CATABASIS PHARMACEUTICALS INC
Form 10-Q
August 13, 2015
Table of Contents

UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-Q

(Mark One)

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

For the quarterly period ended June 30, 2015

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

(617) 349-1971

(Registrant s Telephone Number, Including Area Code)

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

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

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

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

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

As of August 1, 2015, there were 15,297,794 shares of the registrant s Common Stock, par value $0.001 per share, outstanding.

Table of Contents

TABLE OF CONTENTS

		Page
PART I. FINANCIAL INFORMATION		3
Item 1.	Condensed Financial Statements (unaudited)	3
	Condensed Balance Sheets as of June 30, 2015 and December 31, 2014	3
	Condensed Statements of Operations and Comprehensive Loss for the three and six months ended June 30, 2015 and 2014	4
	Condensed Statements of Cash Flows for the six months ended June 30, 2015 and 2014	5
	Notes to Condensed Financial Statements	6
Item 2.	Management s Discussion and Analysis of Financial Condition and Results of Operations	16
Item 3.	Quantitative and Qualitative Disclosures About Market Risk	28
Item 4.	Controls and Procedures	28
PART II. OTHER INFORMATION		29
Item 1A.	Risk Factors	29
Item 2.	Unregistered Sales of Equity Securities and Use of Proceeds	71
Item 6.	Exhibits	72
SIGNATURES		74
EXHIBIT INDEX		

Table of Contents

CAUTIONARY NOTE CONCERNING FORWARD-LOOKING STATEMENTS

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

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

- our plans to identify, develop and commercialize novel small molecule drugs based on our SMART linker technology platform;
- ongoing and planned clinical trials for our product candidates, whether conducted by us or by any future collaborators, including the timing of initiation of these trials and of the anticipated results;
- our plans to enter into collaborations for the development and commercialization of product candidates;
- the potential benefits of any future collaboration;
- our ability to receive research and development funding and achieve anticipated milestones under our 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

Table of Contents

Quarterly Report on Form 10-Q, particularly in the Risk Factors section, that could cause actual results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, collaborations, joint ventures or investments that we may make or enter into.

Table of Contents

PART I FINANCIAL INFORMATION

Item 1. Financial Statements

Catabasis Pharmaceuticals, Inc.

Condensed Balance Sheets

(in thousands, except share and per share data)

(Unaudited)

	June 30, 2015	December 31, 2014
Assets		
Current assets:		
Cash and cash equivalents	$ 81,548	$ 14,668
Prepaid expenses and other current assets	528	354
Total current assets	82,076	15,022
Property and equipment, net	230	288
Restricted cash	113	113
Other non-current assets	35	541
Total assets	$ 82,454	$ 15,964
Liabilities and stockholders equity (deficit)		
Current liabilities:		
Accounts payable	$ 2,486	$ 1,132
Accrued expenses	2,563	2,793
Current portion of notes payable, net of discount	2,346	309
Total current liabilities	7,395	4,234
Deferred rent, net of current portion	42	67
Notes payable, net of current portion and discount	7,352	4,439
Other liability	77	23
Warrant liability		108
Total Liabilities	14,866	8,871
Commitments (Note 7)		
Convertible preferred stock:		
Series A convertible preferred stock, $0.001 par value per share; 0 and 68,837,703 shares authorized, issued and outstanding at June 30, 2015 and December 31, 2014, respectively		47,898
Series B convertible preferred stock, $0.001 par value per share; 0 and 37,830,473 shares authorized, and 0 and 34,129,571 shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively		32,248
Stockholders equity (deficit):		
Preferred stock, $0.001 par value per share, 5,000,000 and 0 shares authorized at June 30, 2015 and December 31, 2014, respectively, 0 shares issued and outstanding		
Common stock, $0.001 par value, 150,000,000 shares authorized at June 30, 2015 and 132,000,000 shares authorized at December 31, 2014; 15,297,794 and 493,200 shares issued	15	1

and outstanding at June 30, 2015 and December 31, 2014, respectively				
Additional paid-in capital		157,493		2,326
Accumulated deficit		(89,920)		(75,380)
Total stockholders equity (deficit)		67,588		(73,053)
Total liabilities, convertible preferred stock and stockholders equity (deficit)	$	82,454	$	15,964

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Condensed Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

(Unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2015	**2014**	**2015**	**2014**
Operating expenses:				
Research and development	$ 5,931	$ 3,722	$ 10,547	$ 6,818
General and administrative	1,833	1,642	3,578	3,016
Total operating expenses	7,764	5,364	14,125	9,834
Loss from operations	(7,764)	(5,364)	(14,125)	(9,834)
Other (expense) income:				
Other income, net	4	1	13	1
Interest expense	(279)		(428)	
Total other (expense) income	(275)	1	(415)	1
Net loss and comprehensive loss	$ (8,039)	$ (5,363)	$ (14,540)	$ (9,833)
Net loss per share - basic and diluted	$ (8.07)	$ (13.42)	$ (19.46)	$ (24.72)
Weighted-average common shares outstanding used in net loss per share - basic and diluted	996,592	399,766	747,117	397,782

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Condensed Statements of Cash Flows

(in thousands)

(Unaudited)

	Six Months Ended June 30, 2015	Six Months Ended June 30, 2014
Operating activities		
Net loss	$ (14,540)	$ (9,833)
Reconciliation of net loss to net cash used in operating activities:		
Depreciation and amortization	93	130
Stock-based compensation expense	655	373
Non-cash interest expense	123	
Changes in assets and liabilities:		
Prepaid expenses and other current assets	(174)	(454)
Accounts payable	649	522
Accrued expenses	(11)	(491)
Deferred rent	(18)	(10)
Net cash used in operating activities	(13,223)	(9,763)
Investing activities		
Purchases of property and equipment	(35)	(157)
Net cash used in investing activities	(35)	(157)
Financing activities		
Proceeds from initial public offering, net of issuance costs	62,763	
Proceeds from issuance of preferred stock, net of issuance costs	12,331	
Proceeds from exercise of common stock options	51	22
Proceeds from borrowings	5,000	
Debt issuance costs	(7)	
Net cash provided by financing activities	80,138	22
Net increase (decrease) in cash and cash equivalents	66,880	(9,898)
Cash and cash equivalents, beginning of period	14,668	30,747
Cash and cash equivalents, end of period	$ 81,548	$ 20,849
Supplemental disclosure of cash flow information		
Cash paid for interest	$ 254	$
Non-cash financing activities		
Warrants for the purchase of series B preferred stock issued in conjunction with credit facility	$ 110	$
Initial public offering costs in accounts payable and accrued liabilities	$ 970	$
Reclassification of deferred IPO costs from non-current assets to additional paid-in capital	$ 1,787	$
Reclassification of warrant liability to additional paid-in capital	$ 206	$

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

Table of Contents

Catabasis Pharmaceuticals, Inc.

Notes to Condensed Financial Statements

(Unaudited)

1. Organization and Operations

The Company

Catabasis Pharmaceuticals, Inc. (the Company) is a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics based on the Company s proprietary Safely Metabolized And Rationally Targeted, or SMART, linker technology platform. The Company s SMART linker technology platform is based on the concept of treating diseases by simultaneously modulating multiple biological targets in one or more related disease pathways. The Company engineers bi-functional product candidates that are conjugates of two molecules, or bioactives, each with known pharmacological activity, joined by one of the Company s proprietary SMART linkers. The SMART linker conjugates are designed for enhanced efficacy and improved safety and tolerability. The Company seeks to develop therapies that modulate multiple targets in the disease pathway. The Company targets therapeutic areas and specific diseases with significant unmet medical needs where it believes it will have a competitive advantage. The Company s focus is on treatment for rare diseases, such as Duchenne Muscular Dystrophy (DMD). The Company is also developing other product candidates for the treatment of serious lipid disorders. The Company was incorporated in the State of Delaware on June 26, 2008.

Initial Public Offering

In June 2015, the Company completed its Initial Public Offering (the IPO). All of the shares issued and sold in the IPO were registered pursuant to a registration statement on Form S-1, as amended. An aggregate of 5,750,000 shares of Common Stock registered pursuant to the registration statement were sold at a price to the public of $12.00 per share (including 750,000 shares of Common Stock sold pursuant to the exercise of an overallotment option granted to the Company s underwriters in connection with the IPO). Net proceeds of the IPO were $61.8 million, after deducting underwriting discounts, commissions and offering-related expenses payable by the Company of approximately $7.2 million. In connection with the IPO, all shares of the Company s convertible preferred stock (the Preferred Stock) were automatically converted into an aggregate of 9,029,549 shares of its Common Stock and its outstanding warrants to purchase 315,688 shares of Preferred Stock were automatically converted into warrants to purchase 24,566 shares of Common Stock.

As of June 30, 2015, the Company had an accumulated deficit of $89.9 million. The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since 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 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.

2. Summary of Significant Accounting Policies

Reverse stock split

In connection with the IPO, the Company s board of directors and stockholders approved a 1-for-12.85 reverse stock split of the Company s Common Stock which was effected on June 11, 2015. Stockholders entitled to fractional shares as a result of the reverse stock split received a cash payment in lieu of receiving fractional shares. All share, share equivalent and per share amounts presented herein have been adjusted to

Table of Contents

reflect the reverse stock split. The ratios by which shares of Preferred Stock are convertible into shares of Common Stock have been adjusted to reflect the effects of the reverse stock split. Shares of Common Stock reserved for future issuance have been presented on an as converted basis and the financial statements disclose the adjusted conversion ratios.

Basis of Presentation

The accompanying financial statements have been prepared in accordance with United States generally accepted accounting principles (U.S. GAAP) and include all adjustments necessary for the fair presentation of the Company s financial position for the periods presented.

The unaudited interim condensed financial statements of the Company included herein have been prepared, pursuant to the rules and regulations of the Securities and Exchange Commission (the SEC). Certain information and footnote disclosures normally included in financial statements prepared in accordance with U.S. GAAP have been condensed or omitted from this report, as is permitted by such rules and regulations. Accordingly, these condensed financial statements should be read in conjunction with the financial statements as of and for the year ended December 31, 2014 and notes thereto, included in the Company s prospectus dated June 24, 2015, filed with the SEC pursuant to Rule 424(b)(4) on June 25, 2015 (the Prospectus).

The unaudited interim condensed 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 financial statements contain all adjustments which are necessary to fairly present the Company s financial position as of June 30, 2015, the results of its operations for the three and six months ended June 30, 2015 and 2014 and its cash flows for the six months ended June 30, 2015 and 2014. Such adjustments are of a normal and recurring nature. The results for the six months ended June 30, 2015 are not necessarily indicative of the results for the year ending December 31, 2015, or for any future period.

Use of Estimates

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

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

The Company utilized various valuation methodologies in accordance with the framework of the American Institute of Certified Public Accountants (AICPA), Audit and Accounting Practice Aid Series: Valuation of Privately Held Company Equity Securities Issued as Compensation (AICPA Practice Aid), to estimate the fair value of its Common Stock. The methodologies included the Option Pricing Method

utilizing the Backsolve Method (a form of the market approach defined in the AICPA Practice Aid) and the Probability-Weighted Expected Return Method based upon the probability of occurrence of certain future liquidity events such as an initial public offering or sale of the Company. Each valuation methodology includes estimates and assumptions

Table of Contents

that require the Company s judgment. Significant changes to the key assumptions used in the valuations could result in different fair values of Common Stock at each valuation date.

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

Fair Value of Financial Instruments

The fair value hierarchy prioritizes the inputs to valuation techniques used to measure fair value into three broad levels as follows: Level 1 inputs are quoted prices (unadjusted) in active markets for identical assets or liabilities; Level 2 inputs are inputs other than quoted prices included within Level 1 that are observable for the asset or liability, either directly or indirectly; and Level 3 inputs are unobservable inputs that reflect the Company s own assumptions about the assumptions market participants would use in pricing the asset or liability. Financial assets and liabilities are classified in their entirety based on the lowest level of input that is significant to the fair value measurement.

The carrying amounts reflected in the balance sheets for cash equivalents, restricted cash, prepaid expenses and other current assets, other assets, accounts payable and accrued expenses approximate their fair values at June 30, 2015 and December 31, 2014, due to their short-term nature. There have been no changes to the valuation methods during the year ended December 31, 2014 or the six months ended June 30, 2015. The Company evaluates transfers between levels at the end of each reporting period. There were no transfers of assets or liabilities between levels during the year ended 2014 or the six months ended June 30, 2015. At June 30, 2015, the carrying value of the Company s debt approximated fair value, which was determined using Level 3 inputs, including a quoted interest rate.

Recent Accounting Pronouncements

From time to time, new accounting pronouncements are issued by the Financial Accounting Standards Board (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 April 2015, the FASB issued ASU No. 2015-03, *Simplifying the Presentation of Debt Issuance Costs*. This standard amends existing guidance to require the presentation of debt issuance costs in the balance sheet as a deduction from the carrying amount of the related debt liability rather than as a deferred charge. It is effective for annual reporting periods beginning after December 15, 2015, but early adoption is permitted. The Company is currently evaluating the impact that this standard will have on its financial statements.

3. Financial Instruments

The following tables present information about the Company s financial assets and liabilities that have been measured at fair value, and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value. The Company determines the fair value of the preferred stock warrants (Note 6) using Level 3 inputs. Below is a summary of assets and liabilities measured at fair value on a recurring basis (in thousands):

Table of Contents

	As of June 30, 2015			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash Equivalents	17,005			17,005
Total	$ 17,005	$	$	$ 17,005

	As of December 31, 2014			
	Quoted Prices in Active Markets (Level 1)	Significant Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Total
Assets:				
Cash Equivalents	13,506			13,506
Total	$ 13,506	$	$	$ 13,506
Liabilities:				
Warrant Liability			108	108
Total	$	$	$	$ 108

As of June 30, 2015 and December 31, 2014, the Company s cash equivalents consisted principally of money market funds, which approximate their fair value due to their short-term nature. In connection with the completion of the IPO, warrants exercisable for Preferred Stock were automatically converted into warrants exercisable for Common Stock, resulting in the reclassification of the related warrant liability to additional paid-in capital as the warrants to purchase shares of Common Stock are accounted for as equity instruments (Note 6).

4. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	June 30, 2015	December 31, 2014
Accrued compensation	$ 752	$ 796
Accrued contracted research costs	1,164	1,109
Accrued professional fees	369	791
Accrued other	278	97
Total	$ 2,563	$ 2,793

Table of Contents

5. Notes Payable

On August 27, 2014, the Company entered into the Credit Facility. The Credit Facility, as amended on March 31, 2015, provides for initial borrowings of $5.0 million under a term loan (Term Loan A) and additional borrowings of up to $20.0 million under other term loans, for a maximum of $25.0 million. On August 27, 2014, the Company received proceeds of $5.0 million from the issuance of promissory notes under Term Loan A. On March 31, 2015, the Company received proceeds of $5.0 million from the issuance of promissory notes under another term loan (Term Loan B). Of the remaining borrowing capacity, (i) $5.0 million was available to be drawn until May 31, 2015, subject to the completion of an equity financing with net cash proceeds of at least $8.0 million and the issuance of warrants to purchase shares of the Company s stock equal in value to 3% of the amount drawn, and (ii) $10.0 million was available until July 31, 2015, subject to the completion of an initial public offering with net cash proceeds to the Company of at least $50.0 million and the issuance of warrants to purchase shares of the Company s common stock equal in value to 3% of the amount drawn. However, these amounts were not drawn. All amounts outstanding under the Credit Facility are due on October 1, 2018 and are collateralized by substantially all of the Company s personal property, other than its intellectual property.

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

In conjunction with Term Loan A, the Company issued warrants to purchase 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share (the 2014 Warrants) to the lenders. In conjunction with Term Loan B, the Company issued warrants to purchase an additional 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share (the 2015 Warrants) to the lenders (see Note 6). The 2014 Warrants and 2015 Warrants were exercisable immediately and have seven-year lives. The 2014 Warrants and 2015 Warrants were initially valued at $0.1 million and $0.1 million, respectively, using the Black-Scholes option-pricing model. The Company recorded debt discounts of $0.1 million and $0.1 million upon issuance of the 2014 Warrants and 2015 Warrants, respectively, which are being accreted as interest expense using the effective-interest method over the remaining term of the loan.

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

Upon the occurrence and continuation of an event of default, the lenders have the right to exercise certain remedies against the Company and the collateral securing the loans under the Credit Facility, including cash. Events of default include, among other things, failure to pay amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, which includes a material adverse change in the business, operations or conditions (financial or otherwise) of the Company or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against the Company in an amount greater than $250,000. The occurrence

10

Table of Contents

of a material adverse change could result in acceleration of the payment of the debt. At June 30, 2015 and December 31, 2014, the Company concluded that the likelihood of the acceleration of the debt was remote, as a material adverse change had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments. Following the occurrence and during the continuance of an event of default, borrowings under the Credit Facility shall bear interest at a rate per annum, which is five hundred basis points, or 5.0%, above the rate that is otherwise applicable.

The Company accounted for the amendment to the Credit Facility, effective March 31, 2015, as a debt modification pursuant to ASC Topic 470-50 *Modifications and Extinguishments.*

Estimated future principal payments at June 30, 2015 are as follows (in thousands):

Six Months Ending June 30, 2015		**Amount**
Remainder 2015	$	834
2016		3,333
2017		3,333
2018		2,500
Total	$	10,000
Less: discount for warrants and costs paid to lender		(302)
Less: current portion		(2,346)
Note payable, net of current portion and discount	$	7,352

Estimated future principal payments at December 31, 2014 are as follows (in thousands):

Year Ending December 31, 2014		**Amount**
2015	$	416
2016		1,667
2017		1,667
2018		1,250
Total	$	5,000
Less: discount for warrants and costs paid to lender		(252)
Less: current portion		(309)
Note payable, net of current portion and discount	$	4,439

During the three and six months ended June 30, 2015, the Company recognized $0.3 million and $0.4 million, respectively, of interest expense related to the Credit Facility.

6. Warrants

On August 27, 2014 and March 31, 2015, the Company issued the 2014 Warrants and 2015 Warrants to purchase an aggregate 315,688 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders in connection with the Credit Facility (Note 5). The 2014 Warrants and 2015 Warrants were exercisable immediately on issuance and have a seven-year life. The 2014 Warrants and 2015 Warrants were recorded as a liability and re-measured at each reporting date using the then-current assumptions. In connection with the completion of the IPO, the 2014 Warrants and the 2015 Warrants were automatically converted into warrants exercisable for Common Stock, resulting in the reclassification of the related warrant liability to

Table of Contents

additional paid-in capital as the warrants to purchase shares of Common Stock met the criteria to be accounted for as equity instruments. The warrant liability was re-measured to fair value prior to reclassification to additional paid-in capital. As of June 30, 2015, the Company had no outstanding warrant liability.

The following table provides a roll-forward of the fair value of the 2014 Warrants and 2015 Warrants determined by Level 3 inputs (in thousands):

	Fair Value
Balance at December 31, 2014	$ 108
Issuance of warrants at fair value	107
Change in fair value, recorded as a component of other income, net	(9)
Reclassification to additional paid-in capital	(206)
Balance at June 30, 2015	$

The fair value of warrants exercisable for 315,688 shares of series B convertible preferred stock, which were automatically converted into warrants exercisable for 24,566 shares of Common Stock with an exercise price of $12.14, was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

	June 30, 2015 (1)	December 31, 2014
Risk-free interest rate	1.97%	1.95%
Expected dividend yield	0.00%	0.00%
Expected term (in years)	6.5	6.7
Expected volatility	75.79%	78.16%

(1) Represents the date the warrants for series B convertible preferred stock converted to warrants for common stock

7. Commitments

In November 2010, the Company entered into a five-year, non-cancelable operating lease for office and laboratory space. In December 2011, the Company signed a lease amendment that expanded the leased premises beginning in the second quarter of 2012. The lease amendment also extended the term of the existing lease through June 30, 2017. The expansion lease includes a free rent period and escalating rent payments. The Company is recognizing rent expense on a straight-line basis over the lease term. The lease agreement provides for a five-year extension upon the completion of the lease term.

Future minimum payments required under the non-cancelable operating lease as of June 30, 2015 are summarized as follows (in thousands):

Year Ending December 31,		Amount
Remainder 2015	$	382
2016		760
2017		378
Total minimum lease payments	$	1,520

Rent expense for the three months ended June 30, 2015 and 2014 was $0.2 million and $0.2 million, respectively. Rent expense for the six months ended June 30, 2015 and 2014 was $0.4 million and $0.4 million, respectively.

Table of Contents

8. Convertible Preferred Stock

Upon closing the Company s IPO on June 30, 2015, all outstanding shares of the Company s preferred stock were automatically converted into 9,029,549 shares of Common Stock. As of June 30, 2015, the Company has 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 of such 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 Corporation may be reissued except as otherwise provided by law.

9. Common Stock Reserved for Future Issuance

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

	June 30, 2015	December 31, 2014
Conversion of Series A Preferred Stock		5,356,996
Conversion of Series B Preferred Stock		2,655,992
Warrants for the purchase of Preferred Stock		12,283
Warrants for the purchase of Common Stock	59,405	34,839
Options to purchase Common Stock	2,572,959	1,385,341
Employee Stock Purchase Plan	182,352	
Total	2,814,716	9,445,451

10. Stock-based compensation

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

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

December 31, 2016 and continuing until, and including, the fiscal year ending December 31, 2025, equal to the lowest of 1,297,334 shares of Common Stock, 4% of the number of shares of Common Stock outstanding on the first day of the fiscal year and an amount determined by the Company s board of directors.

Table of Contents

As of June 30, 2015, the Company had reserved 1,478,731 shares of Common Stock under the 2008 Plan, of which none remained available for future issuance. As of June 30, 2015, the Company had reserved 1,094,228 shares of Common Stock under the 2015 Plan, of which 1,038,758 shares remained available for future issuance.

Stock Options

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

	Shares	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (years)	Agregate Intrinsic Value (in 000 s)
Outstanding at December 31, 2014	1,226,140	$ 4.14	8.15	$ 6,579
Granted	360,530	10.64		
Exercised	(25,045)	2.05		
Cancelled or forfeited	(27,424)	4.09		
Outstanding at June 30, 2015	1,534,201	$ 5.70	8.16	$ 9,980
Exercisable at June 30, 2015	690,832	$ 3.06	7.02	$ 6,318
Vested or expected to vest at June 30, 2015	1,410,518	$ 5.46	8.06	$ 9,524

The total intrinsic value of options exercised for the three months ended June 30, 2015 and 2014 was $0, and $7,000, respectively. The total intrinsic value of options exercised for the six months ended June 30, 2015 and 2014 was $0.2 million and $28 thousand, respectively. The total fair value of options vested for the three months ended June 30, 2015 and 2014 was $0.2 million and $0.1 million, respectively. The total fair value of options vested for the six months ended June 30, 2015 and 2014 was $0.8 million and $0.3 million, respectively.

At June 30, 2015, the total unrecognized compensation expense related to unvested stock option awards, including estimated forfeitures, was $4.2 million. The Company expects to recognize that cost over a weighted-average period of approximately 3.0 years.

Stock-based compensation expense

Total stock-based compensation expense is recognized for stock options granted to employees and non-employees and has been reported in the Company s statements of operations as follows (in thousands):

	Three months ended June 30,		Six months ended June 30,	
	2015	2014	2015	2014
Research and development	$ 165	$ 109	$ 333	$ 189
General and administrative	189	119	322	184

Total	$ 354	$ 228	$ 655	$ 373

Table of Contents

Employee Stock Purchase Plan

In June 2015, the Company s board of directors adopted and the Company s stockholders approved the 2015 Employee Stock Purchase Plan (the 2015 ESPP) which became effective upon closing of the Company s IPO. The 2015 ESPP authorizes the initial issuance of up to a total of 182,352 shares of Common Stock to participating eligible employees. The number of shares increases each January 1, commencing on January 1, 2016 and ending on December 31, 2026, by an amount equal to the lesser of one percent of the outstanding shares as of the end of the immediately preceding fiscal year, 364,705 shares and any lower amount determined by the Company s board of directors. As of June 30, 2015 there has been no activity under the 2015 ESPP.

11. 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 dilutive net loss per share calculation, preferred stock, stock options, warrants to purchase Common Stock and warrants to purchase preferred stock are considered to be common stock equivalents but are 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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Convertible preferred stock		8,012,987		8,012,987
Stock options	1,534,201	1,209,072	1,534,201	1,209,072
Common stock warrants	59,405	34,839	59,405	34,839
	1,593,606	9,256,898	1,593,606	9,256,898

12. Subsequent Events

We consider 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 or to identify matters that require additional disclosure. Subsequent events have been evaluated as required. There were no material recognized subsequent events recorded in the condensed consolidated financial statements as of and for the three and six months ended June 30, 2015.

Operating Lease

On July 16, 2015, the Company entered into a Second Amendment to the Lease (the Second Lease Amendment) with DWF IV One Kendall, LLC (the Landlord), which amends certain terms of the Company s existing lease with the Landlord. The Second Lease Amendment expands the rentable square footage of the Company s leased premises from approximately 14,817 square feet to approximately 18,876 square feet. Pursuant to the Second Lease Amendment, the date on which the Company will become responsible for paying rent with respect to such additional square footage is September 1, 2015. The Second Lease Amendment will increase the future minimum payments described in Note 7 from approximately $1,520,000 to approximately $1,844,000.

Table of Contents

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

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

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

Overview

We are a clinical-stage biopharmaceutical company focused on the discovery, development and commercialization of novel therapeutics based on our proprietary Safely Metabolized And Rationally Targeted, or SMART, linker technology platform. Our SMART linker technology platform is based on the concept of treating diseases by simultaneously modulating multiple biological targets in one or more related disease pathways. We engineer bi-functional product candidates that are conjugates of two molecules, or bioactives, each with known pharmacological activity, joined by one of our proprietary SMART linkers. Our SMART linker conjugates are designed for enhanced efficacy and improved safety and tolerability, and we seek to develop therapies that modulate multiple targets in the disease pathway. We target therapeutic areas and specific diseases with significant unmet medical need where we believe we will have a competitive advantage. Our focus is on treatments for rare diseases, such as Duchenne muscular dystrophy, or DMD. We are also developing other product candidates for the treatment of serious lipid disorders.

We have applied our SMART linker technology platform to build a development pipeline that includes three clinical-stage product candidates and multiple programs in preclinical development. Our current drug candidates are small molecules. CAT-1004 is an oral small molecule that we believe has the potential to be a disease-modifying therapy for the treatment of DMD that may be able to regenerate muscle in boys regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. Our two other clinical-stage product candidates, CAT-2054 and CAT-2003, are members of our CAT-2000 series of molecules. We are initially developing CAT-2054 for the treatment of patients with hypercholesterolemia, or elevated low density lipoprotein cholesterol, or LDL-C, levels, for whom existing treatments are insufficient. Hypercholesterolemia is a disease that increases the risk of cardiovascular events. In January 2015, we initiated a Phase 1 clinical trial to assess the safety, tolerability and pharmacokinetics of CAT-2054 in healthy volunteers. In August 2015, we reported top-line data for the full range of doses tested in the single and multiple ascending dose portions of the trial. We have completed three Phase 2a trials of CAT-2003 in patient populations with elevated triglycerides or hypertriglyceridemia. CAT-4001 is in preclinical studies and is being developed for the treatment of severe, rare neurodegenerative diseases, such as Friedreich s ataxia and amyotrophic lateral sclerosis, two diseases of the central nervous system in which the Nrf2 and NF- • B pathways have been implicated.

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 our three clinical-stage compounds, building our intellectual property portfolio,

Table of Contents

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, a secured debt financing, and our initial public offering, or IPO.

In June 2015, we completed our IPO, in which we sold an aggregate of 5,750,000 shares of our common stock, including 750,000 shares of common stock sold pursuant to the underwriters exercise of their option to purchase additional shares of common stock, at a price to the public of $12.00 per share. Net proceeds from the IPO were $61.8 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $7.2 million.

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

In connection with the IPO, we also effected a one-for-12.85 reverse split of our common stock. All share, share equivalent and per share amounts presented herein have been adjusted to reflect the reverse stock split. The ratios by which shares of preferred stock are convertible into shares of Common Stock have been adjusted to reflect the effects of the reverse stock split.

We have not generated any revenue to date. We have incurred significant annual net operating losses in every year since our inception and expect to incur a net operating loss in 2015 and continue to incur net operating losses for the foreseeable future. As of June 30, 2015, we had an accumulated deficit of $89.9 million. We expect to continue to incur significant expenses and increasing operating losses for the next several years. Our net losses may fluctuate significantly from quarter to quarter and year to year. We anticipate that our expenses will increase significantly if and as we continue to develop and conduct clinical trials with respect to our CAT-1004 and CAT-2054 product candidates; initiate and continue research, preclinical and clinical development efforts for our other product candidates and potential product candidates; maintain, expand and protect our intellectual property portfolio; establish a commercial infrastructure to support the marketing and sale of certain of our product candidates; hire additional personnel, such as clinical, regulatory, quality control and scientific personnel; and operate as a public company.

From our inception through June 30, 2015, we have raised an aggregate of $172.1 million, composed of $92.9 million from private placements of preferred stock, $69.0 million in gross proceeds from our IPO, $10 million from a secured debt financing and $0.2 million from common stock option exercises.

Financial Overview

Revenue

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

strategic partners.

Research and Development Expenses

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

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

Table of Contents

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

- the cost of consultants;

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

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

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

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

- CAT-1004 is an orally administered SMART linker conjugate of salicylate and the omega-3 fatty acid docosahexaenoic acid, or DHA, that we designed to enhance the activity of salicylate and DHA in modulating the NF- B pathway at multiple points. NF- B, or nuclear factor kappa- light-chain-enhancer of activated B cells, is a protein that coordinates cellular response to damage, stress and inflammation and plays an important role in muscle health. We initiated patient enrollment in a Phase 1/2 clinical trial of CAT-1004 for the treatment of DMD in June 2015 and, subject to patient enrollment, expect to report top-line Phase 2 data in late 2016. If the results from our Phase 1/2 clinical trial and discussions with regulatory authorities regarding a pivotal trial are positive, we intend to initiate a six-month Phase 3 pivotal clinical trial of CAT-1004 in 2017 and seek marketing approval based on this Phase 3 trial.

- CAT-2054 is an orally administered SMART linker conjugate of the omega-3 fatty acid eicosapentaenoic acid, or EPA, and nicotinic acid, designed to modulate the SREBP pathway in the liver. SREBP is a master regulator of lipid metabolism and controls levels of both LDL-C and triglycerides. We are initially developing CAT-2054 to treat patients with hypercholesterolemia for whom existing treatments are insufficient. In January 2015, we initiated a Phase 1 clinical trial to assess the safety, tolerability and pharmacokinetics of CAT-2054 in healthy volunteers. In August 2015, we reported top-line data for the full range of doses tested in the single and multiple ascending dose portions of the trial. We intend to initiate a Phase 2a clinical trial of CAT-2054 for the treatment of hypercholesterolemia in the fourth quarter of 2015 and would expect to report Phase 2a data in mid-2016. If the results of the planned Phase 2a clinical trial are positive, we intend to initiate a Phase 2b clinical trial of CAT-2054 in

the fourth quarter of 2016.

- CAT-2003 is an orally administered SMART linker conjugate of EPA and nicotinic acid that we designed to modulate the SREBP pathway. We have completed three Phase 2a trials of CAT-2003 in patient populations with elevated triglycerides or hypertriglyceridemia.

Other research and development programs include our CAT-4001 development program and activities related to exploratory efforts, target validation and lead optimization for our early stage programs and our proprietary platform technology.

Table of Contents

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):

	Six Months Ended June 30,	
	2015	2014
CAT-1004	$ 2,731	$ 213
CAT-2054	2,331	1,356
CAT-2003	542	1,375
Other research and platform programs	1,026	585
Costs not directly allocated to programs:		
Employee expenses including cash compensation, benefits and stock-based compensation	2,857	2,257
Facilities	411	358
Consultants and professional expenses, including stock-based compensation	406	431
Other	243	243
Total costs not directly allocated to programs	$ 3,917	$ 3,289
Total research and development expenses	$ 10,547	$ 6,818

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 CAT-1004, CAT-2054 or any of our other current or potential product candidates. This is due to the numerous risks and uncertainties associated with developing medicines, including the uncertainties of:

- establishing an appropriate safety profile with investigational new drug application, or 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;

Table of Contents

- launching commercial sales of the products, if and when approved, whether alone or in collaboration with others; and

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

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

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect research and development costs to increase significantly for the foreseeable future as our product candidate development programs progress. However, we do not believe that it is possible at this time to accurately project total program-specific expenses through commercialization. There are numerous factors associated with the successful commercialization of any of our product candidates, including future trial design and various regulatory requirements, many of which cannot be determined with accuracy at this time based on our stage of development. Additionally, future commercial and regulatory factors beyond our control will impact our clinical development programs and plans.

General and Administrative Expenses

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

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

Other (Expense) Income, Net

Other (expense) income, net consists of interest expense incurred on debt instruments, amortized deferred financing costs and amortized debt discount, and changes in the fair value of the warrant liability, as offset by any interest income earned on our cash and cash equivalents. Upon completion of our IPO in June 2015, warrants to purchase preferred stock were converted to warrants to purchase common stock and as a result,

the Company no longer records fair value adjustment for its warrants.

Critical Accounting Policies and Significant Judgments and Estimates

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. The preparation of financial

Table of Contents

statements in conformity with U.S. generally accepted accounting principles, or GAAP, requires us to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. On an ongoing basis, we evaluate estimates, which include, but are not limited to, estimates related to clinical trial accruals, stock-based compensation expense, warrants to purchase redeemable securities, and reported amounts of expenses during the reported period. We base our estimates on historical experience and other market-specific or other relevant assumptions that we believe to be reasonable under the circumstances. Actual results may differ materially from those estimates or assumptions.

There have been no material changes to our accounting policies from those described in our prospectus filed with the SEC pursuant to Rule 424(b)(4) on June 25, 2015, or the Prospectus. It is important that the discussion of our operating results that follows be read in conjunction with the critical accounting policies disclosed in our Prospectus.

Results of Operations

Comparison of the Three Months Ended June 30, 2015 and 2014

The following table summarizes our results of operations for the three months ended June 30, 2015 and 2014 (in thousands):

	Three Months Ended June 30, 2015	Three Months Ended June 30, 2014	Period to Period Change
Operating expenses:			
Research and development	$ 5,931	$ 3,722	$ 2,209
General and administrative	1,833	1,642	191
Total operating expenses	7,764	5,364	2,400
Loss from operations	(7,764)	(5,364)	(2,400)
Other (expense) income, net	(275)	1	(276)
Net loss	$ (8,039)	$ (5,363)	$ (2,676)

Table of Contents

Research and Development Expenses

Research and development expenses increased by $2.2 million to $5.9 million for the three months ended June 30, 2015 from $3.7 million for the three months ended June 30, 2014, an increase of 59%. The increase in research and development expenses was primarily attributable to a net increase of $1.9 million in direct program costs, reflecting an increase of $1.8 million for CAT-1004 driven by the start of a Phase 1/2 clinical trial, an increase of $0.4 million for CAT-2054 driven by Phase 1 clinical trial progress, and an increase of $0.3 million in our general research and platform programs, which were partially offset by a decrease of $0.6 million in CAT-2003 clinical trial, manufacturing and preclinical development costs due to the completion of two Phase 2 clinical trials that were active in the three months ended June 30, 2014. In addition, the costs related to internal research and development increased by $0.3 million, primarily attributable to increased stock-based compensation expense.

General and Administrative Expenses

General and administrative expenses increased by $0.2 million to $1.8 million for the three months ended June 30, 2015 from $1.6 million for the three months ended June 30, 2014, an increase of 13%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $0.2 million associated with salaries, benefits, and stock-based compensation expenses from hiring additional senior personnel.

Other (Expense) Income, Net

Other (expense) income consists of interest expense, which increased by $0.3 million for the three months ended June 30, 2015 due to the interest expense on our credit facility, which we entered into in August 2014.

Comparison of the Six Months Ended June 30, 2015 and 2014

The following table summarizes our results of operations for the six months ended June 30, 2015 and 2014, together with the dollar change in those items (in thousands):

	Six Months Ended June 30,		Period to Period
	2015	2014	Change
Operating expenses:			
Research and development	$ 10,547	$ 6,818	$ 3,729
General and administrative	3,578	3,016	562
Total operating expenses	14,125	9,834	4,291
Loss from operations	(14,125)	(9,834)	(4,291)
Other (expense) income, net	(415)	1	(416)
Net loss	$ (14,540)	$ (9,833)	$ (4,707)

Research and Development Expenses

Research and development expenses increased by $3.7 million to $10.5 million for the six months ended June 30, 2015 from $6.8 million for the six months ended June 30, 2014, an increase of 54%. The increase in research and development expenses was primarily attributable to a net increase of $3.1 million in direct program costs, reflecting an increase of $2.5 million in costs related to CAT-1004 primarily related to the

Table of Contents

initiation of a Phase 1/2 clinical trial, an increase of $1.0 million in costs related to CAT-2054 primarily related to the initiation of a Phase 1 clinical trial, and an increase of $0.4 million in our general research and platform programs, which were partially offset by a decrease of $0.8 million in costs related to our CAT-2003 clinical trial, manufacturing and preclinical development costs due to the completion of two Phase 2 clinical trials that were active in the six months ended June 30, 2014. In addition, the costs related to internal research and development increased by $0.6 million, primarily attributable to stock-based compensation expense.

General and Administrative Expenses

General and administrative expenses increased by $0.6 million to $3.6 million for the six months ended June 30, 2015 from $3.0 million for the six months ended June 30, 2014, an increase of 20%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $0.4 million associated with hiring additional senior personnel, and an increase of $0.1 million in consulting expense primarily due to recruiting for two senior business development positions. The remaining increase of $0.1 million was spread in smaller amounts across several categories including insurance, facilities and general office expense.

Other (Expense) Income, Net

Other (expense) income consists of interest expense, which increased by $0.4 million for the six months ended June 30, 2015 due to the interest expense on our credit facility, which we entered into in August 2014.

Liquidity and Capital Resources

Overview

From our inception through June 30, 2015, we have raised an aggregate of $172.1 million, of which $92.9 million consisted of gross proceeds from private placements of preferred stock, $10.0 million consisted of gross proceeds from a secured debt financing, $69.0 million consisted of gross proceeds from our IPO, and $0.2 million resulted from common stock option exercises. As of June 30, 2015, we had $81.6 million in cash and cash equivalents.

Initial Public Offering

In June 2015, we completed the sale of an aggregate of 5,750,000 shares of our common stock, including 750,000 shares of common stock sold pursuant to the underwriters exercise of their option to purchase additional shares of common stock, in our IPO, at a price to the public of $12.00 per share. Net proceeds from the IPO were $61.8 million, after deducting underwriting discounts, commissions and offering-related expenses of approximately $7.2 million.

Credit Facility

On August 27, 2014, we entered into a loan and security agreement with MidCap Financial Trust, Flexpoint MCLS Holdings, LLC and Square 1 Bank. On March 31, 2015, we entered into an amendment to the credit facility, as amended, the Credit Facility. The Credit Facility provides for initial borrowings of $5.0 million and additional borrowings of up to $20.0 million. Concurrently with entering into the Credit Facility in August 2014, we borrowed $5.0 million under a term loan under the Credit Facility and we issued to the lenders warrants to purchase an aggregate of 157,844 shares of our series B preferred stock (24,566 shares of common stock on an as-converted basis) at an exercise price of $0.9503 per share. Concurrently with the amendment to the Credit Facility, we drew down an additional $5.0 million under our term loan under the Credit Facility and we issued to the lenders warrants to purchase an aggregate of 157,844 shares of our series B preferred stock

Table of Contents

(24,566 shares of common stock on an as-converted basis) at an exercise price of $0.9503 per share. An additional $5.0 million was available to us under the Credit Facility until May 31, 2015, subject to our completion of a series B preferred stock financing meeting certain conditions, including gross proceeds to us of at least $8.0 million, and our issuance of warrants to purchase shares of our stock equal in value to 3% of the amount drawn. However, none of this $5.0 million was drawn. The remaining $10.0 million was available to us until July 31, 2015, subject to the completion of an initial public offering with net cash proceeds to us of at least $50.0 million, and our issuance of warrants to purchase shares of our common stock equal in value to 3% of the amount drawn. However, none of this $10.0 million was drawn. All borrowings under the Credit Facility are due on October 1, 2018 and are collateralized by substantially all of our personal property, other than our intellectual property.

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

The Credit Facility also includes events of default, the occurrence and continuation of any of which provides the lenders the right to exercise remedies against us and the collateral securing the loans under the Credit Facility, including cash. These events of default include, among other things, failure to pay amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, which includes a material adverse change in our business, operations or conditions (financial or otherwise) or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against us in an amount greater than $250,000. The occurrence of a material adverse change could result in acceleration of payment of the debt. At June 30, 2015 and December 31, 2014, we concluded that the likelihood of the acceleration of the debt was remote, as a material adverse change had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments.

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

Preferred Stock Financing

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

Cash Flows

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

Table of Contents

	Six Months Ended June 30,	
	2015	2014
Net cash used in operating activities	$ (13,223)	$ (9,763)
Net cash used in investing activities	(35)	(157)
Net cash provided by financing activities	80,138	22
Net increase (decrease) in cash and cash equivalents	$ 66,880	$ (9,898)

Comparison of the Six Months Ended June 30, 2015 and 2014

Net Cash Used in Operating Activities

Net cash used in operating activities was $13.2 million for the six months ended June 30, 2015 and consisted primarily of a net loss of $14.5 million adjusted for non-cash items, including stock-based compensation expense of $0.7 million, non-cash interest expense of $0.1 million and depreciation and amortization expense of $0.1 million, and a net decrease in operating assets and liabilities of $0.4 million, which resulted primarily from a net increase in accounts payable and accrued expenses of $0.6 million partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

Net cash used in operating activities was $9.8 million for the six months ended June 30, 2014 and consisted primarily of a net loss of $9.8 million adjusted for non-cash items, including stock-based compensation expense of $0.4 million and depreciation and amortization expense of $0.1 million, and a net increase in operating assets of $0.5 million, which resulted primarily from an increase in prepaid expenses and other current assets of $0.4 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $35 thousand during the six months ended June 30, 2015 compared to $0.2 million during the six months ended June 30, 2014, a decrease of $0.1 million, which resulted primarily from decreased laboratory equipment expenditures in the six months ended June 30, 2015.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $80.1 million during the six months ended June 30, 2015 compared to $22 thousand during the six months ended June 30, 2014. The cash provided by financing activities for the six months ended June 30, 2015 primarily consisted of net proceeds received from the IPO of $62.8 million in June 2015, net proceeds of $12.3 million from the issuance of 13,062,965 shares of our series B preferred stock in March 2015, and $5.0 million from our borrowings under the Credit Facility in March 2015.

Funding Requirements

We expect our expenses to increase in connection with our ongoing activities, particularly as we continue the research and development of, and conduct clinical trials and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product sales, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are

Table of Contents

unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that our cash and cash equivalents, including the proceeds from our recent IPO, will enable us to fund our operating expenses and capital expenditure requirements at least through 2016. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of CAT-1004, CAT-2054 and our other current and potential product candidates, and because the extent to which we may enter into collaborations with third parties for the development of these product candidates is unknown, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the research and development of our product candidates. Our future capital requirements will depend on many factors, including:

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

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

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

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

Table of Contents

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, or SEC, rules.

Contractual Obligations

On July 16, 2015, the Company entered into a Second Amendment to the Lease (the Second Lease Amendment) with DWF IV One Kendall, LLC (the Landlord), which amends certain terms of the Company s existing lease with the Landlord. The Second Lease Amendment expands the rentable square footage of the Company s leased premises from approximately 14,817 square feet to approximately 18,876 square feet. Pursuant to the Second Lease Amendment, the date on which the Company will become responsible for paying rent with respect to such additional square footage is September 1, 2015 (the Expansion Space Rent Commencement Date). The Second Lease Amendment will increase the future minimum payments described in Note 7 from approximately $1,520,000 to approximately $1,844,000.

There were no other material changes to our contractual obligations and commitments described under Management s Discussion and Analysis of Financial Condition and Results of Operations in the Prospectus.

Table of Contents

Item 3. Qualitative and Quantitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of June 30, 2015, we had cash and cash equivalents of $81.6 million and, as of December 31, 2014, we had cash and cash equivalents of $14.7 million, consisting primarily 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, particularly because our investments are in short-term marketable securities. Our marketable securities are subject to interest rate risk and could fall in value if market interest rates increase. Due to the short-term duration of our investment portfolio and the low risk profile of our investments, an immediate 10% change in interest rates would not have a material effect on the fair market value of our investment portfolio. We have the ability to hold our marketable securities until maturity, and therefore we would not expect our operating results or cash flows to be affected to any significant degree by the effect of a change in market interest rates on our investments.

As of June 30, 2015 and December 31, 2014, we had no liabilities denominated in foreign currencies.

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 Exchange Act) that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Exchange Act is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC s rules and forms and (2) accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate, to allow timely decisions regarding required disclosure.

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

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

Changes in Internal Control Over Financial Reporting.

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

Table of Contents

PART II OTHER INFORMATION

Item 1A. Risk Factors

We operate in a dynamic and rapidly changing business environment that involves multiple 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 SEC. These risks and uncertainties, or other events that we do not currently anticipate or that we currently deem immaterial also may affect our results of operations, cash flows and financial condition. The trading price of our common stock could also decline due to any of these risks, and you could lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

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

We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur significant and increasing operating losses for at least the next several years. Our net losses were $18.1 million and $21.9 million for the years ended December 31, 2013 and 2014, respectively, and $14.5 million for the six months ended June 30, 2015. As of June 30, 2015, we had an accumulated deficit of $89.9 million. We have not generated any revenues from product sales, have not completed the development of any product candidate and may never have a product candidate approved for commercialization. We have financed our operations to date primarily through private placements of our preferred stock and a 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 (deficit) equity and working capital.

We anticipate that our expenses will increase substantially if and as we:

- continue to develop and conduct clinical trials with respect to our product candidates CAT-1004 and CAT-2054, including an ongoing Phase 1/2 clinical trial of CAT-1004 for which we initiated patient enrollment in June 2015 and an ongoing Phase 1 clinical trial of CAT-2054 that we initiated in January 2015;

- 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;

Table of Contents

- maintain, expand and protect our intellectual property portfolio;
- hire and retain additional personnel, such as clinical, quality control and scientific personnel;
- add operational, financial and management information systems and personnel, including personnel to support our product development and help us comply with our obligations as a public company; and
- add equipment and physical infrastructure to support our research and development programs.

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

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

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

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

Developing pharmaceutical products, including conducting preclinical studies and clinical trials, is a very time-consuming, expensive and uncertain process that takes years to complete. We expect our expenses to increase in connection with our ongoing activities, particularly as we initiate new clinical trials of, initiate new research and preclinical development efforts for and seek marketing approval for, our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we may incur significant

Table of Contents

commercialization expenses related to product sales, marketing, manufacturing and distribution to the extent that such sales, marketing, manufacturing and distribution are not the responsibility of a future collaborator. Furthermore, we expect to incur significant additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

We will be required to expend significant funds in order to advance the development of CAT-1004 and CAT-2054, as well as our other product candidates. In addition, while we may seek one or more collaborators for future development of our product candidates, and, in particular, expect that we would conduct any large Phase 3 clinical trial of CAT-2054 for the treatment of hypercholesterolemia in collaboration with one or more partners that would pay most of the associated costs, we may not be able to enter into a collaboration for any of our product candidates on suitable terms or at all. In any event, our existing cash and cash equivalents, including the net proceeds from our initial public offering of common stock, will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. We do not have any committed external source of funds.

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

We believe that our existing cash and cash equivalents as of June 30, 2015, including the net proceeds from our recent initial public offering of common stock, will enable us to fund our operating expenses, debt service and capital expenditure requirements at least through 2016. Our estimate as to how long we expect our existing cash and cash equivalents to be able to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

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

- our ability to identify a collaborator for CAT-2054 and the terms and timing of any collaboration agreement that we may establish for the development and commercialization of CAT-2054;

- 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;

Table of Contents

- 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.

The audit opinion on our financial statements contains a going concern explanatory paragraph.

Based on our cash balances, recurring losses, net capital deficiency and debt outstanding as of December 31, 2014 and our projected spending in 2015, which raise substantial doubt about our ability to continue as a going concern, the audit opinion on our audited financial statements as of and for the year ended December 31, 2014 contains a going concern explanatory paragraph. Given our planned expenditures for the next several years, including, without limitation, expenditures in connection with our clinical trials of CAT-1004 and CAT-2054, our independent registered public accounting firm may conclude, in connection with the audit of our financial statements for fiscal year 2015 or any other subsequent period, that there is substantial doubt regarding our ability to continue as a going concern. If we are unable to continue as a going concern, we might have to liquidate our assets and the values we receive for our assets in liquidation or dissolution could be significantly lower than the values reflected in our financial statements. Additionally, amounts due under our credit facility may become immediately due and payable upon the occurrence of a material adverse change, as defined under the loan agreement. In addition, the inclusion of a going concern explanatory paragraph by our auditors, our lack of cash resources and our potential inability to continue as a going concern may materially adversely affect our share price and our ability to raise new capital or to enter into critical contractual relations with third parties.

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

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, our existing stockholders ownership interest may be substantially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. For example, our credit facility with MidCap, Flexpoint and Square 1 contains restrictive covenants that, among other things and subject to certain exceptions, prohibit us from transferring any of our material assets, exclusively licensing our intellectual property (subject to certain exceptions), merging with or acquiring another entity, entering into a

Table of Contents

transaction that would result in a change of control, incurring additional indebtedness, creating any lien on our property, making investments in third parties or redeeming stock or paying dividends. Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. In addition, securing additional financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management s ability to oversee the development of our product candidates.

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

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

As of June 30, 2015, we had $10.0 million of outstanding borrowings under our credit facility with MidCap, Flexpoint and Square 1. We currently make monthly interest payments and, beginning in October 2015, will be required to repay principal and interest on these borrowings in monthly installments through October 2018. Subject to the restrictions in this existing credit facility, we could in the future incur additional indebtedness beyond our borrowings from MidCap, Flexpoint and Square 1.

Our outstanding indebtedness, including any additional indebtedness beyond our borrowings from MidCap, Flexpoint and Square 1, combined with our other financial obligations and contractual commitments could have significant adverse consequences, including:

- requiring us to dedicate a portion of our cash resources to the payment of interest and principal, reducing money available to fund working capital, capital expenditures, product development and other general corporate purposes;

- increasing our vulnerability to adverse changes in general economic, industry and market conditions;

- subjecting us to restrictive covenants that may reduce our ability to take certain corporate actions or obtain further debt or equity financing;

- limiting our flexibility in planning for, or reacting to, changes in our business and the industry in which we compete; and

- placing us at a competitive disadvantage compared to our competitors that have less debt or better debt servicing options.

We intend to satisfy our current and future debt service obligations with our existing cash and cash equivalents. However, we may not have sufficient funds, and may be unable to arrange for additional financing, to pay the amounts due under our existing debt instruments. Failure to make payments or comply with other covenants under our existing debt instruments could result in an event of default and acceleration of amounts due. Under our loan and security agreement with MidCap, Flexpoint and Square 1, the occurrence of an event that would reasonably be expected to have a material adverse effect on our business, operations, assets or condition is an event of default. If an event of default occurs and the lenders accelerate the amounts due, we may not be able to make accelerated payments, and the lenders could seek to enforce security interests in the collateral securing such indebtedness, which includes substantially all of our assets other than our intellectual

Table of Contents

property. In addition, the covenants under our credit facility, the pledge of our assets as collateral and the negative pledge with respect to our intellectual property could limit our ability to obtain additional debt financing.

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

Our approach to the discovery and development of product candidates based on our SMART linker technology 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 bi-functional small molecule drugs by applying our SMART linker technology platform. While we believe that applying our SMART linker technology platform may potentially enable drug research and clinical development that is more efficient than conventional small molecule drug research and development, this approach is unproven. We have not yet succeeded and may never succeed in demonstrating efficacy and safety for any of our product candidates in later stage clinical trials or in obtaining marketing approval thereafter. For example, although we have discovered and evaluated numerous compounds using our SMART linker technology platform, we have not yet advanced a compound into Phase 3 clinical development and no product created using the SMART linker technology platform has ever been approved for sale.

We are dependent on the success of our product candidates CAT-1004 and CAT-2054. If we are unable to complete the clinical development of, obtain marketing approval for or successfully commercialize at least one of these product candidates, either alone or with a collaborator, or if we experience significant delays in doing so, our business could be substantially harmed.

We currently have no products approved for sale and are investing a significant portion of our efforts and financial resources in the development of CAT-1004 for the treatment of Duchenne muscular dystrophy, or DMD, and CAT-2054 for the treatment of hypercholesterolemia. Our prospects are substantially dependent on our ability, or that of any future collaborator, to develop, obtain marketing approval for and successfully commercialize at least one of these product candidates.

The success of CAT-1004 and CAT-2054 will depend on several factors, including the following:

- successful completion of our ongoing clinical trials;
- initiation and successful enrollment and completion of additional clinical trials;
- safety, tolerability and efficacy profiles that are satisfactory to the U.S. Food and Drug Administration, or FDA, or any comparable foreign regulatory authority for marketing approval;

- timely receipt of marketing approvals from applicable regulatory authorities;
- the performance of our future collaborators, if any;
- the extent of any required post-marketing approval commitments to applicable regulatory authorities;
- establishment of supply arrangements with third-party raw materials suppliers and manufacturers;

Table of Contents

- 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, in the case of CAT-1004, therapies targeting dystrophin, utrophin and myostatin and inflammatory mediators.

Many of these factors are beyond our control, including 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 at least one of CAT-1004 or CAT-2054, on our own or with any future collaborator, or experience delays as a result of any of these or other factors, our business could be substantially harmed.

Our SMART linker technology platform may fail to help us discover and develop additional potential product candidates.

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

- compounds created through our SMART linker technology platform may not demonstrate improved efficacy, safety or tolerability;

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

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

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

Our research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product candidates. If we are unable to identify suitable additional compounds for preclinical and clinical development, our ability to develop

Table of Contents

product candidates and obtain product revenues in future periods could be compromised, which could result in significant harm to our financial position and adversely impact our stock price.

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

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

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

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

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

In some instances, there can be significant variability in safety or efficacy results between different clinical trials of the same product candidate due to numerous factors, including changes in trial procedures set forth in protocols, differences in the size and type of the patient populations, changes in and adherence to the dosing regimen and other clinical trial protocols and the rate of dropout among clinical trial participants. If we fail to receive positive results in clinical trials of our product candidates, the development timeline and regulatory approval and commercialization prospects for our most advanced product candidates, and, correspondingly, our business and financial prospects would be negatively impacted.

Table of Contents

Because we are developing CAT-1004 for the treatment of DMD, a disease for which regulatory authorities have not issued definitive guidance as to how to measure and demonstrate efficacy, there is increased risk that the outcome of our clinical trials will not be satisfactory for marketing approval.

There is currently no approved therapy for DMD in the United States. In addition, there has been limited historical clinical trial experience for the development of drugs to treat the underlying cause of DMD. As a result, the design and conduct of clinical trials for this disease, particularly for drugs to address the underlying cause of this disease, is subject to increased risk. In particular, regulatory authorities in the United States and European Union have not issued definitive guidance as to how to measure and demonstrate efficacy. We anticipate that the primary endpoint in our Phase 1/2 clinical trial of CAT-1004 for the treatment of DMD will be change in muscle inflammation as measured by magnetic resonance imaging, or MRI, of leg muscles. MRI markers of leg muscle inflammation have been observed to increase with age but decrease with initiation of steroid therapy. We intend to include as exploratory endpoints the timed function tests best suited for this age group, specifically the 10 meter walk/run, time to stand and 4-stair climb tests. However, due to the age and development stage of the patients we intend to enroll in this clinical trial, these endpoints may not be sufficiently sensitive to demonstrate efficacy over the period of the trial.

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

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

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

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

Clinical testing is expensive, time-consuming and uncertain as to outcome. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, or at all. The clinical development of our product candidates is susceptible to the risk of failure at any stage of drug development, including failure to demonstrate efficacy in a clinical trial or across a broad population of patients, the occurrence of adverse events that are severe or medically or commercially unacceptable, failure to comply with protocols or applicable regulatory requirements and determination by the FDA or any comparable foreign regulatory authority that a product candidate may not continue development or is not approvable. For example, our IND for CAT-2003 was placed on partial clinical hold by the FDA in November 2012 because of the need for additional nonclinical work to support potential expansion of dosing and duration of our proposed Phase 1 multiple ascending dose trial. Although the partial clinical hold was removed in July 2013, it is possible that any of our development programs may be placed on full or partial clinical hold by regulatory authorities at any point, which would delay and possibly prevent further development of our product candidates. It is possible that even if one or more of our product candidates has a beneficial effect, that effect will not be detected during clinical evaluation as a result of one or more of a variety of factors, including the size, duration, design, measurements, conduct or analysis of our clinical trials. Conversely, as a result of the same factors, our clinical trials may indicate an apparent positive effect of a product candidate that is greater than the actual positive effect, if any. Similarly, in

Table of Contents

our clinical trials we may fail to detect toxicity of or intolerability caused by our product candidates, or mistakenly believe that our product candidates are toxic or not well tolerated when that is not in fact the case.

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

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

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

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

- be delayed in obtaining marketing approval for our product candidates;
- not obtain marketing approval at all;
- obtain approval for indications or patient populations that are not as broad as intended or desired;
- obtain approval with labeling that includes significant use or distribution restrictions or significant safety warnings, including boxed warnings;

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

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

Table of Contents

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

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

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

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

- clinical trials of our product candidates may produce unfavorable or inconclusive results;

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

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

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

- our third-party contractors or those of any future collaborators, including those manufacturing our product candidates or components or ingredients thereof or conducting clinical trials on our behalf or on behalf of any future

collaborators, may fail to comply with regulatory requirements or meet their contractual obligations to us or any future collaborators in a timely manner or at all;

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

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

Table of Contents

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

- we, or any future collaborators, may have to delay, suspend or terminate clinical trials of our product candidates for various reasons, including a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate, such as the delay we experienced in one of our Phase 2 clinical trials of CAT-2003 while we reformulated CAT-2003 in a coated capsule and evaluated its tolerability;

- regulators or institutional review boards may require that we, or any future collaborators, or our or their investigators suspend or terminate clinical research for various reasons, including noncompliance with regulatory requirements or their standards of conduct, a finding that the participants are being exposed to unacceptable health risks, undesirable side effects or other unexpected characteristics of the product candidate or findings of undesirable effects caused by a chemically or mechanistically similar drug or drug candidate;

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

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

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

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

Product development costs for us, or any future collaborators, will increase if we, or they, experience delays in testing or pursuing marketing approvals and we, or they, may be required to obtain additional funds to complete clinical trials and prepare for possible commercialization of our product candidates. We do not know whether any preclinical tests or clinical trials will begin as planned, will need to be restructured, or will be completed on schedule or at all. Significant preclinical or clinical trial delays also could shorten any periods during which we, or any future

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

Table of Contents

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

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

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

In particular, the successful completion of our clinical development program for CAT-1004 for the treatment of DMD is dependent upon our ability to enroll a sufficient number of patients with DMD. DMD is a rare disease with a small patient population. Further, there are only a limited number of specialist physicians that regularly treat patients with DMD and major clinical centers that support DMD treatment are concentrated in a few geographic regions. In addition, other companies are conducting clinical trials and have announced plans for future clinical

trials that are seeking, or are likely to seek, to enroll patients with DMD and patients are generally only able to enroll in a single trial at a time. The small population of patients, competition for these patients and the limited trial sites may make it difficult for us to enroll enough patients to complete our clinical trials for CAT-1004 in a timely and cost-effective manner.

The clinical trials that we conduct may also have inclusion criteria that further limit the population of patients that we are able to enroll. For example, for the Phase 1/2 clinical trial of CAT-1004 for which we initiated patient enrollment in June 2015, we plan to enroll only ambulatory boys between ages four and seven who have not used steroids for at least six months prior to the trial. These inclusion criteria could present challenges to enrollment because steroid therapy for DMD is often initiated in this age range.

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

Table of Contents

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

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

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

- our reputation may suffer.

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

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

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

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

Table of Contents

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

- the timing of market introduction of our approved products as well as competitive products;

- availability and amount of reimbursement from government payors, managed care plans and other third-party payors;

- adverse publicity about the product or favorable publicity about competitive products; and

- potential product liability claims.

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

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

We do not have a sales, marketing or distribution infrastructure and have no experience in the sale, marketing or distribution of pharmaceutical products. To achieve commercial success for any approved product,

Table of Contents

we must either develop a sales and marketing organization or outsource these functions to third parties. We plan to use a combination of focused in-house sales and marketing capabilities and third-party collaboration, licensing and distribution arrangements to sell any of our products that receive marketing approval.

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

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

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

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

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

We are initially developing CAT-1004 for the treatment of DMD. While there are currently no therapies approved for the treatment of DMD in the United States, corticosteroid therapy is often prescribed to treat the inflammation underlying DMD and to delay loss of ambulation. In addition, a number of companies are developing therapies to treat DMD, one of which is already on the market in Europe and others are in the

Table of Contents

process of registration or late stage clinical development, including Eli Lilly, BioMarin Pharmaceuticals, Marathon Pharmaceuticals, PTC Therapeutics, Santhera Pharmaceuticals and Sarepta Therapeutics.

We are initially developing CAT-2054 for the treatment of hypercholesterolemia. There are many widely available products, including statins and cholesterol absorption inhibitors, approved for the treatment of patients with hypercholesterolemia. The market and development pipeline for cholesterol regulating therapies is especially large and competitive. If CAT-2054 is approved for the treatment of hypercholesterolemia, either as monotherapy or in combination therapies, it will face intense competition from current approved therapies as well as a number of therapeutic approaches in development, including PCSK9 inhibitors being developed by Sanofi/Regeneron Pharmaceuticals, Amgen and Pfizer; cholesterol ester transfer protein inhibitors, including those being developed by Merck and Eli Lilly; and other alternative therapies being developed by a range of competitors, including Esperion.

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

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we, or any future collaborators, may develop. Our competitors also may obtain FDA or other marketing approval for their products before we, or any future collaborators, are able to obtain approval for ours, which could result in our competitors establishing a strong market position before we, or any future collaborators, are able to enter the market.

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

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

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

Table of Contents

following the introduction of a generic drug, a significant percentage of the sales of any branded product or reference-listed drug may be typically lost to the generic product.

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

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

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

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

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

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Therefore, our ability, and the ability of any future collaborators, to commercialize any of our product candidates will depend in part on the extent to which

Table of Contents

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

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

In addition, increasingly, third-party payors are requiring higher levels of evidence of the benefits and clinical outcomes of new technologies and are challenging the prices charged. We cannot be sure that coverage will be available for any product candidate that we, or any future collaborator, commercialize and, if available, that the reimbursement rates will be adequate. Further, the net reimbursement for drug products may be subject to additional reductions if there are changes to laws that presently restrict imports of drugs from countries where they may be sold at lower prices than in the United States. An inability to promptly obtain coverage and adequate payment rates from both government-funded and private payors for any of our product candidates for which we, or any future collaborator, obtain marketing approval could significantly harm our operating results, our ability to raise capital needed to commercialize products and our overall financial condition.

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

We face an inherent risk of product liability claims as a result of the clinical testing of our product candidates despite obtaining appropriate informed consents from our clinical trial participants. We will face an even greater risk if we or any future collaborators commercially sell any product that we may or they may develop. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Regardless of the merits or eventual outcome, liability claims may result in:

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

Table of Contents

- withdrawal of clinical trial participants;
- significant costs to defend resulting litigation;
- substantial monetary awards to trial participants or patients;
- loss of revenue;
- reduced resources of our management to pursue our business strategy; and
- the inability to commercialize any products that we may develop.

Although we maintain general liability insurance of $2.0 million in the aggregate and clinical trial liability insurance of $3.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which could adversely affect our business, financial condition, results of operations and prospects.

Risks Related to Our Dependence on Third Parties

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

Our drug development programs and the potential commercialization of our product candidates will require substantial additional cash to fund expenses. We expect to seek one or more collaborators for the development and commercialization of one or more of our product candidates. For example, conducting pivotal Phase 3 clinical trials of CAT-2054 in patients with hypercholesterolemia will likely involve significant cost and we expect that we would conduct any large Phase 3 clinical trial of CAT-2054 in patients with hypercholesterolemia in collaboration with one or more partners. Likely collaborators may include large and mid-size pharmaceutical companies, regional and national pharmaceutical companies and biotechnology companies. In addition, if we are able to obtain marketing approval for CAT-1004 and other product candidates from foreign regulatory authorities, we intend to enter into strategic relationships with international biotechnology or pharmaceutical companies for the commercialization of CAT-1004 and other product candidates outside of the United States and Canada.

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

Table of Contents

Collaborations are complex and time-consuming to negotiate and document. Further, there have been a significant number of recent business combinations among large pharmaceutical companies that have resulted in a reduced number of potential future collaborators. In addition, our loan and security agreement with MidCap, Flexpoint and Square 1 contains, and any collaboration agreements that we enter into in the future may contain, restrictions on our ability to enter into potential collaborations or to otherwise develop specified compounds.

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

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

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

Collaborations involving our product candidates pose a number of risks, including the following:

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;

- collaborators may not perform their obligations as expected;

- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;

- collaborators may delay clinical trials, provide insufficient funding for a clinical trial program, stop a clinical trial or abandon a product candidate, repeat or conduct new clinical trials or require a new formulation of a product candidate for clinical testing;

- a collaborator with marketing and distribution rights to one or more products may not commit sufficient resources to the marketing and distribution of such product or products;

- disagreements with collaborators, including disagreements over proprietary rights, contract interpretation or the preferred course of development, might cause delays or termination of the

Table of Contents

research, development or commercialization of product candidates, might lead to additional responsibilities for us with respect to product candidates, or might result in litigation or arbitration, any of which would be time-consuming and expensive;

- collaborators may not properly maintain or defend our intellectual property rights or may use our proprietary information in such a way as to invite litigation that could jeopardize or invalidate our intellectual property or proprietary information or expose us to potential litigation;

- collaborators may infringe the intellectual property rights of third parties, which may expose us to litigation and potential liability; and

- collaborations may be terminated and, if terminated, may result in a need for additional capital to pursue further development or commercialization of the applicable product candidates.

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

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

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

Further, our reliance on these third parties for clinical development activities limits our control over these activities, but we remain responsible for ensuring that each of our studies is conducted in accordance with the applicable protocol, legal, regulatory and scientific standards. For example, notwithstanding the obligations of a contract research organization for a trial of one of our product candidates, we remain responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial. Moreover, the FDA requires us to comply with standards, commonly referred to as current Good Clinical Practices, or cGCPs, for conducting, recording and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. The FDA enforces these cGCPs through periodic inspections of trial sponsors, principal investigators, clinical trial sites and institutional review boards. If we or our third-party contractors fail to comply with applicable cGCPs, the clinical data generated in our clinical trials may be deemed unreliable and the FDA may require us to perform additional clinical trials before approving our product candidates, which would delay the marketing approval process. We cannot be certain that, upon inspection, the FDA will determine that any of our clinical trials comply with cGCPs. We are also required to register clinical trials and post the results of completed

clinical trials on a government-sponsored database, ClinicalTrials.gov, within certain timeframes. Failure to do so can result in fines, adverse publicity and civil and criminal sanctions.

Table of Contents

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

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

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

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

- manufacturing delays if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;

- the possible termination or nonrenewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;

- the possible breach by the third-party contractors of our agreements with them;

- the failure of third-party contractors to comply with applicable regulatory requirements;

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

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

Table of Contents

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

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

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

If any of our product candidates are approved by any regulatory agency, we plan to enter into agreements with third-party contract manufacturers for the commercial production and distribution of those products. It may be difficult for us to reach agreement with a contract manufacturer on satisfactory terms or in a timely manner. In addition, we may face competition for access to manufacturing facilities as there are a limited number of contract manufacturers operating under current good manufacturing practices, or cGMPs, that are capable of manufacturing our product candidates. Consequently, we may not be able to reach agreement with third-party manufacturers on satisfactory terms, which could delay our commercialization efforts.

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

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

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

Table of Contents

Risks Related to Our Intellectual Property

If we are unable to obtain and maintain sufficient patent protection for our product candidates, or if the scope of the patent protection is not sufficiently broad, our competitors could develop and commercialize products similar or identical to ours, and our ability to commercialize our product candidates successfully may be adversely affected.

Our success depends in large part on our ability to obtain and maintain patent protection in the United States and other countries with respect to our proprietary product candidates. If we do not adequately protect our intellectual property, competitors may be able to erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability. To protect our proprietary position, we file patent applications in the United States and abroad related to our novel product candidates that are important to our business. The patent application and approval process is expensive and time-consuming. We may not be able to file and prosecute all necessary or desirable patent applications at a reasonable cost or in a timely manner.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain. No consistent policy regarding the breadth of claims allowed in biotechnology and pharmaceutical patents has emerged to date in the United States or in many foreign jurisdictions. In addition, the determination of patent rights with respect to pharmaceutical compounds commonly involves complex legal and factual questions, which has in recent years been the subject of much litigation. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain.

Our pending patent applications cannot be enforced against third parties practicing the technology claimed in such applications unless and until a patent issues from such applications. Assuming the other requirements for patentability are met, currently, the first to file a patent application is generally entitled to the patent. However, prior to March 16, 2013, in the United States, the first to invent was entitled to the patent. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in our patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent offices in the United States and abroad. For example, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in post-grant review procedures, oppositions, derivations, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

Our pending and future patent applications may not result in patents being issued which protect our product candidates, in whole or in part, or which effectively prevent others from commercializing competitive products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries

Table of Contents

may diminish the value of our patents or narrow the scope of our patent protection. In addition, the laws of foreign countries may not protect our rights to the same extent or in the same manner as the laws of the United States. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does.

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

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

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

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

Competitors may infringe our patents, trademarks, copyrights or other intellectual property. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time consuming and divert the time and attention of our management and scientific personnel. Any claims we assert against perceived infringers could provoke these parties to assert counterclaims against us alleging that we infringe their patents, in addition to counterclaims asserting that our patents are invalid or unenforceable, or both. In any patent infringement proceeding, there is a risk that a court will decide that a patent of ours is invalid or unenforceable, in whole or in part, and that we do not have the right to stop the other party from using the invention at issue. There is also a risk that, even if the validity of such patents is upheld, the court will construe the patent s claims narrowly or decide that we do not have the right to stop the other party from using the invention at issue on the grounds that our patent claims do not cover the invention. An adverse outcome in a

Table of Contents

litigation or proceeding involving our patents could limit our ability to assert our patents against those parties or other competitors, and may curtail or preclude our ability to exclude third parties from making and selling similar or competitive products. Any of these occurrences could adversely affect our competitive business position, business prospects and financial condition. Similarly, if we assert trademark infringement claims, a court may determine that the marks we have asserted are invalid or unenforceable, or that the party against whom we have asserted trademark infringement has superior rights to the marks in question. In this case, we could ultimately be forced to cease use of such trademarks.

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

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

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

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

Table of Contents

If we are found to infringe a third-party s intellectual property rights, we could be forced, including by court order, to cease developing, manufacturing or commercializing the infringing product candidate or product. Alternatively, we may be required to obtain a license from such third-party in order to use the infringing technology and continue developing, manufacturing or marketing the infringing product candidate. However, we may not be able to obtain any required license on commercially reasonable terms or at all. Even if we were able to obtain a license, it could be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In addition, we could be found liable for monetary damages, including treble damages and attorneys fees if we are found to have willfully infringed a patent. A finding of infringement could prevent us from commercializing our product candidates or force us to cease some of our business operations, which could materially harm our business. Claims that we have misappropriated the confidential information or trade secrets of third parties could have a similar negative impact on our business.

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

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

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

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

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can

Table of Contents

result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Non-compliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. If we fail to maintain the patents and patent applications covering our product candidates, our competitive position would be adversely affected.

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

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

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

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

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

Given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized. We expect to seek extensions of patent terms in the United States and, if available, in other countries where we are prosecuting patents. In the United States, the Drug Price Competition and Patent Term Restoration Act of 1984 permits a patent term extension of up to five years beyond the normal expiration of the

Table of Contents

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

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

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

In addition, while we typically require our employees, consultants and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own, which may result in claims by or against us related to the ownership of such intellectual property. If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to our senior management and scientific personnel.

Risks Related to Regulatory Approval and Other Legal Compliance Matters

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

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

Table of Contents

candidates in the United States or in any other jurisdiction. We have limited experience in conducting and managing the clinical trials necessary to obtain marketing approvals, including FDA approval of an NDA.

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

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

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

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

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

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

Regulatory authorities in some jurisdictions, including the United States and Europe, may designate drugs for relatively small patient populations as orphan drugs. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, which is generally defined as a patient population of fewer than 200,000 individuals annually in the United States. While we have obtained orphan drug designation from the FDA for CAT-1004 for the treatment of DMD, we, or any future collaborators, may seek orphan drug designations for other product candidates or in other jurisdictions and may be unable to obtain such designations.

Even if we, or any future collaborators, obtain orphan drug designation for a product candidate, we, or they, may not be able to obtain orphan drug exclusivity for that product candidate. Generally, a product with orphan drug designation only becomes entitled to orphan drug exclusivity if it receives the first marketing approval for

Table of Contents

the indication for which it has such designation, in which case the FDA or the EMA will be precluded from approving another marketing application for the same drug for that indication for the applicable exclusivity period. The applicable exclusivity period is seven years in the United States and ten years in Europe. The European exclusivity period can be reduced to six years if a drug no longer meets the criteria for orphan drug designation or if the drug is sufficiently profitable so that market exclusivity is no longer justified. Orphan drug exclusivity may be lost if the FDA or the EMA determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

Even if we, or any future collaborators, obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. Even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is clinically superior in that it is shown to be safer, more effective or makes a major contribution to patient care.

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

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

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

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

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

Table of Contents

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

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

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

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

- restrictions on such products, manufacturers or manufacturing processes;
- restrictions on the labeling or marketing of a product;
- restrictions on product distribution or use;
- requirements to conduct post-marketing studies or clinical trials;
- warning letters or untitled letters;

- withdrawal of the products from the market;
- refusal to approve pending applications or supplements to approved applications that we submit;
- recall of products;
- restrictions on coverage by third-party payors;
- fines, restitution or disgorgement of profits or revenues;
- suspension or withdrawal of marketing approvals;
- refusal to permit the import or export of products;

Table of Contents

- product seizure; or

- injunctions or the imposition of civil or criminal penalties.

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

In the United States and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that could, among other things, prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability, or the ability of any future collaborators, to profitably sell any products for which we, or they, obtain marketing approval. We expect that current laws, as well as other healthcare reform measures that will be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we, or any future collaborators, may receive for any approved products.

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

- an annual, non-deductible fee on any entity that manufactures or imports specified branded prescription drugs and biologic agents;

- an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program;

- expansion of federal healthcare fraud and abuse laws, including the False Claims Act and the Anti-Kickback Statute, new government investigative powers and enhanced penalties for noncompliance;

- a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices;

- extension of manufacturers Medicaid rebate liability;

- expansion of eligibility criteria for Medicaid programs;

- expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program new requirements to report financial arrangements with physicians and teaching hospitals;

- a new requirement to annually report drug samples that manufacturers and distributors provide to physicians; and

- a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, was unable to reach required goals,

Table of Contents

thereby triggering the legislation s automatic reduction to several government programs. These changes included aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. The American Taxpayer Relief Act of 2012, among other things, reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. These new laws may result in additional reductions in Medicare and other healthcare funding.

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

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

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

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

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

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

identifiable health information;

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

Table of Contents

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

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

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

We are subject to numerous environmental, health and safety laws and regulations, including those governing laboratory procedures and the handling, use, storage, treatment and disposal of hazardous materials and wastes. From time to time and in the future, our operations may involve the use of hazardous and flammable materials, including chemicals and biological materials, and may also produce hazardous waste products. Although we contract with third parties for the disposal of these materials and waste products, we cannot completely eliminate the risk of contamination or injury resulting from these materials. In the event of contamination or injury resulting from the use or disposal of our hazardous materials, we could be held liable for any resulting damages, and any liability could exceed our resources. We also could incur significant costs associated with civil or criminal fines and penalties for failure to comply with such laws and regulations.

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

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

Table of Contents

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

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

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

If a product is intended for the treatment of a serious or life-threatening condition and the product demonstrates the potential to address unmet needs for this condition, the treatment sponsor may apply for FDA fast track designation. In July 2015, the FDA notified us that we obtained fast track designation for CAT-1004 for the treatment of DMD. Fast track designation does not ensure that we will experience a faster development, regulatory review or approval process compared to conventional FDA procedures. Additionally, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data from our clinical development program.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to retain our Chief Executive Officer and to attract, retain and motivate qualified personnel.

We are highly dependent on the pharmaceutical research and development and business development expertise of Jill C. Milne, our President and Chief Executive Officer. Although we have entered into an employment agreement with Dr. Milne, this agreement does not prevent her from terminating her employment with us at any time. In the future, we may be dependent on other members of our management, scientific and development team.

Our ability to compete in the highly competitive biotechnology and pharmaceuticals industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. Our industry has experienced a high rate of turnover of management personnel in recent years. If we lose one or more of our executive officers or other key employees, our ability to implement our business strategy successfully could be seriously harmed. Furthermore, replacing executive officers or other key employees may be difficult and may take an extended period of time because of the limited number of individuals in our industry with the breadth of skills and experience required to develop, gain marketing approval of and commercialize products successfully. Competition to hire from this limited pool is intense, and we may be unable to hire, train, retain or motivate these additional key employees on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific and clinical personnel from universities and research institutions.

We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our research and development and commercialization strategy. Our consultants and advisors may be employed by other entities and may have commitments under consulting or

advisory contracts with those entities that may limit their availability to us. If we are unable to continue to attract and retain highly qualified personnel, our ability to develop and commercialize our product candidates will be limited.

Table of Contents

We expect to grow our organization, and as a result, we may encounter difficulties in managing our growth, which could disrupt our operations.

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

Risks Related to Our Common Stock

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

Our shares of common stock began trading on The NASDAQ Global Market on June 25, 2015. Given the limited trading history of our common stock, there is a risk that an active trading market for our shares will not be sustained, which could put downward pressure on the market price of our common stock and thereby affect the ability of our stockholders to sell their shares. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

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

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

- the success of existing or new competitive products or technologies;

- the timing and results of clinical trials of CAT-1004, CAT-2054 and any of our other product candidates;
- commencement or termination of collaborations for our development programs;
- failure or discontinuation of any of our development programs;
- results of clinical trials of product candidates of our competitors;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;

Table of Contents

- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to develop additional product candidates or products;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- announcement or expectation of additional financing efforts;
- sales of our common stock by us, our insiders or other stockholders;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in estimates or recommendations by securities analysts, if any, that cover our stock;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors;
- general economic, industry and market conditions; and
- the other factors described in this Risk Factors section.

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

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

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor s report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. In our prospectus filed with the SEC pursuant to Rule 424(b)(4) on June 25, 2015, we did not include all of the executive compensation related information that would be required if we were not an emerging growth company. We cannot predict whether investors will find our common stock less attractive if we rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Table of Contents

In addition, the JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. This allows an emerging growth company to delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

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

As a public company, and particularly after we are no longer an emerging growth company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being a public company and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. For example, we expect that the rules and regulations applicable to us as a public company may make it more difficult and more expensive for us to obtain director and officer liability insurance, which could make it more difficult for us to attract and retain qualified members of our board of directors. We are currently evaluating these rules and regulations, and cannot predict or estimate the amount of additional costs we may incur or the timing of such costs. These rules and regulations are often subject to varying interpretations, in many cases due to their lack of specificity, and, as a result, their application in practice may evolve over time as new guidance is provided by regulatory and governing bodies. This could result in continuing uncertainty regarding compliance matters and higher costs necessitated by ongoing revisions to disclosure and governance practices.

Pursuant to SOX Section 404 we will be required to furnish a report by our management on our internal control over financial reporting beginning with our second filing of an Annual Report on Form 10-K with the Securities and Exchange Commission, or the SEC, after we become a public company. However, while we remain an emerging growth company, we will not be required to include an attestation report on internal control over financial reporting issued by our independent registered public accounting firm. To achieve compliance with SOX Section 404 within the prescribed period, we will be engaged in a process to document and evaluate our internal control over financial reporting, which is both costly and challenging. In this regard, we will need to continue to dedicate internal resources, potentially engage outside consultants and adopt a detailed work plan to assess and document the adequacy of internal control over financial reporting, continue steps to improve control processes as appropriate, validate through testing that controls are functioning as documented and implement a continuous reporting and improvement process for internal control over financial reporting. Despite our efforts, there is a risk that we will not be able to conclude, within the prescribed timeframe or at all, that our internal control over financial reporting is effective as required by SOX Section 404. If we identify one or more material weaknesses, it could result in an adverse reaction in the financial markets due to a loss of confidence in the reliability of our financial statements.

Table of Contents

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

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. As of August 1, 2015, we have outstanding 15,297,794 shares of common stock, of which 9,547,794 shares are subject to restrictions on transfer under 180-day lock up arrangements with the underwriters of our public offering. These restrictions are due to expire on December 21, 2015, resulting in these shares becoming eligible for resale into the public market on December 22, 2015 if they are registered under the Securities Act or if they qualify for an exemption from registration under the Securities Act, including under Rules 144 or 701.

We also plan to register all 3,093,793 shares of common stock that we may issue under our equity compensation plans. Once we register these shares, they can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates and the lock-up agreements.

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

We have never declared nor paid cash dividends on our capital stock. We currently plan to retain all of our future earnings, if any, to finance the operation, development and growth of our business. Furthermore, the terms of our credit facility with MidCap, Flexpoint and Square 1 preclude us from paying dividends, and any future debt agreements may also preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be your sole source of gain for the foreseeable future.

Our executive officers, directors and principal stockholders, if they choose to act together, have the ability to control all matters submitted to stockholders for approval.

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock and their affiliates, in the aggregate, beneficially owned shares representing approximately 79.6% of our capital stock as of August 1, 2015. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

- delay, defer or prevent a change in control;
- entrench our management or the board of directors; or

- impede a merger, consolidation, takeover or other business combination involving us that other stockholders may desire.

Provisions in our corporate charter documents and under Delaware law could make an acquisition of us, which may be beneficial to our stockholders, more difficult and may prevent attempts by our stockholders to replace or remove our current management.

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

Table of Contents

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

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

Moreover, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the General Corporation Law of the State of Delaware, which prohibits a person who owns in excess of 15% of our outstanding voting stock from merging or combining with us for a period of three years after the date of the transaction in which the person acquired in excess of 15% of our outstanding voting stock, unless the merger or combination is approved in a prescribed manner. This could discourage, delay or prevent someone from acquiring us or merging with us, whether or not it is desired by, or beneficial to, our stockholders.

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

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

Table of Contents

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

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

Item 2. Use of Proceeds

Recent Sales of Unregistered Securities

Set forth below is information regarding shares of common stock issued, and options granted, by us during the three months ended June 30, 2015 that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Included is the consideration, if any, we received for such shares and options and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, or the SEC, under which exemption from registration was claimed. No underwriters were involved in any such issuances.

On April 30, 2015, pursuant to the terms of our Amended and Restated 2008 Stock Incentive Plan, as amended, we granted to certain of our employees options to purchase an aggregate of 94,162 shares of our common stock, at an exercise price of $11.05 per share. On June 30, 2015, pursuant to the terms of our 2015 Stock Incentive Plan, we granted to certain of our directors options to purchase an aggregate of 55,470 shares of our common stock, at an exercise price of $12.21 per share. These stock options were issued pursuant to written compensatory plans or arrangements with our employees and directors, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required.

On June 30, 2015, upon the closing of our initial public offering, all 116,030,239 shares of our then-outstanding redeemable convertible preferred stock were automatically converted into 9,029,549 shares of common stock. The issuance of such shares of common stock was exempt from the registration requirements of the Securities Act, pursuant to Section 3(a)(9) and Section 4(s) of the Securities Act.

Use of Proceeds from IPO

In June 2015, we completed our initial public offering, or our IPO, in which we issued and sold 5,750,000 shares of our common stock at a public offering price of $12.00 per share, including 750,000 shares of common stock sold pursuant to the underwriters exercise of their option to purchase additional shares of common stock, for aggregate gross proceeds of $69.0 million. All of the shares issued and sold in the IPO were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333-204144), which was declared effective by the SEC on June 24, 2015. Citigroup Global Markets Inc. and Cowen and Company, LLC acted as joint book-running managers of the offering and

as representatives of the underwriters. Oppenheimer & Co. Inc. and Wedbush Securities Inc. acted as co-managers for the offering. The offering commenced on June 24, 2015 and did not terminate until the sale of all of the shares offered.

The net offering proceeds to us, after deducting underwriting discounts of $4.8 million and offering expenses payable by us totaling $2.4 million, were approximately $61.8 million. No offering expenses were

Table of Contents

paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates.

We had not used any of the net offering proceeds as of June 30, 2015 because the IPO closed on June 30, 2015. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on June 25, 2015.

Item 6. Exhibits

The exhibits filed as part of this Quarterly Report on Form 10-Q are set forth on the Exhibits Index, which Exhibit Index is incorporated herein by reference.

Table of Contents

Exhibit Number	Exhibit
3.1	Restated Certificate of Incorporation of the Registrant, as filed as Exhibit 3.1 to the Registrant s Form 8-K dated July 1, 2015 and incorporated herein by reference.
3.2	Amended and Restated By-Laws of the Registrant, as filed as Exhibit 3.2 to the Registrant s Form 8-K dated July 1, 2015 and incorporated herein by reference.
31.1	Certification of principal executive officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2	Certification of principal financial officer pursuant to Rules 13a-14(a) or 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1	Certifications pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of The Sarbanes-Oxley Act of 2002, by the Registrant s principal executive officer and principal financial officer
101.INS*	XBRL Instance Document
101.SCH*	XBRL Taxonomy Extension Schema Document
101.CAL*	XBRL Taxonomy Calculation Linkbase Document
101.DEF*	XBRL Taxonomy Extension Definition Linkbase Document
101.LAB*	XBRL Taxonomy Label Linkbase Document
101.PRE*	XBRL Taxonomy Presentation Linkbase Document

*Submitted electronically herewith

Table of Contents

SIGNATURES

Pursuant to the requirements of Section 13 or 15(d) of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

Catabasis Pharmaceuticals, Inc.

Date: August 13, 2015	By:	/s/ IAN C. SANDERSON Ian C. Sanderson *Chief Financial Officer and Treasurer (Principal Financial Officer and Principal Accounting Officer)*