CATABASIS PHARMACEUTICALS INC
Form 10-Q
November 13, 2018
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# UNITED STATES

# SECURITIES AND EXCHANGE COMMISSION

**Washington, DC 20549**

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# FORM 10-Q

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**(Mark One)**

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

**For the quarterly period ended September 30, 2018**

**OR**

**o TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934**

**For the transition period from to**

**Commission File Number: 001-37467**

# Catabasis Pharmaceuticals, Inc.

(Exact Name of Registrant as Specified in Its Charter)

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

**(617) 349-1971**

(Registrant s Telephone Number, Including Area Code)

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

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

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

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

Emerging growth company x

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

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

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

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

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

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

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## PART I FINANCIAL INFORMATION

### Item 1. Financial Statements

**Catabasis Pharmaceuticals, Inc.**

**Condensed Consolidated Balance Sheets**

(In thousands, except share and per share data)

*(Unaudited)*

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

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

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**Catabasis Pharmaceuticals, Inc.**

**Condensed Consolidated Statements of Operations**

(In thousands, except share and per share data)

*(Unaudited)*

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

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

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**Catabasis Pharmaceuticals, Inc.**

**Condensed Consolidated Statements of Comprehensive Loss**

(In thousands)

*(Unaudited)*

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

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

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**Catabasis Pharmaceuticals, Inc.**

**Condensed Consolidated Statements of Cash Flows**

(In thousands)

*(Unaudited)*

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

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

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

***Liquidity***

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

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

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

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

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

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

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## 2. Summary of Significant Accounting Policies

***Basis of Presentation and Principles of Consolidation***

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

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

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

***Use of Estimates***

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

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

***Stock-Based Compensation***

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

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

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

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

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

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

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

***Net Loss Per Share***

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

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

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

***Cash, Cash Equivalents and Restricted Cash***

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

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

***Recent Accounting Pronouncements - Adopted***

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

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

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

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***Recent Accounting Pronouncements - Not Yet Adopted***

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

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

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

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

***Summary of Significant Accounting Policies***

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

## 3. Financial Instruments

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

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

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

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

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

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

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

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

## 4. Short-Term Investments

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

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

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

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

## 5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

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

## 6. Notes Payable

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

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

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

## 7. Commitments

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

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

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

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

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

## 8. Revenue Recognition

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

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

## 9. Stockholders Equity

***Preferred Stock***

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

***Common Stock Warrants***

*June 2018 Warrants*

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

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

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

*Warrants Associated with the Term Loan*

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

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## 10. Common Stock Reserved for Future Issuance

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

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

## 11. Stock Incentive Plans

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

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

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

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

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

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

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

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

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

## 12. Restructuring Costs

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

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

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

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

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

## 13. Subsequent Events

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

evaluated as required.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

**Financial Overview**

***Revenue***

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

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

***Research and Development Expenses***

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

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

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

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

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

registration of edasalonexent.

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

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

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

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

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

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

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

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

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

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

development expenses will increase significantly in the near term in connection with the substantial activities required to conduct our PolarisDMD trial and prepare for registration and commercialization of edasalonexent for the treatment of DMD. We do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

***General and Administrative Expenses***

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

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

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***Restructuring***

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

***Other Income (Expense)***

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

**Critical Accounting Policies and Significant Judgments and Estimates**

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

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

**Results of Operations**

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

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

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

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*Revenue*

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

*Research and Development Expenses*

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

*General and Administrative Expenses*

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

*Other Income (Expense), Net*

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

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

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

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

*Revenue*

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

*Research and Development Expenses*

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

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

*General and Administrative Expenses*

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

*Other Income (Expense), Net*

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

**Liquidity and Capital Resources**

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

***June 2018 Financing***

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

***At-the-Market Offering***

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

***Credit Facility***

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

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

***Cash Flows***

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

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

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

*Net Cash Used in Operating Activities*

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

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

*Net Cash (Used In) Provided by Investing Activities*

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

*Net Cash Provided by Financing Activities*

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

***Funding Requirements***

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

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

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

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

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

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

acceptable terms, or at all.

Until such time, if ever, as we can generate substantial product revenues, we expect to finance our cash needs through a combination of equity offerings, debt financings, collaborations, strategic alliances and licensing arrangements. We do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders rights. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

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

**Off-Balance Sheet Arrangements**

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

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**Contractual Obligations**

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

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**Item 3. Qualitative and Quantitative Disclosures about Market Risk**

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

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

**Item 4. Controls and Procedures**

**Management s Evaluation of our Disclosure Controls and Procedures**

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

Our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this Quarterly Report on Form 10-Q. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of September 30, 2018, our disclosure controls and procedures were effective at the reasonable assurance level.

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

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

During the three months ended September 30, 2018, there were no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15(f) and 15(d)-15(f) promulgated under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

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

- the costs of operating as a public company.

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

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

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

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

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

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

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

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

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

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

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

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

conduct clinical trials of our product candidates, completing clinical trials of our product candidates, obtaining marketing approval for these product candidates, manufacturing, marketing and selling those products for which we, or any of our future collaborators, may obtain marketing approval, satisfying any post-marketing requirements and obtaining reimbursement for our products from private insurance or government payors. Because of the uncertainties and risks associated with these activities, we are unable to accurately predict the timing and amount of increased expenses, and if or when we might achieve profitability. We and any future collaborators may never succeed in these activities and, even if we do, or any future collaborator does, we may never generate revenues that are large enough for us to achieve profitability. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would decrease the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our pipeline of product candidates or continue our operations. A decline in the value of our company could cause our investors to lose all or part of their investments.

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

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

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

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

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

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

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

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

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

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

- protection of our rights in our intellectual property portfolio;

- successful launch of commercial sales following any marketing approval;

- a continued acceptable safety profile following any marketing approval;

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

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

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

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

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