 2016	2,270,169	$ 6.11	8.14	$ 613
Granted	815,640	$ 1.24		
Exercised	(11,206)	$ 1.93		
Cancelled or forfeited	(171,106)	$ 6.46		
Outstanding at March 31, 2017	2,903,497	$ 4.74	8.41	$ 311
Vested and Exercisable at March 31, 2017	980,238	$ 5.87	6.72	$ 17

The total intrinsic value of options exercised for the three months ended March 31, 2017 and 2016 was $27 thousand and $124 thousand, respectively. The total fair value of employee and non-employee options vested for the three months ended March 31, 2017 and 2016 was $0.6 million and $0.5 million, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the three months ended March 31, 2017 and 2016 was $0.84 and $2.96, respectively.

At March 31, 2017, the total unrecognized compensation expense related to unvested stock option awards was $4.7 million. The Company expects to recognize that cost over a weighted-average period of approximately 2.5 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 authorized 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

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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, 2017 and 2016 increases to the 2015 ESPP added 188,175 and 153,132 authorized shares, respectively. As of March 31, 2017, 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 months ended March 31, 2017.

At-the-Market Financing

In April 2017, the Company issued 2,472,865 shares under its ATM program for net proceeds of $4.1 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. We have applied our SMART linker drug discovery platform to build an internal pipeline of product candidates for rare diseases, our primary focus, 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 edasalonexent 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. 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 has granted orphan medicinal product designation to edasalonexent for the treatment of DMD.

We are currently conducting the MoveDMD® Phase 1/2 trial of edasalonexent in ambulatory 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 previously reported positive safety, tolerability, pharmacokinetics and biomarker results from Part A of the MoveDMD trial in January 2016 and top-line results from Part B of the MoveDMD trial in January 2017. For Part B, we employed magnetic resonance imaging, or MRI, T2 as the primary endpoint. The trial was designed to provide an early biomarker for demonstrating a benefit on muscle composition. Although the primary endpoint was not met, we observed potential treatment-associated functional effects described below. Functional assessments are known to be clinically meaningful, and they have precedence as pivotal trial endpoints in DMD. We performed two prespecified analyses of functional assessments in Part B. The first prespecified analysis revealed that there were consistent numerical improvements versus placebo across all functional exploratory endpoint measures for the higher dose, as well as numerical improvements versus placebo across multiple functional exploratory endpoint measures for the lower dose, while the lower dose had mixed results versus the higher dose. For the second prespecified analysis

that measured rate of change, we performed functional assessments at baseline of Part A, at baseline of Part B, which was on average eight months later, and following 12 weeks of treatment at the endpoint of Part B. This design enabled the comparison of rate changes in functional ability between an extended off-treatment period prior to Part B dosing, on average eight months, and an edasalonexent treatment period of 12 weeks. In this prespecified rate change analysis, we observed numerical improvements across five functional assessments, and the results were consistent with the results from our first prespecified analysis. These functional assessments are important as the boys in the trial have declining function, and we believe these numerical improvements are indicative of a treatment effect in slowing this declining function with edasalonexent. Changes in these functional measures generally were not statistically significant in Part B of the MoveDMD trial, which was not powered for functional measures. We believe that the potential treatment-associated effects from these exploratory endpoints warrant further evaluation in Part C of the MoveDMD trial, which is the ongoing open-label extension portion of the trial. We have amended the Part C protocol to transition all patients participating in Part C of the trial to the 100 mg/kg/day dose, the higher of the two dosing levels administered in Part B, and have extended Part C by an additional 24 weeks to a total of 60 weeks. We intend to report the results from Part C in 2017, with an interim update in the third quarter of 2017 after all boys participating in Part C have completed 24 weeks of dosing with edasalonexent.

In September 2016, we announced a pre-clinical joint research collaboration with Sarepta Therapeutics, Inc., or Sarepta, a commercial stage developer of RNA targeted therapeutics, established to explore a combination drug treatment approach for DMD. In the Catabasis and Sarepta collaboration, increased dystrophin protein expression was seen with an exon-skip modality in combination with edasalonexent in the designated mouse model of DMD.

In addition to our work in DMD, we are evaluating other diseases where the inhibition of NF-κB may be beneficial for further therapeutic applications of edasalonexent. There are a number of other rare diseases where NF-κB is believed to play an important

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role, such as Becker muscular dystrophy, which is one of nine types of muscular dystrophy and is characterized by slowly progressive muscle weakness of the legs and pelvis, and IgA nephropathy, a kidney disease that is believed to result from activation of mucosal immunity, leading to the synthesis of aberrantly glycosylated polymeric immunoglobulin A1, or IgA1, which enters the circulation and lodges in a patient s kidneys interfering with their proper function.

In addition to edasalonexent, we are developing a pipeline of product candidates using our SMART linker drug discovery platform as potential treatments for rare diseases including cystic fibrosis, or CF, amyotrophic lateral sclerosis, or ALS, and Friedreich s ataxia, or FA. Our pipeline includes CAT-5571 and CAT-4001, for which we are currently conducting preclinical activities. We are developing CAT-5571 initially as a potential oral treatment for CF, with potential beneficial effects on both trafficking and function of cystic fibrosis transmembrane conductance regulator, or CFTR, and the clearance of *Pseudomonas aeruginosa*. In CF, a malfunctioning CFTR ion channel impairs chloride secretion, with deleterious effects on multiple organs, and particularly devastating effects on pulmonary, intestinal and pancreatic function. Patients affected with CF are also predisposed to respiratory failure caused by persistent lung infections, notably bacteria and most commonly *Pseudomonas aeruginosa*, that are difficult to treat with standard antibiotics. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, which are known to be impaired in CF. In addition, we are developing CAT-4001 as a potential treatment for neurodegenerative diseases such as FA and ALS, irrespective of mutation status. FA is a rare genetic disease that causes nervous system damage and compromises motor coordination. ALS, sometimes called Lou Gehrig s disease or classical motor neuron disease, is a rapidly progressive, fatal neurological disease that attacks the nerve cells responsible for controlling voluntary muscles. 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.

We have previously 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 nonalcoholic steatohepatitis, or NASH, based on the role of SREBP in lipid metabolism and known human polymorphisms associated with NASH disease progression. NASH is characterized by the build-up of fat in the liver and chronic inflammation, which can trigger progression to fibrosis and ultimately cirrhosis and sometimes hepatocellular carcinoma. 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, which, in addition to CAT-2003 and CAT-2054, includes other discovery-stage molecules with intermediate rates of hydrolysis.

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 March 31, 2017, we have raised an aggregate of $188.1 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, $3.9 million represented gross proceeds from our at-the-market, or ATM, offering program, and $0.8 million was from common stock option and warrant exercises.

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:

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

- expenses incurred under agreements with third parties, including contract research organizations that conduct clinical trials and research and development and preclinical activities on our behalf;

- the cost of consultants;

- the cost of lab supplies and acquiring, developing and manufacturing preclinical study materials; and

- facilities and other expenses, which include direct and allocated expenses for rent and maintenance of facilities, insurance and other supplies.

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

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

- ***Edasalonexent -*** 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 reported results from Part A of the MoveDMD trial in January 2016 and reported top-line safety and efficacy results for Part B of the trial in January 2017. Results from both Part A and Part B of the MoveDMD trial are described further above under Overview and under Business Our Product Candidates Edasalonexent Edasalonexent Clinical Development in our Annual Report on Form 10-K for the year ended December 31, 2016, which was filed with the Securities and Exchange Commission, or SEC, on March 16, 2017, which we refer to as our 2016 Annual Report on Form 10-K. In July 2016, we initiated an open-label extension, Part C of the MoveDMD trial, which is on-going and is expected to provide additional safety and efficacy data on edasalonexent. Following continued assessment of the effects in patients on edasalonexent in Part C of the MoveDMD trial, we will determine next steps for the development of the edasalonexent program.

- ***CAT-5571 -*** CAT-5571 is a SMART linker conjugate that contains cysteamine, a naturally occurring molecule that is a degradation product of the amino acid cysteine, and DHA. We are developing CAT-5571 initially as a potential oral treatment for CF with potential effects on both the CFTR and on the clearance of *Pseudomonas aeruginosa*. CAT-5571 is a small molecule that activates autophagy, a process that maintains cellular homeostasis and host defense mechanisms, which are known to be impaired in CF. In 2017, we are continuing preclinical evaluation of CAT-5571 in animal models of CF, and are conducting investigational new drug, or IND, application-enabling activities for CAT-5571. If we are successful in these activities, we intend to advance CAT-5571 into a Phase 1 clinical trial in 2018.

- ***CAT-4001 -*** CAT-4001 is a SMART linker conjugate that we designed to combine the potentially beneficial activities of monomethyl fumarate and DHA on the Nrf2 and NF-κB pathways. We are developing CAT-4001 initially for the treatment of severe, rare neurodegenerative diseases, such as FA and ALS, two diseases of the central nervous system in which the Nrf2 and NF-κB pathways have been implicated, irrespective of mutation status. Nrf2 is a gene transcription factor, a protein that works inside of cells to control the expression of genes, that control the body s response to cellular stress and oxidative damage. In 2017, we are continuing preclinical evaluation of CAT-4001 in animal models of FA as well as ALS.

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.

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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):

	Three Months Ended March 31,	
	2017	2016
Edasalonexent	$ 2,192	$ 1,624
CAT-2054	51	1,605
Other research and platform programs	639	1,058
Costs not directly allocated to programs:		
Employee expenses including cash compensation, benefits and stock-based compensation	1,968	1,533
Facilities	322	224
Consultants and professional expenses, including stock-based compensation	29	214
Other	197	178
Total costs not directly allocated to programs	2,516	2,149
Total research and development expenses	$ 5,398	$ 6,436

Since inception, the total direct expenses to support the edasalonexent program have been $25.7 million. Since we began separately tracking CAT-2054 in 2013, the direct expenses to support that program have totaled $12.8 million. We begin to separately track program expenses at candidate nomination.

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

- a continued acceptable safety profile of the products following approval.

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

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect to incur significant research and development costs for the foreseeable future, if and to the extent that our product candidate development programs progress. We expect that our research and development expenses in the year ending December 31, 2017 will be lower than in the year ending December 31, 2016 as a result of our conducting clinical trials supporting one program in 2017 as compared to two programs in 2016. 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

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commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and Administrative Expenses

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

We anticipate that our general and administrative expenses will increase in the future, if and to the extent necessary to support our continued operations, potential commercialization of our product candidates and costs of operating as a public company. These increases, if necessary, 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 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 and net amortization expense on available-for-sale securities, as offset by any interest income earned on our cash, cash equivalents, and available-for-sale securities.

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 three months ended March 31, 2017, there were no material changes to our critical accounting policies as reported in our 2016 Annual Report on Form 10-K.

Results of Operations

Comparison of the Three Months Ended March 31, 2017 and 2016

The following table summarizes our results of operations for the three months ended March 31, 2017 and 2016, together with the dollar change in those items (in thousands):

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	Three Months Ended March 31, 2017	2016	Period-to-Period Change
Operating expenses:			
Research and development	$ 5,398	$ 6,436	$ (1,038)
General and administrative	2,363	2,770	(407)
Total operating expenses	7,761	9,206	(1,445)
Loss from operations	(7,761)	(9,206)	1,445
Other expense, net	(115)	(212)	97
Net loss	$ (7,876)	$ (9,418)	$ 1,542

Research and Development Expenses

Research and development expenses decreased by $1.0 million to $5.4 million for the three months ended March 31, 2017 from $6.4 million for the three months ended March 31, 2016, a decrease of 16%. The decrease in research and development expenses was attributable to a net decrease of $1.4 million in direct program costs, partially offset by a $0.4 million increase in costs not directly allocated to programs. The $1.4 million decrease in direct program expenses included a $1.6 million decrease in costs to support our CAT-2054 program that was partially offset by a $0.2 million increase in costs to support other programs, primarily our edasalonexent program. The $0.3 million increase in costs not directly allocated to programs included a $0.5 million increase in employee compensation and facilities costs due to headcount additions and the effect of the 2016 lease amendment to the Company s operating lease for office and laboratory space that includes escalating rent payments through June 2018, partially offset by a $0.2 million decrease in consulting and professional expenses.

General and Administrative Expenses

General and administrative expenses decreased by $0.4 million to $2.4 million for three months ended March 31, 2017 from $2.8 million for the three months ended March 31, 2016, a decrease of 14%. The decrease in general and administrative expenses was attributable to a $0.5 million decrease in employee compensation due to headcount reductions, partially offset by a $0.1 million increase in consulting and professional services.

Other Expense, Net

Other expense, net decreased by $0.1 million to $0.1 million for the three months ended March 31, 2017 from $0.2 million for the three months ended March 31, 2016, primarily due to a $0.1 million decrease in interest expense due to the principal payments made on our credit facility.

Liquidity and Capital Resources

From our inception through March 31, 2017, we raised an aggregate of $188.1 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, $3.9 million represented gross proceeds from our ATM program, and

$0.8 million was from common stock option and warrant exercises. As of March 31, 2017, we had $31.8 million in cash and cash equivalents.

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 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,

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which became effective on July 19, 2016. We pay Cowen 3% of the gross proceeds from any common stock sold through the sales agreement.

During the three months ended March 31, 2017, we sold an aggregate of 1,168,419 shares of our common stock pursuant to the ATM program, at an average price of $1.95 per share, for gross proceeds of $2.3 million, resulting in net proceeds of $2.2 million after deducting sales commissions and offering expenses. On March 16, 2017, we reduced the amount of common stock that we were offering pursuant to the sales agreement, such that we were only offering $4.9 million of common stock from and after such date in addition to the $3.9 million of common stock we had sold under the ATM program as of that date. As of March 31, 2017, $4.9 million of common stock remained available for sale under the ATM program. In April 2017, we sold an aggregate of 2,472,865 shares of our common stock pursuant to the ATM program, at an average price of $1.72 per share, for gross proceeds of $4.3 million, resulting in net proceeds of $4.1 million after deducting sales commissions and offering expenses. As of April 30, 2017, $0.6 million of common stock remained available for sale under the ATM program.

Credit Facility

On August 27, 2014, we entered into a loan and security agreement with MidCap Financial Trust, Flexpoint MCLS SPV 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. Concurrently with the March 2015 Amendment, we drew down an additional $5.0 million under our term loan under the Credit Facility. 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. In connection with the draw downs under the Credit Facility, we issued warrants to purchase shares of convertible preferred stock to the lenders, which upon the closing of the Company s IPO were automatically converted into warrants to purchase an aggregate of 24,566 shares of our common stock at an exercise price of $12.2114 per share. The warrants were exercisable immediately and have seven-year lives.

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 March 31, 2017 and December 31, 2016, 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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Cash Flows

Comparison of the Three Months Ended March 31, 2017 and 2016

The following table provides information regarding our cash flows for the three months ended March 31, 2017 and 2016 (in thousands):

	Three Months Ended March 31,	
	2017	**2016**
Net cash used in operating activities	$ (8,105)	$ (9,147)
Net cash provided by (used in) investing activities	14,901	(29,069)
Net cash provided by (used in) financing activities	1,403	(746)
Net increase (decrease) in cash and cash equivalents	$ 8,199	$ (38,962)

Net Cash Used in Operating Activities

Net cash used in operating activities was $8.1 million for the three months ended March 31, 2017 and consisted primarily of a net loss of $7.9 million adjusted for non-cash items, including stock-based compensation expense of $0.5 million, depreciation and amortization expense of $0.1 million, non-cash interest expense of $0.1 million, and a net increase in operating assets of $0.9 million, which resulted primarily from a decrease in accounts payable and accrued expenses.

Net cash used in operating activities was $9.1 million for the three months ended March 31, 2016 and consisted primarily of a net loss of $9.4 million adjusted for non-cash items, including stock-based compensation expense of $0.6 million, non-cash interest expense of $0.1 million and depreciation and amortization expense of $0.1 million, and a net increase in operating assets of $0.5 million, which resulted primarily from a decrease in accrued expenses of $0.7 million partially offset by an increase in accounts payable of $0.2 million.

Net Cash Provided by (Used in) Investing Activities

Net cash provided by investing activities was $14.9 million during the three months ended March 31, 2017, which was primarily attributable to maturities and sales of available-for-sale securities. Net cash used in investing activities was $29.1 million during the three months ended March 31, 2016, which was attributable to net purchases of available-for-sale securities of $28.8 million and $0.3 million in laboratory equipment purchases.

Net Cash Provided by (Used in) Financing Activities

Net cash provided by financing activities was $1.4 million during the three months ended March 31, 2017 compared to $0.7 million of net cash used in financing activities during the three months ended March 31, 2016. The cash provided by financing activities in the three months ended March 31, 2017 was attributable to net proceeds of $2.2 million from our ATM offering, partially offset by $0.8 million in repayment of principal on the Credit Facility. The cash used in financing activities in the three months ended March 31, 2016 was primarily attributable to $0.8 million in repayment of principal on the Credit Facility partially offset by $0.1 million in proceeds from common stock option and warrant exercises.

Funding Requirements

If, and to the extent that, we continue the research and development of, and conduct clinical trials and seek marketing approval for, our product candidates, we expect our expenses to increase in connection with such activities. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales,

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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 believe that our existing cash and cash equivalents as of March 31, 2017, together with net proceeds of $4.1 million received from the issuance of common shares under our ATM program in April 2017, will enable us to fund our operating expenses and debt service and capital expenditure requirements based on our current operating plan for at least one year from the date of this Quarterly Report on Form 10-Q.

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 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.

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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

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 2016 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 March 31, 2017, we had cash and cash equivalents of $31.8 million and, as of December 31, 2016, we had cash, cash equivalents and available-for-sale securities of $38.5 million. Our cash and cash equivalents at each date consisted primarily of money market funds and our available-for-sale securities at December 31, 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. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio.

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

Item 4. Controls and Procedures

Management s Evaluation of our Disclosure Controls and Procedures

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

As of March 31, 2017, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this Quarterly Report on Form 10-Q. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of March 31, 2017, 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 March 31, 2017, 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 losses for at least the next several years. We may never achieve or maintain profitability.

We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur significant operating losses for at least the next several years. Our net losses were $36.1 million, $32.6 million and $21.9 million for the years ended December 31, 2016, 2015 and 2014, respectively, and $7.9 million for the three months ended March 31, 2017. As of March 31, 2017, we had an accumulated deficit of $151.9 million. We have not generated any revenues from product sales, have not completed the development of any product candidate and may never have a product candidate approved for commercialization. We have financed our operations to date primarily through private placements of our preferred stock, registered offerings of our common stock, including our initial public offering, or IPO, 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 to the extent we:

- continue to develop and conduct clinical trials with respect to our lead product candidate edasalonexent, including an ongoing Phase 1/2 clinical trial of edasalonexent for the treatment of Duchenne muscular dystrophy, or DMD;
- initiate and continue research and preclinical and clinical development efforts for our other product candidates;
- seek to identify and develop additional product candidates;

- seek regulatory and marketing approvals for our product candidates that successfully complete clinical trials, if any;

- establish sales, marketing, distribution and other commercial infrastructure in the future to commercialize various products for which we may obtain marketing approval, if any;

- require the manufacture of larger quantities of product candidates for clinical development and potentially commercialization;

- maintain, expand and protect our intellectual property portfolio;

- hire and retain additional personnel, such as clinical, quality control and scientific personnel;

- add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and

- add equipment and physical infrastructure to support our research and development programs.

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

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raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our company could cause our investors to lose all or part of their investments.

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

We began operations in 2008. Our operations to date have been limited to financing and staffing our company and developing our technology and conducting preclinical research and 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, our investors should consider our prospects in light of the costs, uncertainties, delays and difficulties frequently encountered by companies in the early stages of development, especially clinical-stage biopharmaceutical companies such as ours. Predictions about our future success or viability may not be as accurate as they could be if we had a longer operating history or a history of successfully developing and commercializing pharmaceutical products.

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, if and to the extent of certain ongoing activities, particularly if 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 or programs, such as our CAT-2000 program in nonalcoholic steatohepatitis, or NASH, or for our platform technology, we may not be able to enter into a collaboration for any of our product candidates or programs or for our platform technology on suitable terms or at all. In any event, our existing cash 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. We do not have any committed external source of funds.

Adequate additional financing may not be available to us on acceptable terms, on a timely basis 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 on acceptable terms as and when needed would have a material adverse effect on our business, results of operations, financial condition and ability to pursue our business strategy.

We believe that our existing cash and cash equivalents as of March 31, 2017, together with the net proceeds of $4.1 million received from the issuance of common shares under our ATM program in April 2017, will enable us to fund our operating expenses and debt service and capital expenditure requirements based on our current operating plan for at least one year from the date of this Quarterly Report on Form 10-Q. 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;

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

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

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

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

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

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

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

- the costs of operating as a public company.

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

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.

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 March 31, 2017, we had $5.0 million of outstanding principal payments 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.

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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 small molecule drugs by applying our Safely Metabolized And Rationally Targeted, or SMART, linker drug discovery platform. We have not yet succeeded and may never succeed in demonstrating efficacy and safety for any of our product candidates in 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.

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

- completion of the ongoing open-label extension of our MoveDMD 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 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.

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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 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, while we observed positive NF-κB biomarker data in Part A of our MoveDMD Phase 1/2 clinical trial of edasalonexent for the treatment of DMD that demonstrated NF-κB target engagement via statistically

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significant reduction in NF-κB controlled gene expression for the 67 mg/kg/day and 100 mg/kg/day dosing levels, the primary efficacy endpoint in Part B of the trial for the same dosing levels was not met. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted.

Because we are developing edasalonexent for the treatment of DMD, a disease for which regulatory authorities have not issued definitive guidance as to how to measure and demonstrate efficacy, there is increased risk that the outcome of our clinical trials will not be satisfactory for marketing approval.

There are currently only two therapies approved in the United States for the treatment of DMD. In addition, 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, while a general FDA Guidance for Industry on developing drugs for the treatment of DMD has been issued, regulatory authorities in the United States have not issued definitive direction as to how to measure and demonstrate efficacy. For example, we chose the primary endpoint in our MoveDMD Phase 1/2 clinical trial of edasalonexent for the treatment of DMD as change in muscle inflammation as measured by magnetic resonance imaging, or MRI, of leg muscles, which we believe had not previously been used as a primary endpoint for a Phase 2 or Phase 3 trial in DMD. We also included as exploratory endpoints the timed function tests best suited for this age group, specifically the 10-meter walk/run, 4-stair climb and time-to-stand tests, as well as other strength and functional measures, including the North Star ambulatory assessment questionnaire and the pediatric outcome data collection instrument. However, there is no definitive guidance from regulatory authorities that any of these endpoints, if met in a Phase 3 trial for DMD, would be satisfactory for marketing approval. In addition, since we believe we were the first company to use MRI T2 at 12 weeks as a primary endpoint in a DMD clinical trial, it is unclear whether the failure of edasalonexent to meet this efficacy endpoint is indicative of edasalonexent not having a treatment effect over the 12-week period, or if MRI T2 is an appropriate measure of treatment effect over a 12-week period.

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

Due to the lack of precedent, broad discretion of regulatory authorities, and a multitude of unique factors that impact the regulatory approval process, the likelihood of the approval of any of our product candidates that target rare diseases, such as DMD, cystic fibrosis, Friedreich s Ataxia and ALS, is uncertain, and we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned investigational new drug applications and NDAs for our product candidates, in a timely manner, or at all. For example, DMD is a rare disease for which there are only two FDA approved therapeutics. Further, the FDA may determine, after evaluation of our data and analyses, that such data and analyses do not support an NDA submission, filing or approval. Due to this lack of predictability, we may not have the resources necessary to meet regulatory requirements and successfully complete a potentially protracted, expensive and wide-ranging approval process for commercialization of product candidates for rare diseases.

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

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

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

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

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. Further, the clinical development of our product candidates is susceptible to

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

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

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

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

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. Any inability to complete preclinical and clinical development successfully could result in additional costs to us, or any future collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if (1) we, or any future collaborators, are required to modify our trial designs, such as required modifications with respect to patient populations, endpoints, comparators or trial duration, (2) we, or any future collaborators, are required to conduct additional clinical trials or other testing of our product candidates beyond the trials and testing that we, or they contemplate, (3) we, or any future collaborators, are unable to successfully complete clinical trials of our product candidates or other testing, (4) the results of these trials or tests are unfavorable, uncertain or are only modestly favorable, or (5) there are unacceptable safety concerns associated with our product candidates, we, or any future collaborators, may:

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;

- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;

- be subject to additional post-marketing testing or other requirements; or

- be required to remove the product from the market after obtaining marketing approval.

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

Adverse events or undesirable side effects caused by, or other unexpected properties of, our product candidates could cause us, any future collaborators, an institutional review board or regulatory authorities to interrupt, delay or halt clinical trials of one or more of our product candidates and could result in a more restrictive label or the delay or denial of marketing approval by the FDA or comparable foreign regulatory authorities. For example, in 2014, in our clinical trials of 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

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acceptable from a risk-benefit perspective. Many compounds that initially showed promise in clinical or earlier stage testing have later been found to cause undesirable or unexpected side effects that prevented further development of the compound.

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

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

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

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

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

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

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

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

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

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

- we, or any future collaborators, may have to delay, suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate, such as the delay we experienced in 2014 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

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candidates or allow our competitors, or the competitors of any future collaborators, to bring products to market before we, or any future collaborators, do and impair our ability, or the ability of any future collaborators, to successfully commercialize our product candidates and may harm our business and results of operations. In addition, many of the factors that lead to clinical trial delays may ultimately lead to the denial of marketing approval of any of our product candidates.

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

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

- the size and nature of the patient population;
- the severity of the disease under investigation;
- the proximity of patients to clinical sites;
- the eligibility criteria for the trial;
- the design of the clinical trial;
- efforts to facilitate timely enrollment;
- competing clinical trials; and
- clinicians and patients perceptions as to the potential advantages and risks of the drug being studied in relation to other available therapies, including any new drugs that may be approved for the indications we are investigating.

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

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

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;

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- we may be subject to fines, injunctions or the imposition of civil or criminal penalties;
- regulatory authorities may require the addition of labeling statements, such as a black box warning or a contraindication;
- we, or any future collaborators, may be required to create a Medication Guide outlining the risks of the previously unidentified side effects for distribution to patients;
- we, or any future collaborators, could be sued and held liable for harm caused to patients;
- the drug may become less competitive; and
- our reputation may suffer.

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

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

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

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

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;
- the prevalence and severity of any side effects;
- the clinical indications for which the product is approved;

- whether the product is designated under physician treatment guidelines as a first-line therapy or as a second- or third-line therapy;
- limitations or warnings, including distribution or use restrictions, contained in the product s approved labeling;
- our ability, or the ability of any future collaborators, to offer the product for sale at competitive prices;
- the product s convenience and ease of administration compared to alternative treatments;
- the willingness of the target patient population to try, and of physicians to prescribe, the product;
- the strength of sales, marketing and distribution support;
- the approval of other new products for the same indications;
- changes in the standard of care for the targeted indications for the product;
- the timing of market introduction of our approved products as well as competitive products;
- availability and amount of reimbursement from government payors, managed care plans and other third-party payors;
- adverse publicity about the product or favorable publicity about competitive products; and
- potential product liability claims.

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

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

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

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

We may collaborate with third parties for commercialization of any products that require a large sales, marketing and product distribution infrastructure. We intend to potentially commercialize our product candidates through collaboration, licensing and distribution arrangements with third parties. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

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

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

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

There are currently two therapies approved for the treatment of DMD in the United States, Sarepta Therapeutics drug Exondys 51, also known as eteplirsen, and Marathon Pharmaceuticals EMFLAZA , also known as deflazacort, a corticosteroid. Additionally, corticosteroid therapy, including prednisone, is often prescribed to treat the inflammation underlying DMD and to delay loss of ambulation. In addition, a number of companies are developing 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, PTC Therapeutics, Santhera Pharmaceuticals and Sarepta Therapeutics.

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

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or

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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 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

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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;
- 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.

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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 co