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Filed pursuant to Rule 424(b)(4)
Registration Statement No. 333-204144

PROSPECTUS

5,000,000 Shares

Catabasis Pharmaceuticals, Inc.

Common Stock
$12.00 per share

This is the initial public offering of our common stock. We are selling 5,000,000 shares of common stock in this offering.

We have granted the underwriters an option to purchase up to 750,000 additional shares of common stock to cover over-allotments.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "CATB."

Investing in our common stock involves risks. See "Risk Factors" beginning on page 11.

We are an "emerging growth company" under applicable Securities and Exchange Commission rules and will be eligible for reduced public company disclosure requirements. See "Summary—Implications of Being an Emerging Growth Company."

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities or passed upon the accuracy or adequacy of this prospects. Any representation to the contrary is a criminal offense.

	Per Share	Total
Public Offering Price	$ 12.00	$ 60,000,000
Underwriting Discount(1)	$ 0.84	$ 4,200,000
Proceeds to Catabasis Pharmaceuticals, Inc. (before expenses)	$ 11.16	$ 55,800,000

(1) We refer you to "Underwriting" beginning on page 164 for additional information regarding underwriter compensation.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to approximately $15.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these potential purchasers and any of these potential purchasers could determine to purchase more, less or no shares in this offering.

The underwriters expect to deliver the shares to purchasers on or about June 30, 2015 through the book-entry facilities of The Depository Trust Company.

Citigroup **Cowen and Company**

Oppenheimer & Co. **Wedbush PacGrow**

Prospectus dated June 24, 2015

We are responsible for the information contained in this prospectus. We have not authorized anyone to provide you with different information, and we take no responsibility for any other information others may give you. If anyone provides you with different or inconsistent information, you should not rely on it. We are not, and the underwriters are not, making an offer to sell these securities in any jurisdiction where the offer or sale is not permitted. You should not assume that the information contained in this prospectus is accurate as of any date other than the date on the front of this prospectus.

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SUMMARY

This summary highlights, and is qualified in its entirety by, the more detailed information and financial statements included elsewhere in this prospectus. This summary does not contain all of the information that may be important to you. You should read and carefully consider the entire prospectus, especially our financial statements and the notes thereto appearing at the end of this prospectus and the "Risk Factors" section of this prospectus, before deciding to invest in our common stock.

Except as otherwise indicated herein or as the context otherwise requires, references in this prospectus to "Catabasis," "the company," "we," "us" and "our" refer to Catabasis Pharmaceuticals, Inc.

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. Our initial focus is on treatments for rare diseases. We are also developing other product candidates for the treatment of serious lipid disorders. We target therapeutic areas and specific diseases with significant unmet medical need where we believe we will have a competitive advantage. We seek to develop therapies that modulate multiple targets in the disease pathway.

Our Product Candidates

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. The following chart summarizes key information regarding our product candidates. We hold worldwide rights to all of our product candidates.

Series	Pathway	Product Candidate	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	Status
CAT-1000	*NF-κB*	CAT-1004	• Duchenne muscular dystrophy					• Expect to enroll patients in Phase 1/2 trial in June 2015
CAT-2000	*SREBP*	CAT-2054	• Hypercholesterolemia					• Completed preliminary analysis of Phase 1 data, expect full data in the third quarter of 2015 • Expect to initiate Phase 2a trial in the fourth quarter of 2015
		CAT-2003	• Hypertriglyceridemias					• Three Phase 2a trials completed
CAT-4000	*NRF2/ NF-κB*	CAT-4001	• Friedreich's ataxia • Amyotrophic lateral sclerosis					• Expect to continue pre-clinical studies in 2015

CAT-1004

CAT-1004 is an oral small molecule that we believe has the potential to be a disease-modifying therapy for the treatment of Duchenne muscular dystrophy, or 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. CAT-1004 is a SMART linker conjugate of salicylate, a non-steroidal anti-inflammatory drug, and the omega-3 fatty acid docosahexaenoic acid, or DHA, a naturally occurring unsaturated fatty acid with anti-inflammatory properties. We designed CAT-1004 to enhance the activity of salicylate and DHA in modulating the NF-κB pathway at multiple points. NF-κB, 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 believe that CAT-1004 modulates the disease pathway in DMD by inhibiting activated NF-κB and reducing the movement of activated NF-κB to the nucleus of the cell. Activated NF-κB drives muscle degeneration and suppresses muscle regeneration. Chronic activation of NF-κB has been reported in multiple skeletal muscle disorders, including muscular dystrophies, atrophy and inflammatory myopathies. In animal models of DMD, CAT-1004 inhibited activated NF-κB, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials in adults, CAT-1004 inhibited activated NF-κB and was well tolerated with no observed safety concerns. We plan to initiate patient enrollment in a Phase 1/2 clinical trial of CAT-1004 in boys with DMD in June 2015. Subject to patient enrollment, we 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.

DMD is a rare pediatric disorder caused by various mutations in the dystrophin gene that result in a lack of functional dystrophin in muscle fibers, leading to inexorable muscle weakness. DMD occurs almost exclusively in males, occurring in approximately 1 in 3,500 live male births. Based on this incidence rate, we estimate that DMD affects a total of approximately 15,000 patients in the United States and approximately 19,000 patients in the European Union.

There are 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. While corticosteroids have demonstrated efficacy in DMD patients, corticosteroids also can cause significant complications due to systemic toxicities, including growth suppression, reduction in bone strength and compromise of the immune system. A number of companies are developing therapies to treat DMD in patients with specific mutations in the dystrophin gene. Based on the prevalence of the specific mutations that the three most advanced of these product candidates are designed to address, these product candidates would be expected to be effective in an aggregate of approximately 26% of DMD patients. We believe that DMD patients, including those treated with these dystrophin therapies, will continue to require treatments to reduce muscle inflammation and enhance muscle regeneration.

Based on its mechanism of action in suppressing activated NF-κB, we believe that CAT-1004 has the potential to combine reduction of inflammation and muscle degeneration with positive effects on muscle regeneration, all of which may allow patients to retain muscle function longer. In addition, we believe that CAT-1004 has the potential to be effective in all DMD patients, regardless of the underlying mutation, and provide significant benefit to patients both as monotherapy and when used in combination with other therapies.

CAT-2000 Series

Our two other clinical-stage product candidates, CAT-2054 and CAT-2003, are members of our CAT-2000 series of molecules. We applied our SMART linker technology to engineer these molecules as SMART linker conjugates of the omega-3 fatty acid eicosapentaenoic acid, or EPA, and nicotinic

acid to modulate the Sterol Regulatory Element Binding Protein, or SREBP, pathway. EPA is a naturally occurring unsaturated fatty acid that has anti-inflammatory properties and beneficial effects on triglycerides. Nicotinic acid, which is also known as vitamin B3, is a naturally occurring essential vitamin that has beneficial lipid effects at high doses. Because we used different SMART linkers for CAT-2054 and CAT-2003, they possess different pharmacokinetic and biodistribution characteristics. CAT-2003, our first generation product candidate in the CAT-2000 series, is an orally administered molecule that modulates the SREBP pathway predominately in the intestine. CAT-2054, our second generation product candidate in the CAT-2000 series, is an orally administered molecule that modulates the SREBP pathway predominately in the liver.

SREBP is a master regulator of lipid metabolism, the processing of fats, triglycerides and cholesterol by the body, and controls the metabolism of both low density lipoprotein cholesterol, or LDL-C, and triglycerides. We believe that the CAT-2000 product candidates modulate the disease pathway by inhibiting the maturation of the SREBP protein, thereby reducing its activity and reducing the production of proteins involved in lipid metabolism.

We have focused our initial development efforts for the CAT-2000 series on lipid disorders such as hypercholesterolemia and hypertriglyceridemia because these disorders have clear short-term efficacy biomarkers, enabling us to rapidly and efficiently demonstrate proof of concept for our technology. In the CAT-2000 series, our development priority is CAT-2054 for the treatment of hypercholesterolemia, or elevated LDL-C, given what we believe is an attractive potential commercial opportunity in hypercholesterolemia and other SREBP-related metabolic disorders.

We are initially developing CAT-2054 for the treatment of hypercholesterolemia in patients for whom existing treatments are insufficient. Hypercholesterolemia is a disease that increases the risk of cardiovascular events. By modulating the SREBP pathway, CAT-2054 may inhibit production of important cholesterol metabolism proteins, such as proprotein convertase subtilisin kexin 9, or PCSK9; 3-hydroxy-3-methyl-glutaryl-CoA reductase, or HMG-CoA reductase; adenosine triphosphate citrate lyase, or ATP citrate lyase; as well as Niemann-Pick C1-like 1, or NPC1L1. In a clinical trial of our first generation SREBP modulator CAT-2003, we observed statistically significant reductions in LDL-C, suggesting an impact of SREBP modulation on cholesterol metabolism. Because the liver is the primary regulator of cholesterol metabolism, we specifically designed the SMART linker in CAT-2054 to deliver more of the intact conjugate to the liver than CAT-2003. We believe that CAT-2054, if approved, has the potential to be the first therapy to simultaneously modulate cholesterol synthesis, clearance and absorption. In January 2015, we initiated a Phase 1 clinical trial to assess the safety, tolerability and pharmacokinetics of CAT-2054 in healthy volunteers. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the trial. If the final results of this clinical trial are positive, we intend to initiate a Phase 2a clinical trial in patients with 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. We intend to seek to commercialize CAT-2054 through one or more collaborations.

Hypercholesterolemia is a major risk factor for cardiovascular disease, a leading cause of mortality and morbidity in the United States. Hypercholesterolemia is a complex disease involving redundant biological pathways that are tightly regulated and have built-in feedback mechanisms. Current treatment guidelines recognize lowering of LDL-C as a primary target for reducing the risk of cardiovascular disease.

Several of the lipid-lowering therapies currently available or in development target proteins in the SREBP pathway to lower LDL-C. Despite the availability of these drugs, many patients are unable to achieve their LDL-C goals. A 2011 report of the Centers for Disease Control and Prevention estimated that, of the 34 million adults in the United States receiving treatment for high LDL-C, 11 million had

uncontrolled LDL-C. Directly reducing active SREBP may have a significant benefit on LDL-C levels in circulation. SREBP modulators may work synergistically with inhibitors of proteins that are downstream of SREBP such as PCSK9, HMG-CoA reductase and ATP citrate lyase. In addition, SREBP modulators may substantially reduce feedback mechanisms that are activated by other classes of LDL-C lowering drugs, such as statins and ezetimibe. We believe that CAT-2054, if approved, has the potential to be the first therapy to simultaneously modulate cholesterol synthesis, clearance and absorption.

CAT-2003 is our first generation product candidate in the CAT-2000 series. We engineered CAT-2003 as an orally administered SMART linker conjugate of EPA and nicotinic acid to modulate the SREBP pathway. We designed CAT-2003 to target triglyceride levels in the blood. We have studied CAT-2003 for the treatment of multifactorial chylomicronemia syndrome, or MFC, and refractory severe hypertriglyceridemia, or rSHTG, diseases with niche patient populations with elevated triglycerides or hypertriglyceridemia. We have completed three Phase 2a trials with CAT-2003 in patient populations with hypertriglyceridemia. While we have chosen to prioritize CAT-2054 over CAT-2003, we believe that the clinical trial data for CAT-2003 support the utility of our SMART linker technology and the potential to treat lipid and metabolic disorders by modulating the SREBP pathway. We intend to pursue collaborations to conduct exploratory evaluation of CAT-2003 in other serious diseases that involve alterations in the SREBP pathway, such as nonalcoholic steatohepatitis and hepatocellular carcinoma, either to develop CAT-2003 as a product candidate or to support our development efforts for CAT-2054.

CAT-4001

CAT-4001, our most advanced preclinical product candidate, is a SMART linker conjugate of monomethyl fumarate and DHA. CAT-4001 is a small molecule that activates the Nrf2 pathway and inhibits activated NF-κB. Nrf2, or Nuclear factor (erythroid-derived 2)-like 2, is a gene transcription factor, a protein that works inside of cells to control the expression of genes, that controls the body's response to cellular stress and oxidative damage. We believe that CAT-4001 modulates the disease pathway by enhancing the movement of Nrf2 to the nucleus of the cells and inhibits NF-κB by reducing the movement of activated NF-κB to the nucleus of the cells. We are exploring CAT-4001 as a potential treatment for severe, rare neurodegenerative diseases, such as Friedreich's ataxia and amyotrophic lateral sclerosis, or ALS, two diseases of the central nervous system in which the Nrf2 and NF-κB pathways have been implicated. We plan to conduct additional preclinical evaluation of CAT-4001 in 2015, and if the results are positive we intend to advance CAT-4001 into investigational new drug application enabling studies in 2016.

SMART Linker Technology Platform

We have developed our SMART linker technology platform to create molecules that simultaneously modulate multiple biological targets within one or more related disease pathways. The linkers used in our technology platform are small chemicals designed to join two separate bioactives into a single conjugate molecule. In systemic circulation, our SMART linker conjugates are stable and inactive, potentially reducing off-target toxicities and side-effects. The conjugates are designed to be cleaved by specific enzymes exclusively within cells in order to release the two bioactives inside the cells. By releasing the bioactive components of the conjugate molecule only inside cells, the SMART linker allows the bioactives to reach their targets more efficiently and have greater efficacy than if the bioactives were dosed independently or in combination. The stability of our SMART linker conjugates outside of cells and the release of the bioactives exclusively within cells are differentiating features of our SMART linker technology platform.

We believe our SMART linker technology platform has the potential to:

- enhance activity on disease pathways through modulation of multiple biological targets;
- improve efficacy by matching the pharmacokinetics and tissue distribution of the component bioactives; and
- improve safety and tolerability by releasing the component bioactives only within cells.

Our Strategy

Our objective is to apply our proprietary SMART linker technology platform to discover, develop and commercialize novel, bi-functional therapeutics, with an initial focus on rare diseases and serious lipid disorders, either on our own or through collaborations. To achieve our goals, we are pursuing the following strategies:

- complete the development of CAT-1004 through registration for DMD;
- advance the development of CAT-2054;
- advance the development of CAT-4001;
- continue to apply our SMART linker technology platform; and
- maintain flexibility in commercializing and maximizing the value of our development programs.

Risks Associated with Our Business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk Factors" section of this prospectus immediately following this prospectus summary. These risks include the following:

- 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 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.
- 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 dependent on the successful development and commercialization of our most advanced product candidates.
- Our SMART linker technology platform may fail to help us discover and develop additional potential product candidates.
- Clinical drug development involves a lengthy and expensive process with an uncertain outcome.
- We face substantial competition from other pharmaceutical and biotechnology companies, and our operating results may suffer if we fail to compete effectively.
- 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 Corporate Information

We were incorporated under the laws of the State of Delaware on June 26, 2008 under the name Catabasis Pharmaceuticals, Inc. Our executive offices are located at One Kendall Square, Bldg. 1400E, Suite B14202, Cambridge, Massachusetts 02139, and our telephone number is (617) 349-1971. Our website address is www.catabasis.com. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

Implications of Being an Emerging Growth Company

As a company with less than $1 billion of revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we 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 intend to rely on exemptions from certain disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

THE OFFERING

Common stock offered	5,000,000 shares
Common stock to be outstanding immediately following this offering	14,547,796 shares
Over-allotment option	750,000 shares
Use of proceeds	We intend to use the net proceeds from this offering, together with our existing cash and cash equivalents, to fund our ongoing development of CAT-1004 and CAT-2054, as well as for working capital and other general corporate purposes. See the "Use of Proceeds" section in this prospectus for a more complete description of the intended use of proceeds from this offering.
Risk factors	You should read the "Risk Factors" section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
NASDAQ Global Market symbol	"CATB"

The number of shares of our common stock to be outstanding after this offering is based on 518,245 shares of our common stock outstanding as of April 30, 2015 and 9,029,551 additional shares of our common stock issuable upon the automatic conversion of all outstanding shares of our preferred stock upon the closing of this offering.

The number of shares of our common stock to be outstanding after this offering excludes:

- 59,405 shares of our common stock issuable upon the exercise of warrants outstanding as of April 30, 2015, at a weighted-average exercise price of $6.03 per share;
- 1,478,731 shares of our common stock issuable upon the exercise of stock options outstanding as of April 30, 2015, at a weighted-average exercise price of $5.46 per share;
- 25,942 shares of our common stock available for future issuance as of April 30, 2015 under our amended and restated 2008 equity incentive plan; and
- 1,068,287 and 182,352 additional shares of our common stock that will become available for future issuance in connection with this offering under our 2015 stock incentive plan and our 2015 employee stock purchase plan, respectively.

Unless otherwise indicated, all information in this prospectus assumes:

- a one-for-12.85 reverse stock split that was effected on June 11, 2015;
- no exercise of the outstanding options or warrants described above;
- no exercise by the underwriters of their option to purchase up to 750,000 additional shares of our common stock to cover over-allotments;
- the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 9,029,551 shares of our common stock upon the closing of this offering;
- the automatic conversion of warrants to purchase 315,688 shares of preferred stock into warrants to purchase 24,566 shares of common stock upon the closing of this offering and the related reclassification of our warrant liability to stockholders' (deficit) equity; and

- the restatement of our certificate of incorporation and the amendment and restatement of our bylaws upon the closing of this offering.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to approximately $15.0 million in shares of our common stock in this offering at the initial public offering price. At the initial public offering price of $12.00 per share, these stockholders would purchase up to an aggregate of approximately 1,250,000 of the 5,000,000 shares offered in this offering based on these indications of interest. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these potential purchasers and any of these potential purchasers could determine to purchase more, less or no shares in this offering.

SUMMARY FINANCIAL INFORMATION

You should read the following summary financial data together with our financial statements and the related notes appearing at the end of this prospectus and the "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations" sections of this prospectus. We have derived the statement of operations data for the years ended December 31, 2013 and 2014 and the balance sheet data as of December 31, 2014 from our audited financial statements appearing at the end of this prospectus. The statement of operations data for the three months ended March 31, 2014 and 2015 and the balance sheet data as of March 31, 2015 have been derived from our unaudited financial statements appearing at the end of this prospectus and have been prepared on the same basis as the audited financial statements. In the opinion of management, the unaudited financial data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information as of and for the periods presented. Our historical results are not necessarily indicative of results that should be expected in any future period, and our results for any interim period are not necessarily indicative of results that should be expected for any full year.

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
	(in thousands, except share and per share data)			
Statement of Operations Data:				
Operating expenses:				
Research and development	$ 13,994	$ 15,686	$ 3,096	$ 4,616
General and administrative	4,125	5,995	1,373	1,744
Total operating expenses	18,119	21,681	4,469	6,360
Loss from operations	(18,119)	(21,681)	(4,469)	(6,360)
Other income (expense):				
Other income (expense), net	1	3	—	9
Interest expense	—	(206)	—	(149)
Total other income (expense), net	1	(203)	—	(140)
Net loss and comprehensive loss	$ (18,118)	$ (21,884)	$ (4,469)	$ (6,500)
Net loss per share—basic and diluted	$ (47.80)	$ (51.56)	$ (11.29)	$ (13.14)
Weighted-average number of common shares used in net loss per share—basic and diluted	379,025	424,477	395,774	494,590
Pro forma net loss per share—basic and diluted (unaudited)		$ (2.59)		$ (0.75)
Weighted-average number of common shares used in pro forma net loss per share—basic and diluted (unaudited)		8,437,464		8,665,359

See Note 2 in the notes to our financial statements appearing at the end of this prospectus for a description of the method used to calculate basic and diluted net loss per share and unaudited pro forma basic and diluted net loss per share.

The following table sets forth summary balance sheet data as of March 31, 2015:

- on an actual basis;
- on a pro forma basis to give effect to (i) the conversion of all outstanding shares of our preferred stock into 9,029,551 shares of our common stock, and (ii) the conversion of our outstanding warrants to purchase 315,688 shares of preferred stock into warrants to purchase 24,566 shares of common stock, resulting in the reclassification of our warrant liability to stockholders' (deficit) equity, upon the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 5,000,000 shares of our common stock in this offering at the initial public offering price of $12.00 per

share after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

	As of March 31, 2015		
	Actual	Pro Forma	Pro Forma As Adjusted
		(in thousands)	
Balance Sheet Data:			
Cash and cash equivalents	$ 24,303	$ 24,303	$ 77,903
Total assets	26,725	26,725	80,325
Current liabilities	4,993	4,993	4,993
Notes payable, net of current portion and discount	8,151	8,151	8,151
Warrant liability	211	—	—
Convertible preferred stock	92,477	—	—
Accumulated deficit	(81,880)	(81,880)	(81,880)
Total stockholders' (deficit) equity	(79,201)	13,487	67,087

RISK FACTORS

Investing in our common stock involves a high degree of risk. Before you decide to invest in our common stock, you should consider carefully the risks described below, together with the other information contained in this prospectus, including our financial statements and the related notes appearing at the end of this prospectus. We believe the risks described below are the risks that are material to us as of the date of this prospectus. If any of the following risks actually occur, our business, financial condition, results of operations and future growth prospects could be materially and adversely affected. In these circumstances, the market price of our common stock could decline, and you may 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 $6.5 million for the three months ended March 31, 2015. As of March 31, 2015, we had an accumulated deficit of $81.9 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 a planned Phase 1/2 clinical trial of CAT-1004 for which we plan to initiate 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;
- 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 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, following the completion of this offering, 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 plan to use the net proceeds of this offering primarily to fund our ongoing research and development 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, the net proceeds of this offering and our existing cash and cash equivalents will not be sufficient to fund all of the efforts that we plan to undertake or to fund the completion of development of any of our product candidates. Accordingly, we will be required to obtain further funding through public or private equity offerings, debt financings, collaborations and licensing arrangements or other sources. With the exception of our credit facility with MidCap Financial Trust, or MidCap, Flexpoint MCLS SPV LLC, or Flexpoint, and Square 1 Bank, or Square 1, 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 the net proceeds from this offering, together with our existing cash and cash equivalents as of March 31, 2015, 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 the net proceeds from this offering, together with 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;
- 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. If we are unable to obtain sufficient capital in this offering, our business, financial condition and results of operations will be materially and adversely affected and we will need to obtain alternative financing or significantly modify our operational plans 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. Further, even if we successfully complete and receive the net proceeds from this offering, 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. 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, including purchasers of common stock in this offering, restrict our operations or require us to relinquish rights to our technologies or product candidates.

We expect that significant additional capital will be needed in the future to continue our planned operations. To the extent that we raise additional capital through the sale of common stock, convertible securities or other equity securities, our existing stockholders' ownership interest may be substantially diluted, and the terms of these securities could include liquidation or other preferences and anti-dilution protections that could adversely affect your rights as a common stockholder. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures, creating liens, redeeming stock or declaring dividends, that could adversely impact our ability to conduct our business. For example, our credit facility with MidCap, Flexpoint and Square 1 contains restrictive covenants that, among other things and subject to certain exceptions, prohibit us from transferring any of our material assets, exclusively licensing our intellectual property (subject to certain exceptions), merging with or acquiring another entity, entering into a transaction that would result in a change of control, incurring additional indebtedness, creating any lien on our property, making investments in third parties or redeeming stock or paying dividends. Future debt securities or other financing arrangements could contain similar or more restrictive negative covenants. In addition, securing additional financing could require a substantial amount of time and attention from our management and may divert a disproportionate amount of their attention away from day-to-day activities, which may adversely affect our management's ability to oversee the development of our product candidates.

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

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

As of March 31, 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 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;
- 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 factors or otherwise, 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 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.

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 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.

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;

- 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.

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 expect to initiate 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.

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

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

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

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

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

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

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

- the efficacy and safety of the product;
- the potential advantages of the product compared to alternative treatments;

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

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

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

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

We generally plan to seek to retain full commercialization rights 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 process of registration or late stage clinical development, including Eli Lilly, BioMarin 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, Eli Lilly 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.

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

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

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

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

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

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

exclusivity, the FDA may approve generic versions of such product three years after its date of approval. Manufacturers may seek to launch these generic products following the expiration of the applicable marketing exclusivity period, even if we still have patent protection for our product.

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

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

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

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

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

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

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

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

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

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

Although we maintain general liability insurance of $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.

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 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.

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

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

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

We currently have no manufacturing facilities and limited personnel with manufacturing experience. We rely on contract manufacturers to produce both drug substance and drug product required for our clinical trials. We plan to continue to rely upon contract manufacturers, and,

potentially collaboration partners, to manufacture commercial quantities of our products, if approved. Reliance on such third-party contractors entails risks, including:

- manufacturing delays if our third-party contractors give greater priority to the supply of other products over our product candidates or otherwise do not satisfactorily perform according to the terms of the agreements between us and them;
- the possible termination or nonrenewal of agreements by our third-party contractors at a time that is costly or inconvenient for us;
- the possible breach by the third-party contractors of our agreements with them;
- the failure of third-party contractors to comply with applicable regulatory requirements;
- the possible mislabeling of clinical supplies, potentially resulting in the wrong dose amounts being supplied or active drug or placebo not being properly identified;
- the possibility of clinical supplies not being delivered to clinical sites on time, leading to clinical trial interruptions, or of drug supplies not being distributed to commercial vendors in a timely manner, resulting in lost sales; and
- the possible misappropriation of our proprietary information, including our trade secrets and know-how.

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

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

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

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

these facilities are not approved for commercial manufacture, we may need to find alternative manufacturing facilities, which could result in delays in obtaining approval for the applicable product candidate.

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

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

Risks Related to Our Intellectual Property

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

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

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

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

Moreover, because the issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, our patents or pending patent applications may be challenged in the courts or patent

offices in the United States and abroad. For example, we may be subject to a third party preissuance submission of prior art to the U.S. Patent and Trademark Office, or USPTO, or become involved in post-grant review procedures, oppositions, derivations, reexaminations, inter partes review or interference proceedings, in the United States or elsewhere, challenging our patent rights or the patent rights of others. An adverse determination in any such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. In addition, given the amount of time required for the development, testing and regulatory review of new product candidates, patents protecting such candidates might expire before or shortly after such candidates are commercialized.

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

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

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

While we have obtained composition of matter patents with respect to our most advanced product candidates, we also rely on trade secret protection for certain aspects of technology platform, including certain aspects of our SMART linker 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 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 products 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.

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 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 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 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 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.

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;
- 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, 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

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.

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.

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.

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 and This Offering

No public market for our common stock currently exists, and an active trading market for our common stock may not develop.

Prior to this offering, there has been no public market for our common stock. The initial public offering price for our common stock was determined through negotiations with the underwriters. This price may not reflect the price at which investors in the market will be willing to buy and sell our shares following this offering. Although our common stock has been approved for listing on The NASDAQ Global Market, an active trading market for our shares may never develop or, if developed, be maintained following this offering. If an active market for our common stock does not develop or is not maintained, it may be difficult for you to sell shares you purchase in this offering without depressing the market price for the shares or at all. An inactive trading market may also impair our ability to raise capital to continue to fund operations by selling shares and may impair our ability to acquire other companies or technologies by using our shares as consideration.

If you purchase shares of common stock in this offering, you will suffer immediate dilution in the book value of your investment.

The initial public offering price of our common stock is substantially higher than the net tangible book value per share of our common stock. Therefore, if you purchase shares of our common stock in this offering, you will pay a price per share that substantially exceeds our net tangible book value per share after this offering. Based on the initial public offering price of $12.00 per share, you will experience immediate dilution of $7.49 per share, representing the difference between our pro forma net tangible book value per share after giving effect to this offering and the initial public offering price. Purchasers of common stock in this offering will have contributed approximately 39% of the aggregate price paid by all purchasers of our stock and will own approximately 34% of our common stock outstanding after this offering, excluding any shares of our common stock that they may have acquired prior to this offering. Furthermore, if the underwriters exercise their over-allotment option or our previously issued options and warrants to acquire common stock at prices below the initial public offering price are exercised, you will experience further dilution. For a further description of the dilution that you will experience immediately after this offering, see "Dilution."

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

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 not be able to sell your common stock at or above the initial public offering price and 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;
- 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 pharmaceutical 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 have broad discretion in the use of the net proceeds from this offering and may not use them effectively.

Our management will have broad discretion in the application of the net proceeds from this offering and could spend the proceeds in ways that do not improve our results of operations or enhance the value of our common stock. The failure by our management to apply these funds effectively could result in financial losses that could significantly harm our business, cause the price of our common stock to decline and delay the development of our product candidates. Pending their use, we may invest the net proceeds from this offering in a manner that does not produce income or that loses value.

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 this prospectus, we have not included 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.

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 becoming, and 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.

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. After this offering and after giving effect to the conversion of all outstanding shares of our preferred stock into 9,029,551 shares of our common stock upon the closing of this offering, we will have 14,547,796 shares of common stock outstanding based on the 518,245 shares of our common stock outstanding as of April 30, 2015 (or 15,297,796 shares if the underwriters exercise their over-allotment option in full). Of these shares, the 5,000,000 shares we are selling in this offering (or 5,750,000 shares if the underwriters exercise their over-allotment option in full) may be resold in the public market immediately, unless purchased by our affiliates. The remaining 9,547,796 shares are currently restricted under securities laws or as a result of lock-up or other agreements, but will be able to be sold after this offering as described in the "Shares Eligible for Future Sale" section of this prospectus. Moreover, after this offering, holders of an aggregate of 9,029,551 shares of our common stock, along with the holders of warrants to purchase 24,566 shares of common stock, will have rights, subject to conditions, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. 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 described in the "Underwriting" section of this prospectus.

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.

After this offering, our executive officers, directors and principal stockholders, if they choose to act together, will continue to have the ability to control all matters submitted to stockholders for approval.

Upon the closing, our executive officers and directors, combined with our stockholders who owned more than 5% of our outstanding common stock before this offering and their affiliates will, in the aggregate, beneficially own shares representing approximately 58.9% of our capital stock. 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.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to approximately $15.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements of commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these potential purchasers and any of these potential purchasers could determine to purchase more, less or no shares in this offering. Accordingly, the foregoing discussion does not reflect any purchases by these potential purchasers.

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

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

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

SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This prospectus contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this prospectus, 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," "predict," "project," "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;
- our plans to have up to three product candidates in clinical trials in 2015;
- 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 expectations related to the use of proceeds from this offering;
- 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 prospectus, 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.

You should read this prospectus and the documents that we have filed as exhibits to the registration statement of which this prospectus is a part completely and with the understanding that our actual future results may be materially different from what we expect. We do not assume any obligation to update any forward-looking statements, whether as a result of new information, future events or otherwise, except as required by law.

USE OF PROCEEDS

We estimate that the net proceeds from our issuance and sale of 5,000,000 shares of our common stock in this offering will be approximately $53.6 million, based on the initial public offering price of $12.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. If the underwriters exercise in full their over-allotment option, we estimate that the net proceeds from this offering will be approximately $62.0 million.

As of March 31, 2015, we had cash and cash equivalents of approximately $24.3 million. We currently estimate that we will use the net proceeds from this offering, together with our cash and cash equivalents, as follows:

- approximately $30.0 million for the clinical development of CAT-1004;
- approximately $15.0 million for the clinical development of CAT-2054; and
- the remainder for other product candidates, working capital and other general corporate purposes.

This expected use of the net proceeds from this offering and our existing cash and cash equivalents represents our intentions based upon our current plans and business conditions. The amounts and timing of our actual expenditures may vary significantly depending on numerous factors, including the progress of our development and commercialization efforts, the status of and results from clinical trials, any collaborations that we may enter into with third parties for our product candidates and any unforeseen cash needs. As a result, our management will retain broad discretion over the allocation of the net proceeds from this offering. We have no current agreements, commitments or understandings for any material acquisitions or licenses of any products, businesses or technologies.

Based on our planned use of the net proceeds from this offering and our existing cash and cash equivalents, we estimate that such funds will be sufficient to enable us to complete our planned Phase 1/2 clinical trial of CAT-1004 and our planned Phase 2 development of CAT-2054, and to fund our operating expenses, debt service and capital expenditure requirements at least through 2016. We have based this estimate on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. We do not expect that the net proceeds from this offering and our existing cash and cash equivalents will be sufficient to enable us to fund the completion of development of any of our product candidates.

Pending our use of the net proceeds from this offering, we intend to invest the net proceeds in short-term, investment-grade, interest-bearing instruments and U.S. government securities.

DIVIDEND POLICY

We have never declared nor paid cash dividends on our common stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. We do not intend to pay cash dividends in respect of our common stock in the foreseeable future. In addition, our ability to pay cash dividends on our common stock is prohibited by the covenants of our credit facility with MidCap Financial Trust, Flexpoint MCLS SPV LLC and Square 1 Bank.

CAPITALIZATION

The following table sets forth our capitalization as of March 31, 2015:

- on an actual basis;
- on a pro forma basis to give effect to (i) the conversion of all outstanding shares of our preferred stock into 9,029,551 shares of our common stock, (ii) the conversion of our outstanding warrants to purchase 315,688 shares of preferred stock into warrants to purchase 24,566 shares of common stock, resulting in the reclassification of our warrant liability to stockholders' (deficit) equity, and (iii) the filing and effectiveness of our restated certificate of incorporation, all upon the closing of this offering; and
- on a pro forma as adjusted basis to give further effect to our issuance and sale of 5,000,000 shares of our common stock in this offering at the initial public offering price of $12.00 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table together with our financial statements and the related notes appearing at the end of this prospectus and the sections of this prospectus titled "Selected Financial Data" and "Management's Discussion and Analysis of Financial Condition and Results of Operations."

	As of March 31, 2015		
	Actual	Pro Forma	Pro Forma As Adjusted
	(in thousands, except share and per share data)		
Notes payable, net of current portion and discount	$ 8,151	$ 8,151	$ 8,151
Warrant liability	211	—	—
Series A convertible preferred stock, par value $0.001 per share; 68,837,703 shares authorized, issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	47,898	—	—
Series B convertible preferred stock, par value $0.001 per share; 56,026,590 shares authorized, 47,192,536 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	44,579	—	—
Preferred stock, par value $0.001 per share; no shares authorized, issued or outstanding actual; 5,000,000 shares authorized, no shares issued or outstanding pro forma and pro forma as adjusted	—	—	—
Common stock, par value $0.001 per share; 155,000,000 shares authorized, 518,245 shares issued and outstanding, actual; 150,000,000 shares authorized, pro forma and pro forma as adjusted; 9,547,796 shares issued and outstanding, pro forma; 14,547,796 shares issued and outstanding, pro forma as adjusted	1	10	15
Additional paid-in capital	2,678	95,357	148,952
Accumulated deficit	(81,880)	(81,880)	(81,880)
Total stockholders' (deficit) equity	(79,201)	13,487	67,087
Total capitalization	$ 21,638	$ 21,638	$ 75,238

The table above does not include:

- 59,405 shares of our common stock issuable upon the exercise of warrants outstanding as March 31, 2015, at a weighted-average exercise price of $6.03 per share;
- 1,388,218 shares of our common stock issuable upon the exercise of stock options outstanding as of March 31, 2015, at a weighted-average exercise price of $5.08 per share;

- 116,455 shares of our common stock available for future issuance as of March 31, 2015 under our amended and restated 2008 equity incentive plan; and

- 1,068,287 and 182,352 additional shares of our common stock that will become available for future issuance in connection with this offering under our 2015 stock incentive plan and our 2015 employee stock purchase plan, respectively.

DILUTION

If you invest in our common stock in this offering, your ownership interest will be diluted immediately to the extent of the difference between the public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book value (deficit) as of March 31, 2015 was $(80.7) million, or $(155.63) per share of our common stock. Our historical net tangible book value (deficit) is the amount of our total tangible assets less our total liabilities and preferred stock, which is not included within our stockholders' (deficit) equity. Historical net tangible book value (deficit) per share represents historical net tangible book value (deficit) divided by the 518,245 shares of our common stock outstanding as of March 31, 2015.

Our pro forma net tangible book value as of March 31, 2015 was $12.0 million, or $1.26 per share of our common stock. Pro forma net tangible book value represents the amount of our total tangible assets less our total liabilities, after giving effect to (i) the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 9,029,551 shares of our common stock upon the closing of this offering, and (ii) the conversion of warrants to purchase 315,688 shares of preferred stock into warrants to purchase 24,566 shares of common stock resulting in the reclassification of our warrant liability to stockholders' (deficit) equity. Pro forma net tangible book value per share represents pro forma net tangible book value divided by the total number of shares outstanding as of March 31, 2015, after giving effect to the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 9,029,551 shares of our common stock upon the closing of this offering.

After giving effect to our issuance and sale of 5,000,000 shares of our common stock in this offering at the initial public offering price of $12.00 per share, and after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma as adjusted net tangible book value as of March 31, 2015 would have been $65.6 million, or $4.51 per share. This represents an immediate increase in pro forma as adjusted net tangible book value per share of $3.25 to existing stockholders and immediate dilution of $7.49 in pro forma as adjusted net tangible book value per share to new investors purchasing common stock in this offering. Dilution per share to new investors is determined by subtracting pro forma as adjusted net tangible book value per share after this offering from the initial public offering price per share paid by new investors. The following table illustrates this dilution on a per share basis:

Initial public offering price per share		$ 12.00
Historical net tangible book value (deficit) per share as of March 31, 2015	$ (155.63)	
Increase per share attributable to the conversion of outstanding preferred stock and the reclassification of the warrant liability	156.89	
Pro forma net tangible book value per share as of March 31, 2015	1.26	
Increase in net tangible book value per share attributable to new investors	3.25	
Pro forma as adjusted net tangible book value per share after this offering		4.51
Dilution per share to new investors		$ 7.49

If the underwriters exercise their over-allotment option in full, the pro forma as adjusted net tangible book value will increase to $4.84 per share, representing an immediate increase to existing stockholders of $0.33 per share and an immediate dilution of $7.16 per share to new investors. If any shares are issued upon exercise of outstanding options or outstanding warrants, you will experience further dilution.

The following table summarizes, on a pro forma as adjusted basis as of March 31, 2015, after giving effect to the conversion of all of our outstanding preferred stock into common stock, the differences between the number of shares of common stock purchased from us, the total consideration paid to us and the average price per share paid by existing stockholders and by new investors purchasing shares of common stock in this offering. The calculation below is based on the initial public offering price of $12.00 per share before deducting the underwriting discounts and commissions and estimated offering expenses payable by us:

	Shares Purchased		Total Consideration		Average Price Per Share
	Number	Percent	Amount	Percent	
Existing stockholders	9,547,796	66%	$ 93,124,554	61%	$ 9.75
New investors	5,000,000	34	60,000,000	39	12.00
Total	14,547,796	100%	$ 153,124,554	100%	

The number of shares purchased from us by existing stockholders is based on 9,547,796 shares of our common stock outstanding as of March 31, 2015, after giving effect to the automatic conversion of all of our outstanding shares of preferred stock into 9,029,551 shares of common stock upon the closing of this offering, and excludes:

- 59,405 shares of our common stock issuable upon the exercise of warrants outstanding as of March 31, 2015 at a weighted-average exercise price of $6.03 per share;
- 1,388,218 shares of our common stock issuable upon the exercise of stock options outstanding as of March 31, 2015, at a weighted-average exercise price of $5.08 per share;
- 116,455 additional shares of our common stock available for future issuance as of March 31, 2015 under our amended and restated 2008 equity incentive plan; and
- 1,068,287 and 182,352 additional shares of our common stock that will become available for future issuance in connection with this offering under our 2015 stock incentive plan and our 2015 employee stock purchase plan, respectively.

If the underwriters exercise their over-allotment option in full, the following will occur:

- the percentage of shares of our common stock held by existing stockholders will decrease to 62% of the total number of shares of our common stock outstanding after this offering; and
- the number of shares of our common stock held by new investors will increase to 5,750,000, or 38% of the total number of shares of our common stock outstanding after this offering.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to approximately $15.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these existing stockholders and any of these existing stockholders could determine to purchase more, less or no shares in this offering. The foregoing discussion and tables do not reflect any potential purchases by these existing stockholders or their affiliated entities. If these existing stockholders are allocated and purchase all of the shares in which they have indicated an interest in purchasing, our existing stockholders would hold 74% (71% if the underwriters exercise their over-allotment option in full) of the total number of shares of our common stock outstanding after this offering and our new investors would hold 26% (29% if the underwriters exercise their over-allotment option in full) of the total number of shares of our common stock outstanding after this offering.

SELECTED FINANCIAL DATA

You should read the following selected financial data together with our financial statements and the related notes appearing at the end of this prospectus and the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this prospectus. We have derived the statement of operations data for the years ended December 31, 2013 and 2014 and the balance sheet data as of December 31, 2013 and 2014 from our audited financial statements appearing at the end of this prospectus. The statement of operations data for the three months ended March 31, 2014 and 2015 and the balance sheet data as of March 31, 2015 have been derived from our unaudited financial statements appearing at the end of this prospectus and have been prepared on the same basis as the audited financial statements. In the opinion of management, the unaudited financial data reflects all adjustments, consisting only of normal recurring adjustments, necessary for a fair presentation of the financial information as of and for the periods presented. Our historical results are not necessarily indicative of results that should be expected in any future period, and our results for any interim period are not necessarily indicative of results that should be expected for any full year.

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
	(in thousands, except share and per share data)			
Statement of Operations Data:				
Operating expenses:				
Research and development	$ 13,994	$ 15,686	$ 3,096	$ 4,616
General and administrative	4,125	5,995	1,373	1,744
Total operating expenses	18,119	21,681	4,469	6,360
Loss from operations	(18,119)	(21,681)	(4,469)	(6,360)
Other income (expense):				
Other income, net	1	3	—	9
Interest expense	—	(206)	—	(149)
Total other income (expense), net	1	(203)	—	(140)
Net loss and comprehensive loss	$ (18,118)	$ (21,884)	$ (4,469)	$ (6,500)
Net loss per share—basic and diluted	$ (47.80)	$ (51.56)	$ (11.29)	$ (13.14)
Weighted-average number of common shares used in net loss per share —basic and diluted	379,025	424,477	395,774	494,590
Pro forma net loss per share—basic and diluted (unaudited)		$ (2.59)		$ (0.75)
Weighted-average number of common shares used in pro forma net loss per share—basic and diluted (unaudited)		8,437,464		8,665,359

See Note 2 in the notes to our financial statements appearing at the end of this prospectus for a description of the method used to calculate basic and diluted net loss per share and unaudited pro forma basic and diluted net loss per share.

	As of December 31,		As of March 31,
	2013	2014	2015
	(in thousands)		
Balance Sheet Data:			
Cash and cash equivalents	$ 30,474	$ 14,668	$ 24,303
Total assets	31,002	15,964	26,725
Current liabilities	2,930	4,234	4,993
Notes payable, net of current portion and discount	—	4,439	8,151
Warrant liability	—	108	211
Convertible preferred stock	80,146	80,146	92,477
Accumulated deficit	(53,496)	(75,380)	(81,880)
Total stockholders' deficit	(52,184)	(73,053)	(79,201)

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 our financial statements and the related notes and other financial information included elsewhere in this prospectus. Some of the information contained in this discussion and analysis or set forth elsewhere in this prospectus, including information with respect to our plans and strategy for our business, includes forward-looking statements that involve risks and uncertainties. You should review the "Risk Factors" section of this prospectus for a discussion of important factors that could cause actual results to differ materially from the results described in or implied by the forward-looking statements contained in the following discussion and analysis.

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. Our initial 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 target therapeutic areas and specific diseases with significant unmet medical need where we believe we will have a competitive advantage. We seek to develop therapies that modulate multiple targets in the disease pathway.

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 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. Preliminary data are available 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-κB 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, organizing and staffing our company, business planning, raising capital, and providing general and administrative support for these operations. To date, we have financed our operations primarily through private placements of our preferred stock and a debt financing. From our inception through March 31, 2015, we have raised an aggregate of $103.1 million, of which

$92.9 million consisted of gross proceeds from private placements of preferred stock and $10.0 million consisted of gross proceeds from a secured debt financing.

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. Our net losses were $18.1 million and $21.9 million for the years ended December 31, 2013 and 2014, respectively, and $6.5 million for the three months ended March 31, 2015. As of March 31, 2015, we had an accumulated deficit of $81.9 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 CAT-1004 and our 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; and hire additional personnel, such as clinical, regulatory, quality control and scientific personnel. In addition, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company.

Financial Operations Overview

Revenue

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

Research and Development Expenses

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

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

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

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

- 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-κB pathway at multiple points. NF-κB, 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 plan to initiate 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. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the trial. If the final results of this clinical trial are positive, 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.

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:

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
	(in thousands)			
CAT-1004	$ 108	$ 879	$ 12	$ 777
CAT-2054	652	3,208	478	1,033
CAT-2003	6,727	3,807	607	351
Other research and platform programs	680	1,276	308	468
Costs not directly allocated to programs:				
Employee expenses including cash compensation, benefits and share-based compensation	3,992	4,575	1,172	1,443
Facilities	754	733	182	207
Consultants and professional expenses, including share-based compensation	605	736	217	211
Other	476	472	120	126
Total costs not directly allocated to programs	5,827	6,516	1,691	1,987
Total research and development expenses	$ 13,994	$ 15,686	$ 3,096	$ 4,616

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;
- 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.

Critical Accounting Policies and Estimates

This discussion and analysis of our financial condition and results of operations is based on our financial statements, which we have prepared in accordance with United States generally accepted

accounting principles. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements, as well as the reported amounts of expenses during the reporting periods. On an ongoing basis, we evaluate our estimates and judgments, including those described in greater detail below. We base our estimates on historical experience and on various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying value of assets and liabilities that are not readily apparent from other sources. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in the notes to our financial statements included elsewhere in this prospectus, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our financial condition and results of operations.

Accrued Research and Development Expenses

As part of the process of preparing our financial statements, we are required to estimate our accrued expenses. This process involves reviewing quotations and contracts, identifying services that have been performed on our behalf and estimating the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of the actual cost. The majority of our service providers invoice us monthly in arrears for services performed or when contractual milestones are met. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers and make adjustments if necessary. The significant estimates in our accrued research and development expenses include the costs incurred for services performed by CROs in connection with research and development activities for which we have not yet been invoiced.

We base our expenses related to CROs on our estimates of the services received and efforts expended pursuant to quotes and contracts with CROs that conduct research and development on our behalf. The financial terms of these agreements are subject to negotiation, vary from contract to contract and may result in uneven payment flows. There may be instances in which payments made to our CROs will exceed the level of services provided and result in a prepayment of the research and development expense. In accruing service fees, we estimate the time period over which services will be performed and the level of effort to be expended in each period. If the actual timing of the performance of services or the level of effort varies from our estimate, we adjust the accrual or prepaid expense accordingly. Although we do not expect our estimates to be materially different from amounts actually incurred, if our estimates of the status and timing of services performed differ from the actual status and timing of services performed, it could result in us reporting expense amounts that are too high or too low in any particular period. To date, there have been no material differences between our estimates of such expenses and the amounts actually incurred.

Stock-Based Compensation

We measure stock-based awards granted to employees and members of the board of directors at fair value on the date of grant and recognize the corresponding stock-based compensation expense of those awards, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award. We have historically granted stock options with exercise prices equivalent to the fair value of our common stock as of the date of grant.

We measure other stock-based awards granted to non-employees at fair value as the awards vest and recognize the resulting value as expense during the period the related services are rendered. At the end of each financial reporting period prior to completion of the service, we re-measure the unvested portion of these awards.

We estimate the fair value of each stock option grant using the Black-Scholes option-pricing model. We historically have been a private company and lack company-specific historical and implied volatility information. Therefore, we estimate our expected volatility based on the historical volatility of a representative group of publicly traded biopharmaceutical companies and expect to continue to do so until we have adequate historical data regarding the volatility of our traded stock price. We determine the expected term of our options utilizing the "simplified" method for awards that qualify as "plain-vanilla" options, while we determine the expected term of other nonemployee options based on the contractual term of the options. We determine the risk-free interest rate by reference to the U.S. Treasury yield curve in effect at the time of grant of the award for time periods approximately equal to the expected term of the award. We assume an expected dividend yield of zero because we have never paid cash dividends and do not expect to pay any cash dividends in the foreseeable future.

We estimated the fair value of stock options granted using the Black-Scholes option-pricing model based on the assumptions noted in the following table:

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
Risk-free interest rate	0.92 - 2.03%	1.71 - 3.01%	1.96 - 3.01%	1.54 - 2.11%
Expected dividend yield	—	—	—	—
Expected term (in years)	6.25 - 10.0	6.25 - 10.0	6.25 - 10	6.25 - 10
Expected volatility	75.0 - 81.5%	75.2 - 83.4%	75.2 - 82.8%	76.5 - 84.0%

These assumptions represented our best estimates, but the estimates involve inherent uncertainties and the application of our judgment. As a result, if factors change and we use significantly different assumptions or estimates, our stock-based compensation expense could be materially different. We recognize stock-based compensation expense for only the portion of awards that are expected to vest. In developing a forfeiture rate estimate for pre-vesting forfeitures, we have considered our historical experience of actual forfeitures. If our future actual forfeiture rate is materially different from our estimate, our stock-based compensation expense could be significantly different from what we have recorded in the prior periods. However, estimates will not be necessary to determine the fair value of new awards once our common stock begins to be publicly traded.

The following table summarizes the classification of our stock-based compensation expense recognized in our statements of operations:

	Year Ended December 31,		Three Months Ended March 31, 2015	
	2013	2014	2014	2015
	(in thousands)			
Research and development expenses	$ 224	$ 434	$ 81	$ 168
General and administrative expenses	119	463	65	133
	$ 343	$ 897	$ 146	$ 301

Valuations of Common Stock

Our board of directors determines the fair value of our common stock on each date of grant, with input from management. Due to the absence of a public trading market for our common stock, our

board of directors' determination of the fair value of our common stock has historically been performed using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants Audit and Accounting Practice Aid Series: *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. We performed contemporaneous valuations, with the assistance of a third-party specialist, as of October 31, 2013, December 31, 2014 and March 17, 2015. For financial reporting purposes, we also performed common stock valuations retrospectively, with the assistance of a third-party specialist, as of April 1, 2014 and August 28, 2014. Our board of directors has considered various objective and subjective factors, along with input from management, to determine its best estimate of the fair value of our common stock as of each grant date, including the following:

- the prices at which we sold shares of preferred stock and the superior rights and preferences of the preferred stock relative to our common stock;
- the progress of our research and development programs, including the status of clinical trials for our product candidates;
- our stage of development and business strategy;
- our financial condition, including cash on hand and borrowings under our credit facility;
- our historical and forecasted performance and operating results;
- the composition of, and changes to, our management team and board of directors;
- the lack of an active public market for our common stock and our preferred stock;
- the likelihood of achieving a liquidity event such as a sale of our company or an initial public offering, or IPO, given prevailing market conditions;
- the analysis of initial public offerings and the market performance of similar companies in the biopharmaceutical industry;
- external market conditions affecting the biopharmaceutical industry; and
- trends within the biopharmaceutical industry.

Historically, the dates of our contemporaneous valuations have not always coincided with the dates of our stock-based compensation grants. In determining the exercise prices of the options granted, our board of directors considered, among other things, the most recent contemporaneous or retrospective valuations of our common stock and our assessment of additional objective and subjective factors we believed were relevant as of the grant date. The additional factors considered when determining any changes in fair value between the most recent contemporaneous valuation, or if available the most recent retrospective valuation, and the grant dates included, when available, the prices paid in recent transactions involving our equity securities, as well as our stage of development, our operating and financial performance and current business conditions.

There are significant judgments and estimates inherent in the determination of the fair value of our common stock, including the contemporaneous and retrospective valuations. These judgments and estimates include assumptions regarding our future operating performance, the time to completing an IPO or other liquidity event and the determinations of the appropriate valuation methods. If we had made different assumptions, our stock-based compensation expense, net loss and net loss per common share could have been significantly different.

Valuation Methodologies

Our common stock valuations were prepared using a hybrid of the option-pricing method, or OPM, and the probability-weighted expected return method, or PWERM.

OPM. The OPM treats each class of common stock and preferred stock as call options on the total equity value of a company, with exercise prices based on the value thresholds at which the allocation among the various holders of a company's securities changes. Under this method, the common stock has value only if the funds available for distribution to stockholders exceed the value of the preferred stock liquidation preference at the time of a liquidity event, such as a strategic sale, merger or IPO. The common stock is modeled as a call option on the underlying equity value at a predetermined exercise price. In the model, the exercise price is based on a comparison with the total equity value rather than, as in the case of a regular call option, a comparison with a per share stock price. Thus, common stock is considered to be a call option with a claim on the enterprise at an exercise price equal to the remaining value immediately after the preferred stock liquidation preference is paid.

The OPM uses the Black-Scholes option-pricing model to price the call options. This model defines the securities' fair values as functions of the current fair value of a company and uses assumptions such as the anticipated timing of a potential liquidity event and the estimated volatility of the equity securities. The aggregate value of the common stock derived from the OPM is then divided by the number of shares of common stock outstanding to arrive at the per share value.

We used the OPM backsolve approach to estimate enterprise value under the OPM. The OPM backsolve approach uses the OPM to calculate the implied equity value based on recent sales of the company's securities. For the OPM, we based our assumed volatility factor on the historical trading volatility of our publicly traded peer companies. At each valuation date, we determined the appropriate volatility to be used, considering such factors as our expected time to a liquidity event and our stage of development.

To derive the fair value of our common stock using the OPM, we calculated the proceeds to the common stockholders based on the preferences and priorities of the preferred and common stock. We then applied a discount for lack of marketability to the common stock to account for the lack of access to an active public market.

PWERM. The PWERM is a scenario-based methodology that estimates the fair value of common stock based upon an analysis of future values for the company, assuming various outcomes. The common stock value is based on the probability-weighted present value of expected future investment returns considering each of the possible outcomes available as well as the rights of each class of stock. The future value of the common stock under each outcome is discounted back to the valuation date at an appropriate risk-adjusted discount rate and probability weighted to arrive at an indication of value for the common stock. A discount for lack of marketability is then applied to the common stock to account for the lack of access to an active public market.

For our common stock valuations as of October 31, 2013, April 1, 2014, August 28, 2014, December 31, 2014 and March 17, 2015, we used a hybrid of the OPM and PWERM and considered two types of future event scenarios: an IPO and a sale transaction. We valued the IPO scenario using the OPM backsolve approach for the October 31, 2013 valuation. We used the guideline public company method, which includes comparisons to publicly traded companies in our industry that recently completed IPOs, for the April 1, 2014, August 28, 2014, December 31, 2014 and March 17, 2015 valuations. We valued the sale scenario using the OPM backsolve approach. Our board of directors determined the relative probability of each type of future event scenario based on an analysis of market conditions at the time, including then-current IPO valuations of similarly situated companies, and expectations as to the timing and likely prospects of the future-event scenarios.

To derive the fair value of the common stock for each scenario using the hybrid PWERM and OPM, we calculated the proceeds to the common stockholders based on the preferences and priorities of the preferred and common stock. We then applied a discount for lack of marketability to the common stock to account for the lack of access to an active public market.

Option Grants

The following table summarizes by grant date the number of shares of common stock subject to options granted between January 1, 2014 and May 31, 2015, the per share exercise price of the options, the fair value of the common stock underlying the options on the date of grant and the per share estimated fair value of the options. For financial reporting purposes, the value of the April 1, 2014 valuation has been applied retrospectively to our March 19, 2014 option grants and the value of the October 28, 2014 valuation has been applied retrospectively to our August 28, 2014, October 21, 2014 and November 5, 2014 option grants.

Grant Date	Number of Common Shares Underlying Options Granted	Per Share Exercise Price of Options(1)	Fair Value of Common Stock on Grant Date(1)	Retrospective Fair Value Per Share on Grant Date(2)
March 19, 2014	434,321	$ 6.81	$ 6.81	$ 7.20
August 28, 2014	56,595	$ 7.20	$ 7.20	$ 7.71
October 21, 2014	54,474	$ 7.20	$ 7.20	$ 7.71
November 5, 2014	3,891	$ 7.20	$ 7.20	$ 7.71
February 12, 2015	137,662	$ 9.51	$ 9.51	—
March 26, 2015	73,236	$ 11.05	$ 11.05	—
April 30, 2015	94,162	$ 11.05	$ 11.05	—

(1) Represents the determination by our board of directors of the fair value of our common stock on the date of grant, taking into consideration the various objective and subjective factors described above.

(2) The fair value of common stock at the grant date was adjusted in connection with a retrospective fair value assessment for financial reporting purposes.

JOBS Act

In April 2012, the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, was enacted. Section 107 of the JOBS Act provides that an "emerging growth company," or EGC, can take advantage of the extended transition period for complying with new or revised accounting standards. Thus, an EGC can 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 extended transition period and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

We are in the process of evaluating the benefits of relying on other exemptions and reduced reporting requirements under the JOBS Act. Subject to certain conditions, as an EGC, we intend to rely on certain of these exemptions, including exemptions from the requirement to provide an auditor's attestation report on our system of internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and from 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, known as the auditor discussion and analysis. We will remain an EGC until the earlier of: the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; the last day of the fiscal year following the fifth anniversary of the date of the completion of this offering; the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission.

Results of Operations

Comparison of Three Months Ended March 31, 2014 and 2015

The following table summarizes our results of operations for the three months ended March 31, 2014 and 2015, together with the dollar increase or decrease in those items:

	Three Months Ended March 31,		
	2014	2015	Change
		(in thousands)	
Operating expenses:			
Research and development	$ 3,096	$ 4,616	$ 1,520
General and administrative	1,373	1,744	371
Total operating expenses	4,469	6,360	1,891
Loss from operations	(4,469)	(6,360)	(1,891)
Other expense	—	(140)	(140)
Net loss	$ (4,469)	$ (6,500)	$ (2,031)

Research and Development Expenses

Research and development expenses increased by $1.5 million to $4.6 million for the three months ended March 31, 2015 from $3.1 million for the three months ended March 31, 2014, an increase of 49%. The increase in research and development expenses was partially attributable to a net increase of $1.2 million in direct program costs, reflecting an increase of $0.8 million for CAT-1004 driven by activities in preparation for the start of a Phase 1/2 clinical trial, an increase of $0.5 million for CAT-2054 driven by the start of a Phase 1 clinical trial, and an increase of $0.6 million in our general research and platform programs, which were partially offset by a decrease of $0.3 million in CAT-2003 clinical trial, manufacturing and preclinical development costs due to the completion of two Phase 2 clinical trials in late 2013 and early 2014. In addition, the costs related to internal research and development increased by $0.3 million, primarily attributable to increased research and development employee headcount.

General and Administrative Expenses

General and administrative expenses increased by $0.4 million to $1.7 million for the three months ended March 31, 2015 from $1.4 million for the three months ended March 31, 2014, an increase of 21%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $0.2 million associated with hiring additional senior personnel, and an increase of $0.2 million in consulting expense primarily due to recruiting for two senior business development positions.

Other Expense

Other expense consists of interest expense, which increased by $0.1 million for the three months ended March 31, 2015 due to the interest expense on our credit facility which we entered into in August 2014.

Comparison of Years Ended December 31, 2013 and 2014

The following table summarizes our results of operations for the years ended December 31, 2013 and 2014, together with the dollar change in those items:

	Year Ended December 31,		
	2013	2014	Change
	(in thousands)		
Operating expenses:			
Research and development	$ 13,994	$ 15,686	$ 1,692
General and administrative	4,125	5,995	1,870
Total operating expenses	18,119	21,681	3,562
Loss from operations	(18,119)	(21,681)	(3,562)
Other expense	1	(203)	(204)
Net loss	$ (18,118)	$ (21,884)	$ (3,766)

Research and Development Expenses

Research and development expenses increased by $1.7 million to $15.7 million for the year ended December 31, 2014 from $14.0 million for the year ended December 31, 2013, an increase of 12%. The increase in research and development expenses was partially attributable to a net increase of $1.0 million in direct program costs, reflecting an increase of $2.6 million for CAT-2054 manufacturing and preclinical development costs associated with IND-enabling studies, an increase of $0.6 million in our general research and platform programs and an increase of $0.8 million for CAT-1004 manufacturing and preclinical development costs, which were partially offset by a decrease of $3.0 million in CAT-2003 clinical trial, manufacturing and preclinical development costs due to the completion of two Phase 2 clinical trials in late 2013 and early 2014. In addition, the costs related to internal research and development increased by $0.7 million, primarily attributable to stock-based compensation expense.

General and Administrative Expenses

General and administrative expenses increased by $1.9 million to $6.0 million for the year ended December 31, 2014 from $4.1 million for the year ended December 31, 2013, an increase of 46%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $0.7 million and increased professional and consulting fees of $0.9 million. The $0.7 million increase in employee costs consisted of an increase of $0.6 million in salaries and benefits and an increase of $0.3 million in stock-based compensation expense, partially offset by a decrease of $0.2 million in travel expense. The increase in employee costs was primarily due to the hiring of additional members of our management team. The $0.9 million increase in professional and consulting fees primarily consisted of an increase of $0.4 million in intellectual property legal fees and an increase of $0.4 million in consulting expense associated with market studies for our product candidates.

Other Expense

Other expense consists primarily of interest expense, which increased by $0.2 million for the year ended December 31, 2014, due to the interest expense on our credit facility which we entered into in August 2014.

Liquidity and Capital Resources

Sources of Liquidity

From our inception through March 31, 2015, we have raised an aggregate of $103.1 million, of which $92.9 million consisted of gross proceeds from private placements of preferred stock and $10.0 million consisted of gross proceeds from a secured debt financing. As of March 31, 2015, we had $24.3 million in cash and cash equivalents.

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 (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 will be 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. 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 March 31, 2015, 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.

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.

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 years ended December 31, 2013 and 2014 and the three months ended March 31, 2014 and 2015:

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
	(in thousands)			
Net cash used in operating activities	$ (16,366)	$ (20,412)	$ (4,603)	$ (7,228)
Net cash used in investing activities	(43)	(228)	(14)	(25)
Net cash provided by financing activities	41,449	4,834	19	16,888
Net increase (decrease) in cash and cash equivalents	$ 25,040	$ (15,806)	$ (4,598)	9,635

Net Cash Used in Operating Activities

Net cash used in operating activities was $7.2 million for the three months ended March 31, 2015 and consisted primarily of a net loss of $6.5 million adjusted for non-cash items, including stock-based compensation expense of $0.3 million and depreciation and amortization expense of $0.1 million, and a net increase in operating assets of $1.1 million, which resulted primarily from a net decrease in accounts payable and accrued expenses of $0.9 million and an increase in prepaid expenses and other current assets of $0.2 million.

Net cash used in operating activities was $4.6 million for the three months ended March 31, 2014 and consisted primarily of a net loss of $4.5 million adjusted for non-cash items, including stock-based compensation expense of $0.1 million and depreciation and amortization expense of $0.1 million, and a net decrease in operating assets of $0.3 million, which resulted primarily from a net decrease in accounts payable and accrued expenses of $0.3 million.

Net cash used in operating activities was $20.4 million for the year ended December 31, 2014 and consisted primarily of a net loss of $21.9 million adjusted for non-cash items, including stock-based compensation expense of $0.9 million and depreciation and amortization expense of $0.2 million, and a net increase in operating assets of $0.8 million, which resulted primarily from a net increase in accounts payable and accrued expenses of $0.5 million partially offset by an increase in prepaid expenses and other current assets of $0.2 million.

Net cash used in operating activities was $16.4 million for the year ended December 31, 2013, and consisted primarily of a net loss of $18.1 million adjusted for non-cash items, including stock-based compensation expense of $0.3 million and depreciation and amortization expense of $0.3 million, and a net increase in operating assets of $1.1 million, which resulted primarily from a net increase in accounts payable and accrued expenses of $1.0 million and a decrease in prepaid expenses and other current assets of $0.1 million.

Net Cash Used in Investing Activities

Net cash used in investing activities was $25,000 during the three months ended March 31, 2015 compared to $13,000 during the three months ended March 31, 2014, which resulted primarily from increased laboratory equipment expenditures in the three months ended March 31, 2015.

Net cash used in investing activities was $0.2 million during the year ended December 31, 2014 compared to $43,000 during the year ended December 31, 2013, which resulted primarily from increased laboratory equipment expenditures in the year ended December 31, 2014. The cash used in investing activities for the years ended December 31, 2014 and 2013 was primarily the result of purchases of property and equipment.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $16.9 million during the three months ended March 31, 2015 compared to $19,000 during the three months ended March 31, 2014. The cash provided by financing activities for the three months ended March 31, 2015 primarily consisted of net proceeds of $12.3 million from the issuance 13,062,965 shares of our series B preferred stock in March 2015 and gross proceeds of $5.0 million from our borrowings under the Credit Facility offset by payments of deferred offering costs of $0.5 million. The cash provided by financing activities for the three months ended March 31, 2014 primarily consisted of net proceeds of $19,000 from stock option exercises.

Net cash provided by financing activities was $4.8 million during the year ended December 31, 2014 compared to $41.4 million during the year ended December 31, 2013. The cash provided by financing activities for the year ended December 31, 2014 primarily consisted of gross proceeds of $5.0 million from our borrowings under the Credit Facility, partially offset by $0.3 million of debt issuance costs. The cash provided by financing activities for the year ended December 31, 2013 consisted of net proceeds of $9.2 million from the issuance of 13,136,951 shares of our series A preferred stock in January and June 2013, net proceeds of $32.2 million from the issuance of 34,129,571 shares of our series B preferred stock in October 2013 and proceeds received from stock option exercises.

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, upon the closing of this offering, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we would be forced to delay, reduce or eliminate our research and development programs or future commercialization efforts.

We expect that the net proceeds from this offering, together with our existing cash and cash equivalents, 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. With the exception of the Credit Facility, we do not have any committed external source of funds. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our stockholders' ownership interests will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect our stockholders' rights. Additional debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include restrictive covenants that limit our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends, that could adversely impact our ability to conduct our business.

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

Contractual Obligations

The following table summarizes our significant contractual obligations as of payment due date by period at December 31, 2014:

	Payments due by period				
(In thousands)	Total	Less than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
Term loan(1)	$ 6,044	$ 794	$ 3,787	$ 1,463	$ —
Operating lease obligations(2)	1,894	756	1,138	—	—
Total contractual cash obligations	$ 7,938	$ 1,550	$ 4,925	$ 1,463	$ —

(1) Consists of repayment obligations under the Credit Facility, including interest.

(2) Represents future minimum lease payments under our non-cancelable operating lease. The minimum lease payments above do not include any related common area maintenance charges or real estate taxes.

On March 31, 2015, we entered into an amendment to the Credit Facility. Concurrently with entering into the Credit Facility amendment, we borrowed $5.0 million under a term loan under the Credit Facility with repayment terms that are equivalent to the initial term loan under the Credit Facility, including interest only payments until September 1, 2015 and 36 consecutive, equal monthly installment payments of principal and interest from October 1, 2015 through September 1, 2018.

We enter into agreements in the normal course of business with CROs for clinical trials and clinical supply manufacturing and with vendors for preclinical research studies and other services and products for operating purposes. We have not included these payments in the table of contractual obligations above since the contracts are cancelable at any time by us, generally upon 30 days prior written notice to the CRO, and therefore we believe that our non-cancelable obligations under these agreements are not material.

Off-Balance Sheet Arrangements

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

Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. As of December 31, 2014, we had cash and cash equivalents of $14.7 million and, as of March 31, 2015, we had cash and cash equivalents of $24.3 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 December 31, 2014 and March 31, 2015, we had no liabilities denominated in foreign currencies.

BUSINESS

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. Our initial 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 target therapeutic areas and specific diseases with significant unmet medical need where we believe we will have a competitive advantage. We seek to develop therapies that modulate multiple targets in the disease pathway. 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.

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. CAT-1004 is a SMART linker conjugate of salicylate, a non-steroidal anti-inflammatory drug, and the omega-3 fatty acid docosahexaenoic acid, or DHA, a naturally occurring unsaturated fatty acid with anti-inflammatory properties. We designed CAT-1004 to enhance the activity of salicylate and DHA in modulating the NF-κB pathway at multiple points. NF-κB, 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 believe that CAT-1004 modulates the disease pathway by inhibiting activated NF-κB and reducing the movement of activated NF-κB to the nucleus of the cell. Activated NF-κB drives muscle degeneration and suppresses muscle regeneration. Chronic activation of NF-κB has been reported in multiple skeletal muscle disorders, including muscular dystrophies, atrophy and inflammatory myopathies. In animal models of DMD, CAT-1004 inhibited activated NF-κB, reduced muscle inflammation and degeneration and increased muscle regeneration. In Phase 1 clinical trials in adults, CAT-1004 inhibited activated NF-κB and was well tolerated with no observed safety concerns. We plan to initiate patient enrollment in a Phase 1/2 clinical trial of CAT-1004 in boys with DMD in June 2015. Subject to patient enrollment, we 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. The U.S. Food and Drug Administration, or FDA, has granted CAT-1004 orphan drug designation for the treatment of DMD. We hold worldwide rights to CAT-1004.

Our two other clinical-stage product candidates, CAT-2054 and CAT-2003, are members of our CAT-2000 series. We applied our SMART linker technology to engineer these molecules as SMART linker conjugates of the omega-3 fatty acid eicosapentaenoic acid, or EPA, and nicotinic acid to modulate the Sterol Regulatory Element Binding Protein, or SREBP, pathway. EPA is a naturally occurring unsaturated fatty acid that has anti-inflammatory properties and beneficial effects on triglycerides. Nicotinic acid, which is also known as vitamin B3, is a naturally occurring essential vitamin that has beneficial lipid effects at high doses. Because we used different SMART linkers for CAT-2054 and CAT-2003, they possess different pharmacokinetic and biodistribution characteristics. CAT-2003, our first generation product candidate in the CAT-2000 series, is an orally administered molecule that modulates the SREBP pathway predominately in the intestine. CAT-2054, our second generation

product candidate in the CAT-2000 series, is an orally administered molecule that modulates the SREBP pathway predominately in the liver.

SREBP is a master regulator of lipid metabolism, the processing of fats, triglycerides and cholesterol by the body, and controls the metabolism of both low density lipoprotein cholesterol, or LDL-C, and triglycerides. We believe that the CAT-2000 product candidates modulate the disease pathway by inhibiting the maturation of the SREBP protein, thereby reducing its activity and reducing the production of proteins involved in lipid metabolism.

We have focused our initial efforts on the CAT-2000 series on lipid disorders such as hypercholesterolemia and hypertriglyceridemia because these disorders have clear short-term efficacy biomarkers, enabling us to rapidly and efficiently demonstrate proof of concept for our technology. In the CAT-2000 series, our development priority is CAT-2054 for the treatment of hypercholesterolemia, or elevated LDL-C, given what we believe is an attractive potential commercial opportunity in hypercholesterolemia and other SREBP-related metabolic disorders.

We are initially developing CAT-2054 for the treatment of hypercholesterolemia in patients for whom existing treatments are insufficient. Hypercholesterolemia is a disease that increases the risk of cardiovascular events. By modulating the SREBP pathway, CAT-2054 may inhibit production of important cholesterol metabolism proteins, such as proprotein convertase subtilisin kexin 9, or PCSK9; 3-hydroxy-3-methyl-glutaryl-CoA reductase, or HMG-CoA reductase; and adenosine triphosphate citrate lyase, or ATP citrate lyase, and Niemann-Pick C1-like 1, or NPC1L1. In a clinical trial of our first generation SREBP modulator CAT-2003, we observed statistically significant reductions in LDL-C, suggesting an impact of SREBP modulation on cholesterol metabolism. Because the liver is the primary regulator of cholesterol metabolism, we specifically designed the SMART linker in CAT-2054 to deliver more of the intact conjugate to the liver than CAT-2003. We believe that CAT-2054, if approved, has the potential to be the first therapy to simultaneously modulate cholesterol synthesis, clearance and absorption. In January 2015, we initiated a Phase 1 clinical trial to assess the safety, tolerability and pharmacokinetics of CAT-2054 in healthy volunteers. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the trial. If the final results of this clinical trial are positive, we intend to initiate a Phase 2a clinical trial in patients with 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. We hold worldwide rights to CAT-2054 and we intend to seek to commercialize CAT-2054 through one or more collaborations.

CAT-2003 is our first generation product candidate in the CAT-2000 series. We engineered CAT-2003 as an orally administered SMART linker conjugate of EPA and nicotinic acid to modulate the SREBP pathway. We designed CAT-2003 to target triglyceride levels in the blood and have studied CAT-2003 for the treatment of multifactorial chylomicronemia syndrome, or MFC, and refractory severe hypertriglyceridemia, or rSHTG, diseases with niche patient populations with elevated triglycerides or hypertriglyceridemia. We have completed three Phase 2a trials with CAT-2003 in patient populations with hypertriglyceridemia. While we have chosen to prioritize the development of CAT-2054 over CAT-2003, we believe that the clinical trial data for CAT-2003 support the utility of our SMART linker technology and the potential to treat lipid and metabolic disorders by modulating the SREBP pathway. We intend to pursue collaborations to conduct exploratory evaluation of CAT-2003 in other serious diseases that involve alterations in the SREBP pathway, such as nonalcoholic steatohepatitis, or NASH, and hepatocellular carcinoma, either to develop CAT-2003 as a product candidate or to support our development efforts for CAT-2054. We hold worldwide rights to CAT-2003.

CAT-4001, our most advanced preclinical product candidate, is a SMART linker conjugate of monomethyl fumarate and DHA. CAT-4001 is a small molecule that activates the Nrf2 pathway and inhibits activated NF-κB. Nrf2, or Nuclear factor (erythroid-derived 2)-like 2, is a gene transcription

factor, a protein that works inside of cells to control the expression of genes, that controls the body's response to cellular stress and oxidative damage. We believe that CAT-4001 modulates the disease pathway by enhancing the movement of Nrf2 to the nucleus of the cells and inhibits NF-κB by reducing the movement of activated NF-κB to the nucleus of the cells. We are exploring CAT-4001 as a potential treatment for severe, rare neurodegenerative diseases, such as Friedreich's ataxia and amyotrophic lateral sclerosis, or ALS, two diseases of the central nervous system in which the Nrf2 and NF-κB pathways have been implicated. We plan to conduct additional preclinical evaluation of CAT-4001 in 2015, and if the results are positive we intend to advance CAT-4001 into investigational new drug application, or IND, enabling studies in 2016. We hold worldwide rights to CAT-4001.

As of April 30, 2015, we owned two issued U.S. patents relating to composition of matter and method of use claims directed to CAT-1004 and two issued U.S. patents relating to composition of matter and method of use claims directed to the CAT-2000 series. These patents are expected to expire between 2029 and 2031, without taking potential patent term extensions into account. In addition, our patent portfolio includes over 10 issued foreign patents, over 25 pending U.S. patent applications and over 100 pending foreign patent applications.

Our Strategy

Our objective is to apply our proprietary SMART linker technology platform to discover, develop and commercialize novel bi-functional therapeutics, with an initial focus on rare diseases and serious lipid disorders, either on our own or through collaborations. We target therapeutic areas and specific diseases with significant unmet medical need where we believe we will have a competitive advantage. We seek to develop therapies that modulate multiple targets in the disease pathway. To achieve our goals, we are pursuing the following strategies:

- *Complete the development of CAT-1004 through registration for DMD.* We are devoting a significant portion of our resources to developing CAT-1004 for the treatment of DMD. We plan to initiate patient enrollment in a Phase 1/2 clinical trial of CAT-1004 for the treatment of DMD in June 2015. Subject to patient enrollment, we 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. Our goal is to develop the first disease-modifying oral therapy for the treatment of DMD that promotes muscle regeneration.

- *Advance the development of CAT-2054.* In January 2015, we initiated a Phase 1 clinical trial of CAT-2054 to assess its safety, tolerability and pharmacokinetics in healthy volunteers. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the trial. If the final results of this clinical trial are positive, we intend to initiate a Phase 2a clinical trial in patients with 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. We also plan to evaluate CAT-2054 and other CAT-2000 series molecules in other SREBP-mediated diseases.

- *Advance the development of CAT-4001.* We plan to conduct additional preclinical evaluation of CAT-4001 in Friedreich's ataxia and ALS in 2015. If the results are positive, we intend to advance CAT-4001 into IND-enabling studies in 2016.

- *Continue to apply our SMART linker technology platform.* We have used our SMART linker technology platform to rapidly and efficiently identify product candidates that we have been able to advance into clinical development. To date we have engineered more than 20 conjugate series, of which two are now in clinical development. Examples of our pre-clinical assets include

conjugates of fumarate, cysteamine, statins, antivirals and chemotherapeutics. We are continually advancing our conjugation capabilities and exploring new applications of our technology platform to develop bi-functional therapeutics to treat diseases that we believe can be effectively addressed by modulating multiple biological targets in one or more related disease pathways. We are also advancing our technology for application to the intracellular delivery of protein therapeutics. While we intend to continue our internal drug discovery and development efforts, we also plan to leverage our SMART linker technology platform through selective collaborations with leading biotechnology and pharmaceutical companies to further broaden our pipeline of product candidates. We have a goal of identifying at least one novel conjugate per year, either on our own or through collaborations, that we can advance into preclinical development.

- *Maintain flexibility in commercializing and maximizing the value of our development programs.* We intend to enter into strategic relationships with biotechnology and pharmaceutical companies where maximizing the commercial value of our development programs will require access to broader geographic markets or the pursuit of broader patient populations or indications, such as with the CAT-2000 series and the commercialization of CAT-1004 outside of the United States and Canada. We also plan to build focused capabilities in the United States and Canada to commercialize development programs, such as CAT-1004 for DMD, where we believe that the medical specialists for the indications are sufficiently concentrated to allow us to effectively promote the product with a targeted sales team.

Our Scientific Approach

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. The traditional model for drug discovery has focused on identifying and evaluating drug candidates with the goal of modifying a single biological target implicated in a specific disease process. This approach of selecting a single bioactive to modulate a single target has been successful for certain types of diseases. However, many diseases are caused by multiple abnormalities rather than by a single defect. In these cases, the traditional single-target approach to drug discovery and development may be less effective because a single target may not address the multiple underlying defects causing the disease.

Multi-target therapies have in many cases been developed to provide treatment options where single-target therapies have been ineffective. These multi-target therapies have traditionally followed one of two approaches: either use of a single drug that binds to multiple biological targets or co-administration of two or more drugs that interact with different targets. While each of these approaches has well-established benefits in a variety of indications, each is also characterized by significant limitations. For example, use of a single broadly targeted drug can lead to off-target toxicities, side-effects and tolerability issues, and co-administration of two or more drugs can be confounded by differences in the pharmacokinetics and tissue distribution of the drugs, thereby reducing the likelihood of each agent being simultaneously active in the same cell.

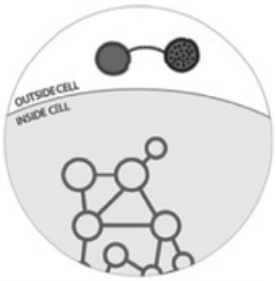
Our aim is to leverage the growing body of knowledge associated with disease pathways, and to rationally design orally bioavailable product candidates that simultaneously interact with multiple biological targets within one or more related disease pathways. While other technologies exist to conjugate or combine two bioactives, we believe that our SMART linker technology platform provides substantial improvements over previous approaches to bioactive conjugation.

SMART Linker Technology Platform

We have leveraged our SMART linker technology platform to create molecules that simultaneously modulate multiple biological targets within one or more related disease pathways. Our technology platform includes a broad array of linkers that we use to engineer molecular series, which are

susceptible to intracellular cleavage only. The linkers used in our technology platform are small chemicals designed to join two separate bioactives into a single conjugate molecule. In systemic circulation, our SMART linker conjugates are stable and inactive, potentially reducing off-target toxicities and side-effects. The conjugates are designed to be cleaved by specific enzymes exclusively within cells in order to release the two bioactives inside the cells. By releasing the bioactive components of the conjugate molecule only inside cells, the SMART linker allows the bioactives to reach their targets more efficiently and have greater efficacy than if the bioactives were dosed independently or in combination. The stability of our SMART linker conjugates outside of cells and the release of the bioactives exclusively within cells are important differentiating features of our SMART linker technology platform.

To create a conjugate using our SMART linker technology platform, we begin by analyzing pathways that are disrupted in a disease. We then select two bioactive molecules known for their clinical safety and demonstrated effect along one or more of these biological pathways. We then design a SMART linker that will conjugate the two selected bioactives, allow the conjugate to be carried to biological tissues and, following entry into cells, be cleaved by enzymes resident in the cells to release the bioactives, as shown in the figures below.



A SMART linker joins two selected bioactives to form a conjugate that is stable in systemic circulation.

Once the SMART linker conjugate enters a cell, it is cleaved into its two component bioactives.

As a result, the component bioactives modulate different targets in the disease pathway.

Our SMART linker conjugates are designed to be stable to oral dosing, as well as stable in both the lumen of the intestine and in systemic circulation, which we have now observed in clinical trials for two product candidate series. We design the SMART linker to chemically link the two bioactive molecules through their pharmacophores, the regions of the bioactive molecules that are responsible for carrying out their biological activity, resulting in inactivation of the bioactives. Once the conjugate enters a cell, the SMART linker is cleaved by specific enzymes which reside only within cells, releasing the two bioactives to interact with their biological targets. Delivery of the bioactives through the SMART linker conjugate into the cell results in the two bioactives having the same pharmacokinetics and tissue distribution. As a result, our SMART linker conjugates can simultaneously modulate two biological targets in disease pathways of interest within the same cell. In addition, release of the bioactives exclusively inside cells can potentially reduce or eliminate off-target, extracellular activity of the bioactives, which may improve safety and tolerability.

We have observed in multiple preclinical studies that our SMART linker conjugates achieved greater efficacy than administration of the two bioactives either independently or in combination. In clinical trials, SMART linker conjugates have demonstrated significant improvements in activity on disease pathways and tolerability relative to equivalent doses of the two bioactives delivered in combination. We also have observed statistically significant efficacy with SMART linker conjugates at dose levels significantly lower than the prescribed doses of the two component bioactives.

We believe our SMART linker technology platform has the potential to:

- enhance activity on disease pathways through modulation of multiple biological targets;
- improve efficacy by matching the pharmacokinetics and tissue distribution of the component bioactives; and
- improve safety and tolerability by releasing the component bioactives only within cells.

In addition, we are advancing our technology for application to the intracellular delivery of protein therapeutics. We have early evidence of the intracellular delivery of proteins into cells with test proteins and are now exploring potential protein therapeutics.

Our Product Candidates

The following chart summarizes key information regarding our product candidates. We hold worldwide rights to all of our product candidates.

Series	Pathway	Product Candidate	Indication	Pre-clinical	Phase 1	Phase 2	Phase 3	Status
CAT-1000	*NF-κB*	CAT-1004	• Duchenne muscular dystrophy					• Expect to enroll patients in Phase 1/2 trial in June 2015
CAT-2000	*SREBP*	CAT-2054	• Hypercholesterolemia					• Completed preliminary analysis of Phase 1 data, expect full data in the third quarter of 2015 • Expect to initiate Phase 2a trial in the fourth quarter of 2015
		CAT-2003	• Hypertriglyceridemias					• Three Phase 2a trials completed
CAT-4000	*NRF2/ NF-κB*	CAT-4001	• Friedreich's ataxia • Amyotrophic lateral sclerosis					• Expect to continue pre-clinical studies in 2015

CAT-1004

We are developing CAT-1004 for the treatment of DMD, with the potential to regenerate muscle and be disease modifying in boys with DMD regardless of the underlying dystrophin mutation. CAT-1004 is an orally administered SMART linker conjugate of salicylate and DHA that we designed to enhance the activity of salicylate and DHA in modulating the NF-κB pathway at multiple points. Emerging data suggest that activation of NF-κB drives the loss of skeletal muscle mass in multiple diseases, including muscular dystrophies, atrophy and inflammatory myopathies. In December 2014, we submitted an IND to the FDA for CAT-1004 for DMD. We plan to initiate patient enrollment in a Phase 1/2 clinical trial of CAT-1004 for the treatment of DMD in June 2015. Subject to patient enrollment, we 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. We believe that CAT-1004 has the potential to be the first disease-modifying oral therapy for the treatment of DMD that promotes muscle regeneration. The FDA has granted CAT-1004 orphan drug designation for the treatment of DMD and we plan to submit an

orphan drug designation request to the European Medicines Agency, or EMA, in the second half of 2015.

Overview of DMD

DMD is a rare pediatric disorder involving progressive muscle degeneration that eventually leads to death. DMD is caused by various mutations in the dystrophin gene that result in a lack of functional dystrophin in muscle fibers, which renders muscle fibers more susceptible to mechanical stress. Dystrophin is a protein that resides in the membrane of muscle cells and is critical to the structural and membrane stability of muscle fibers in skeletal, diaphragm and heart muscle. When muscles contract or stretch during normal use, the absence of normally functioning dystrophin results in activation of the NF-κB pathway, triggering inflammation in the muscles, resulting in muscle damage and reducing the ability of muscles to regenerate. As muscle damage progresses, connective and adipose tissues replace muscle fibers, resulting in inexorable muscle weakness.

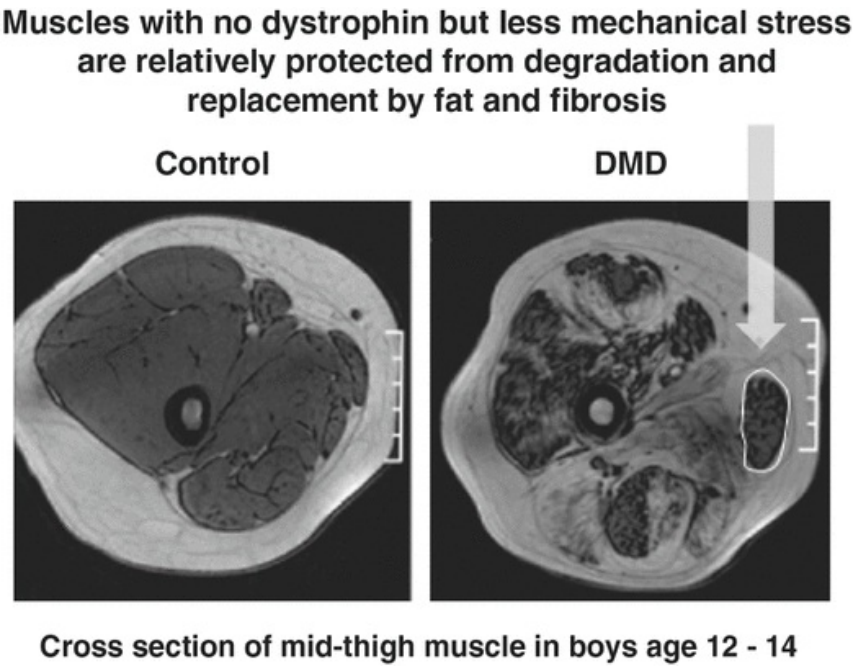
DMD occurs almost exclusively in males, occurring in approximately 1 in 3,500 live male births. Based on this incidence rate, we estimate that DMD affects a total of approximately 15,000 patients in the United States and approximately 19,000 patients in the European Union.

Children with DMD typically begin to show symptoms of disease between ages two and five, when they develop a waddling gait, frequently fall and have difficulty rising from the floor. Progressive weakness then develops in the voluntary muscles in the arms, legs and trunk. This muscle weakness results in fixations, or contractures, of joints, such as knees, hips and elbows. By age eight, most patients have difficulty ascending stairs. By their early teens, patients typically lose walking ability and are confined to wheelchairs. Patients' cardiac and respiratory muscles are also adversely affected, typically requiring use of ventilators in their late teens. Progressive weakening of cardiac and respiratory muscles eventually results in death, generally by patients' mid-twenties.

The Role of NF-κB in Duchenne Muscular Dystrophy

NF-κB plays an important role in regulating skeletal muscle health and appears to be especially important in regulating skeletal muscle mass in chronic diseases such as DMD. Activated NF-κB promotes the degradation of specific muscle proteins and leads to the induction of pro-inflammatory mediators such as cytokines, including tumor necrosis factor alpha, or TNF-α, interleukin 6, or IL-6, and interleukin-1 beta, or IL-1β; chemokines; cell adhesion molecules; and tissue degrading enzymes, such as matrix metallopeptidase 9, or MMP-9. In addition, activated NF-κB suppresses muscle stem cell differentiation that is required for muscle regeneration by preventing satellite stem cells from differentiating into myoblasts, progenitor cells that differentiate, to give rise to muscle cells. Activation of NF-κB is observed in muscle tissues of patients with DMD prior to the onset of other clinical manifestations, and activated NF-κB is persistently elevated in the immune cells and degenerating muscle fibers of patients with DMD. Moreover, evidence exists that mechanical stress activates NF-κB in muscles by a factor of three to four times and drives NF-κB mediated inflammation. Muscles with increased mechanical stress and inflammation, such as quadriceps and hamstrings, show the greatest progression of disease. This more rapid deterioration of muscles bearing greater mechanical stress, and thus more activated NF-κB mediated inflammation, in boys with DMD can be observed through magnetic resonance imaging, or MRI, such as in the following image from a study conducted by

ImagingDMD, a group of investigators at clinical sites in the United States with clinical leadership and expertise in the use of MRI as an assessment tool for DMD.



Unaddressed Market Opportunity

There are 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. Corticosteroids have demonstrated efficacy in DMD patients, which is believed to be driven by reductions in activated NF-κB. However, corticosteroids primarily act through another pathway called the glucocorticoid receptor-mediated pathway, and also can cause significant complications including growth suppression, reduction in bone strength and compromise of the immune system. Over time, corticosteroids induce chronic myopathy in many diseases through induction of muscle protein breakdown, which ultimately leads to muscle damage. DMD patients treated with corticosteroids typically show an initial improvement in measures of muscle function but then resume a progressive decline. Approximately half of DMD patients treated with steroids lose the ability to walk by age eleven and almost all are in wheelchairs by age sixteen. DMD patients typically live until their mid-twenties, despite the availability of corticosteroids.

Several companies are exploring new therapies for the treatment of DMD. The three most advanced product candidates, PTC Therapeutics' ataluren, BioMarin's drisapersen and Sarepta's eteplirsen, target mechanisms to increase levels of dystrophin in muscles. Each of these product candidates compensates for a specific genetic mutation in order to produce a partially functional dystrophin protein. The therapeutic goal of these product candidates is to reduce disease severity and extend survival in those DMD patients with the specific mutation. Based on the prevalence of the specific mutations that these product candidates are designed to address, they would be expected to be effective in an aggregate of approximately 26% of DMD patients. We believe that DMD patients, including those treated with these dystrophin therapies, will continue to require treatments to reduce muscle inflammation and enhance muscle regeneration.

CAT-1004 for the Treatment of Duchenne Muscular Dystrophy

CAT-1004 is a SMART linker conjugate of salicylate and DHA that we designed to enhance the activity of salicylate and DHA in modulating the NF-κB pathway at multiple points. The CAT-1004 conjugate is inactive outside the cell, and, once inside the cell CAT-1004 is cleaved, releasing DHA and salicylate simultaneously inside the cell. Based on its mechanism of action in suppressing activated NF-κB, we believe that CAT-1004 has the potential to combine reduction of inflammation and muscle degeneration with positive effects on muscle regeneration, all of which may allow patients to retain muscle function longer. In addition, we believe that CAT-1004 has the potential to be effective in all DMD patients, regardless of the underlying mutation, and to provide significant benefit to patients, both as monotherapy and when used in combination with other therapies, including dystrophin-targeted therapies and agents targeting myostatin.

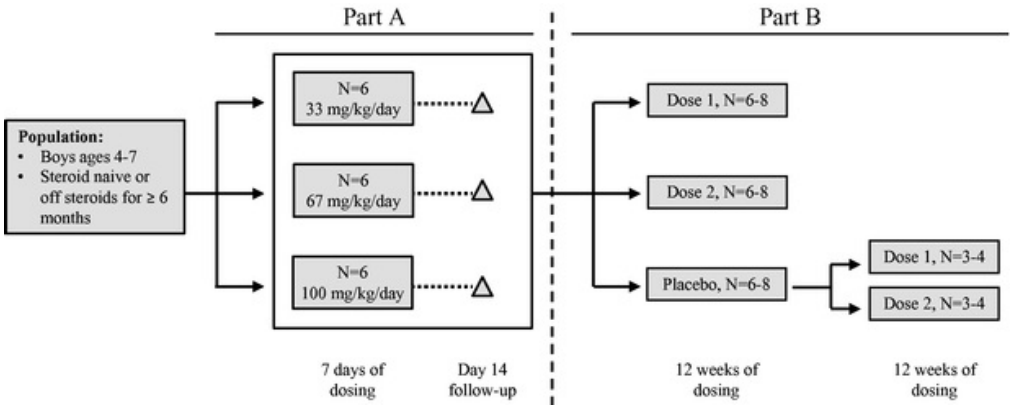
In Phase 1 clinical trials in adults, CAT-1004 was observed to be well tolerated and to inhibit activated NF-κB. Based on the positive effects observed in the Phase 1 trials and in animal studies, we plan to initiate enrollment in a Phase 1/2 clinical trial in boys with DMD in June 2015.

CAT-1004 Clinical Development

Planned Phase 1/2 Trial of CAT-1004 in Patients with DMD

Our planned CAT-1004 Phase 1/2 trial will enroll ambulatory boys between ages four and seven with a genetically confirmed diagnosis of DMD. We refer to the trial as the MoveDMD trial. The enrolled boys will be steroid naive or have not used steroids for at least six months prior to the trial. The enrolled boys will not be limited to any specific dystrophin mutations. We will conduct the trial at three sites in the United States. We plan to conduct the MoveDMD trial in two sequential parts, Part A and Part B, as illustrated in the following diagram.

Planned MoveDMD Trial Design



In Part A of the MoveDMD trial, we will assess the safety, tolerability and pharmacokinetics of CAT-1004 in patients at three dosing levels following seven days of dosing. We also will compare CAT-1004 exposure levels to exposure levels achieved in previous CAT-1004 clinical trials where NF-κB biomarker activity was achieved.

Part B of the MoveDMD trial will be a randomized, double-blind, placebo-controlled trial. In Part B, we plan to treat patients with one of two dosing levels of CAT-1004 or placebo for 12 weeks. After 12 weeks of dosing, patients receiving placebo may be crossed over to one of two doses of CAT-1004 for an additional 12 weeks. We have designed the MoveDMD trial with the assistance of

ImagingDMD. We expect that the MoveDMD trial will be conducted at ImagingDMD's three clinical sites.

We anticipate that the primary efficacy endpoint in Part B of the MoveDMD trial will be change in muscle inflammation as measured by MRI of leg muscles. MRI studies in DMD have recently shown that inflammatory changes occur before development of fibrosis and infiltration of fat into muscle. Inflammatory changes are most evident in muscles that ultimately show the greatest replacement by non-contractile tissues. Both inflammation and fatty infiltration are correlated with functional ability in boys with DMD. Additionally, third party studies have shown that in young DMD patients that are still ambulatory, decreases in muscle inflammation over 12 weeks of glucocorticoid therapy can be clearly identified through MRI imaging. Similarly, glucocorticoids have been observed to improve muscle strength and performance in timed functional tests after short periods of treatment. In early ambulatory DMD boys, functional abilities such as the 10 meter walk/run are relatively stable and more homogeneous than in older boys in whom functional ability is declining. We plan to include as exploratory endpoints timed function tests best suited for the age group of the trial subjects, specifically the 10 meter walk/run, time to stand and 4-stair climb tests. In addition, assessments of muscle strength and a parent-proxy measure of functional ability will be included.

Subject to patient enrollment, we expect to report top-line data from Part A of the MoveDMD trial in late 2015 and top-line data from Part B of the trial in late 2016. If the results of this 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.

Completed Clinical Trials

To date, we have studied CAT-1004 in three completed Phase 1 clinical trials. The design and results for these clinical trials are discussed below.

CAT-1004—Completed Phase 1 Clinical Trials

			Subjects	
Trial	**Description**	**Duration**	**Total**	**Treated with CAT-1004**
CAT-1004-101	Randomized, double-blind, placebo-controlled, single ascending dose clinical trial to evaluate safety, tolerability and pharmacokinetics of CAT-1004 in healthy subjects	1 day	52	39
CAT-1004-102	Randomized, double-blind, placebo-controlled multiple ascending dose clinical trial to evaluate safety, tolerability, pharmacokinetics and pharmacodynamics of CAT-1004 in adults with Type 2 diabetes	14 days	44	32
CAT-1004-103	Single-blind biomarker trial in healthy adults to compare activity of CAT-1004, a combination of salicylate and DHA, or placebo on activated NF-κB	1 day	9	8

Phase 1 Single Ascending Dose Trial (CAT-1004-101): We conducted a randomized, double-blind, placebo-controlled, single ascending dose Phase 1 clinical trial in 52 healthy volunteers at a single site in the United States to assess the safety, tolerability and pharmacokinetics of CAT-1004 in both fasted and fed states. The participants were randomized to receive CAT-1004 or placebo. CAT-1004 was administered orally in soft gelatin capsules at doses ranging from 300 mg to 6000 mg.

Single doses of CAT-1004, administered to subjects in both fed and fasted conditions appeared to be well tolerated. Subjects in the fasted state reported few adverse events, or AEs, with the most commonly reported AEs being headache, diarrhea and dizziness. Of the 44 subjects in the fasted state, five reported headache, three reported diarrhea and two reported dizziness. The majority of the AEs in the fasted state were mild in severity. The most common AEs in the fed state were diarrhea, headache and abdominal pain and all of the AEs in the fed state were mild in severity. Of the 35 subjects in the fed state, six reported diarrhea, six reported headache and four reported abdominal pains. Subjects in the fed state receiving single doses of CAT-1004 of 4000 mg or more reported gastrointestinal AEs more frequently than subjects receiving lower doses. No treatment-related severe AEs were reported. There were no observed trends in laboratory, vital signs or electrocardiogram results following CAT-1004 administration in either the fasted or fed state.

CAT-1004 was rapidly absorbed in plasma, with mean maximum and overall plasma exposure generally increasing with CAT-1004 dose levels. Neither component bioactive, salicylate or DHA, was detected in plasma at levels above background, consistent with intracellular cleavage of CAT-1004 and intracellular delivery of the component bioactives. Administration of a high-fat meal increased CAT-1004 mean maximum and overall exposure by approximately 3- to 8-fold.

Phase 1 Multiple Ascending Dose Trial (CAT-1004-102): We conducted a randomized, double-blind, placebo-controlled, multiple ascending dose Phase 1 clinical trial in 44 subjects at a single center in the United States to assess the safety, tolerability, pharmacokinetics and pharmacodynamics of CAT-1004. These subjects had Type 2 diabetes and mild background inflammation, which enabled us to assess the activity of CAT-1004 on activated NF-κB. Subjects were randomized to receive CAT-1004 or placebo. CAT-1004 was administered orally in soft gelatin capsules at total daily doses ranging from 300 mg to 4000 mg.

CAT-1004 administered for two weeks appeared to be well tolerated. The AEs reported in more than one subject were each reported by two subjects. These AEs were diarrhea (both instances reported by subjects receiving 4000 mg daily doses of CAT-1004), gastroenteritis (one instance reported by a subject in the placebo group and the other by a subject receiving 1000 mg daily doses of CAT-1004) and upper respiratory tract infection (both instances reported by subjects receiving 4000 mg daily doses of CAT-1004). The majority of the AEs were mild in severity. No treatment-related severe AEs were reported.

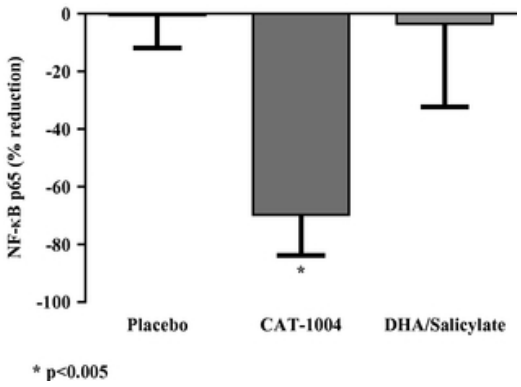
CAT-1004 was rapidly absorbed in plasma, with mean maximum and overall plasma exposure generally increasing with escalating single or multiple doses of CAT-1004. Neither component bioactive, salicylate or DHA, was detected in plasma at levels above background, again consistent with intracellular cleavage of CAT-1004 and intracellular delivery of the component bioactives.

In the Phase 1 multiple ascending dose trial, we observed by two methods that CAT-1004 inhibited activated NF-κB. For the first method, we stimulated NF-κB activity *ex vivo* in whole blood from subjects treated with CAT-1004 or placebo, and then observed NF-κB activity in monocytes, or immune cells, that we isolated from the whole blood. NF-κB activity was reduced in a majority of subjects following two weeks of CAT-1004 treatment but not following treatment with placebo. For the second method, we performed gene expression analyses on whole blood taken from subjects prior to treatment and after two weeks of treatment with CAT-1004 or placebo. CAT-1004 significantly reduced the expression of a set of genes that are controlled by NF-κB. In contrast, treatment with placebo for two weeks did not significantly reduce expression of NF-κB regulated genes.

Phase 1 NF-κB Biomarker Trial (CAT-1004-103): We conducted a single-blind, crossover Phase 1 clinical trial with CAT-1004 in nine healthy adult volunteers at a single center in the United States to compare activity of a single dose of 2000 mg of CAT-1004 on activated NF-κB to a combination of salicylate and DHA or placebo. No AEs were reported in this clinical trial. The salicylate and DHA were dosed at approximately equivalent amounts to those contained in the CAT-1004 conjugate. We

assessed NF-κB activity in peripheral blood mononuclear cells, or PBMCs, isolated from subjects before dosing and two hours after dosing. PBMCs are circulating immune cells that can mount an NF-κB response and migrate into tissue such as muscle and drive inflammation. Prior to the determination of NF-κB activity, we stimulated whole blood with lipopolysaccharide, or LPS, to activate the NF-κB pathway. As shown in the graph below, treatment of subjects with CAT-1004 significantly reduced the level of activated NF-κB, as measured by nuclear p65, a surrogate marker for activated NF-κB. In contrast, no change in the level of activated NF-κB was observed upon treatment with the combination of salicylate and DHA, or upon treatment with placebo. In this trial, CAT-1004, which is a SMART linker conjugate of salicylate and DHA, exhibited greater activity on the NF-κB pathway than the combination of its component bioactives.

Effect of CAT-1004 on Activated NF-κB



These results were statistically significant, with a p-value of less than 0.005. P-value is a conventional statistical method for measuring the statistical significance of clinical results. A p-value of 0.05 or less represents statistical significance, meaning that there is a 1-in-20 or less statistical probability that the observed results occurred by chance.

CAT-1004 Preclinical Development

In preclinical studies, we have observed that CAT-1004 inhibited NF-κB activity *in vitro* and *in vivo*, and produced disease-modifying effects in two established animal models of DMD, the *mdx* mouse model and the Golden Retriever muscular dystrophy, or GRMD, dog model.

In Vivo Studies in Animal Models of DMD

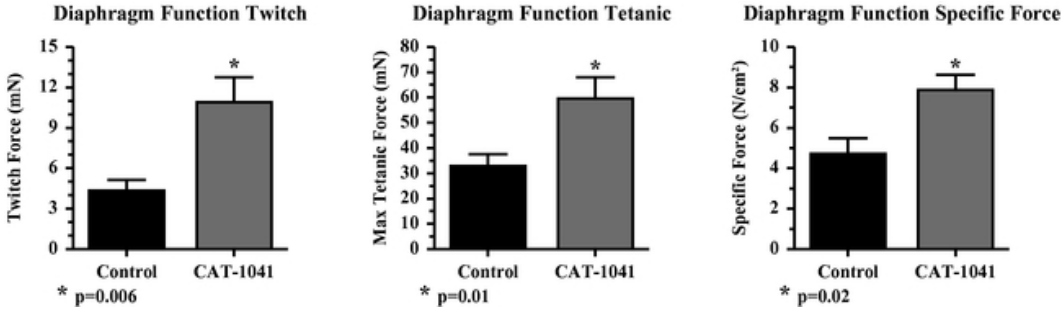
We have created several SMART linker conjugates that inhibit activated NF-κB. Two of these conjugates, CAT-1004 and CAT-1041, exhibit very similar effects on NF-κB activity in cell based assays, in animal studies and on functional activity in animal models. CAT-1041 is a closely related analog of CAT-1004 in which the DHA component of the salicylate-DHA conjugate has been replaced with EPA. In some preclinical studies, we used CAT-1041 as a surrogate for CAT-1004. Both CAT-1004 and CAT-1041 produced disease-modifying efficacy in established animal models of DMD. We decided to advance CAT-1004 into clinical trials rather than CAT-1041 based on scientific literature suggesting that DHA has superior anti-inflammatory activity compared to EPA.

mdx Mouse Model. We examined the potential therapeutic effects of CAT-1004 using the *mdx* mouse model of DMD. We observed that four weeks of treatment with CAT-1004 or prednisolone, a steroid, reduced muscle inflammation and the number of degenerating muscle fibers in *mdx* mice. However, only CAT-1004-treated animals showed preservation of muscle mass and an increase in the

number of regenerating fibers, suggesting that chronic treatment with CAT-1004 can protect muscle from the damage expected to occur over time in *mdx* mice.

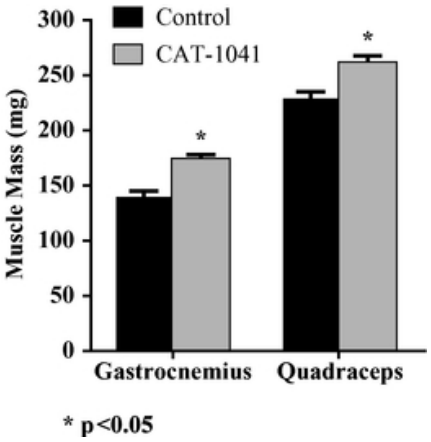
In a long-term *mdx* mouse study, we observed that, compared to the control group of *mdx* mice, six months of treatment with CAT-1041 significantly improved muscle endurance as measured by mean weekly and total running distance determined based upon cumulative revolutions on a running wheel. As shown in the graphs below, improvements in muscle endurance following CAT-1041 treatment versus control were also observed in post-mortem assessments of twitch force, tetanic force and specific force generation, each of which is an established measurement of muscle endurance, in excised diaphragm muscle.

CAT-1041 Activity on Diaphragm Function in the mdx Mouse Model



As shown in the graph below, we also observed in this same study that *mdx* mice treated with CAT-1041 showed significantly increased mass of two major leg muscles, the gastrocnemius and quadriceps. These increases were independent of changes in total body weight. CAT-1041-treated mice also had a statistically significant reduction in heart mass, suggesting that chronic treatment with CAT-1041 may have reduced the dilated cardiomyopathy typically observed in *mdx* mice.

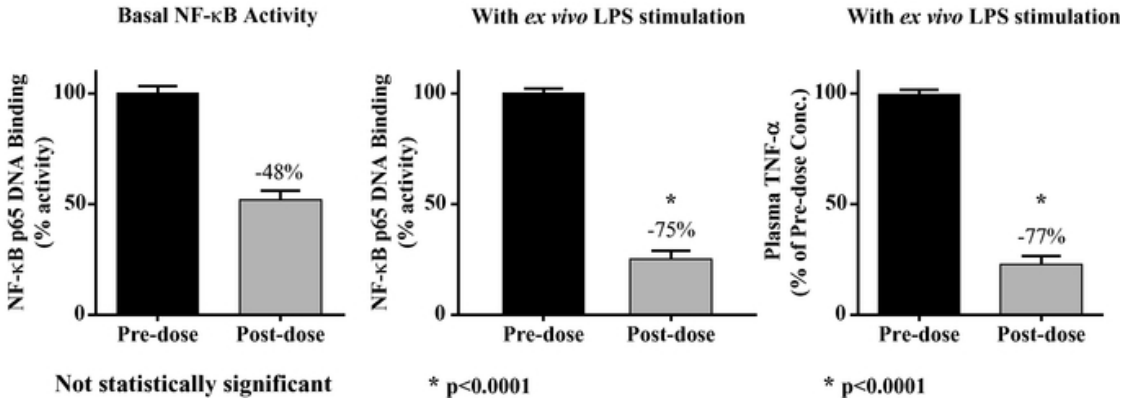
CAT-1041 Activity on Muscle Mass in the mdx Mouse Model



In this study, we also observed that CAT-1004 and CAT-1041 exhibited similar activity on muscle contractions of the extensor digitorum longus muscle in *mdx* mice with significant preservation of muscle function compared to control. Finally, in this study we observed a reduction in diaphragm and quadricep muscle fibrosis in *mdx* mice treated with CAT-1041 in comparison to control.

Golden Retriever Dog Model. We also evaluated the effects of CAT-1004 in the GRMD dog model. As shown in the graph below, a single oral dose of CAT-1004 inhibited basal, or unstimulated, NF-κB activity by 48% in GRMD dogs. CAT-1004 also inhibited LPS-stimulated NF-κB activity by 75% and LPS-stimulated plasma levels of TNFα protein, a key marker of inflammatory response, by 77%. Together, these data suggest that a single oral dose of CAT-1004 achieves sufficient exposure levels to inhibit activated NF-κB in a dog model of DMD.

Effect of CAT-1004 on NF-κB in the GRMD Dog Model



In Vitro Studies

In an *in vitro* study in a mouse macrophage cell line, we observed that CAT-1004 inhibited LPS-stimulated NF-κB activity to a greater extent than either of its components, salicylate and DHA, alone or in combination. We also observed that CAT-1004 inhibited LPS-stimulated NF-κB activity in human PBMCs, which are a potential target tissue for CAT-1004. In studies performed with a mouse macrophage cell line, CAT-1004 reduced the LPS-stimulated expression of a set of genes that encode pro-inflammatory mediators and whose expression is controlled by NF-κB.

CAT-1004 Orphan Drug Designation

The FDA has granted CAT-1004 orphan drug designation for the treatment of DMD and we plan to submit an orphan drug designation request to the European Medicines Agency, or EMA, in the second half of 2015. A product may be designated by the FDA as an "orphan drug" if it is intended to treat a rare disease or condition affecting fewer than 200,000 individuals in the United States. Similarly, the EMA may designate a product as an orphan drug if it is intended for the treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons. If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation, the FDA will not approve another sponsor's marketing application for the same product for the same use or indication before the expiration of seven years, except in certain limited circumstances. In Europe, marketing authorization for an orphan drug generally leads to a ten-year period of market exclusivity.

CAT-2000 Series

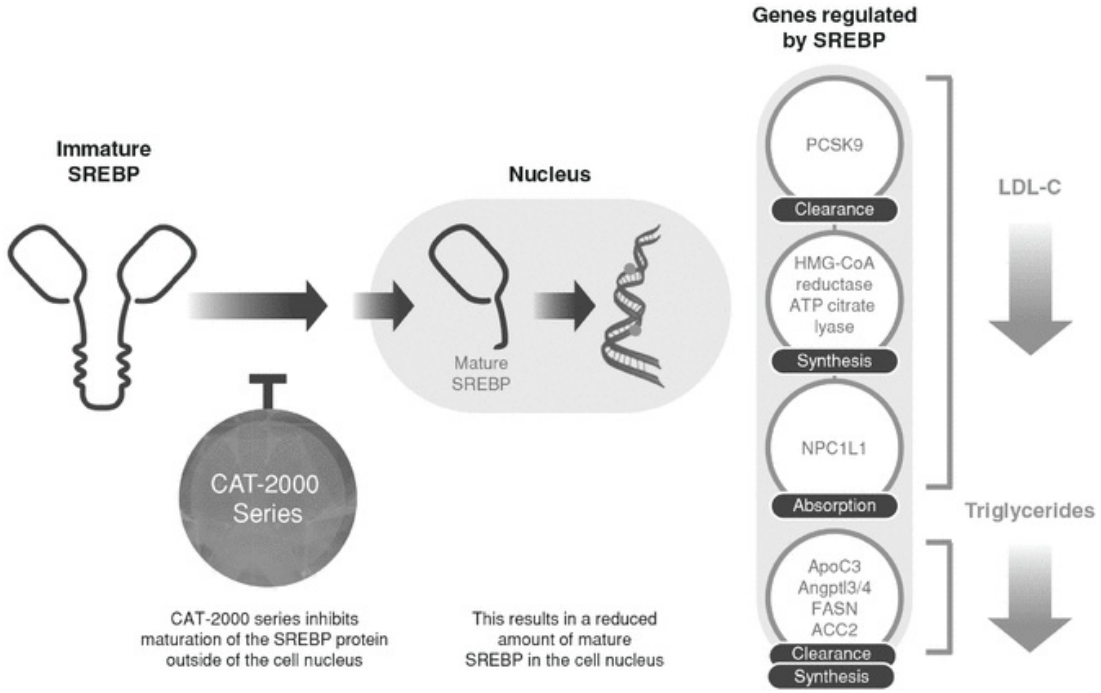
Our two other clinical-stage product candidates, CAT-2054 and CAT-2003, are members of our CAT-2000 series. We applied our SMART linker technology to engineer these molecules as SMART linker conjugates of EPA and nicotinic acid in order to modulate the SREBP pathway in different organs. Because we used different SMART linkers for CAT-2054 and CAT-2003, they possess different characteristics such as rates of cleavage, pharmacokinetics and biodistribution. CAT-2003, our first generation product candidate in the CAT-2000 series, is an orally administered molecule that modulates the SREBP pathway predominately in the intestine. CAT-2054, our second generation product candidate in the CAT-2000 series, is an orally administered molecule designed to modulate the SREBP pathway predominately in the liver. We have focused our initial efforts on the CAT-2000 series on lipid disorders such as hypercholesterolemia and hypertriglyceridemia because these disorders have clear short-term efficacy biomarkers, which we believe will enable us to rapidly and efficiently determine clinical proof of concept of our technology.

Overview of the SREBP Pathway

SREBP is a master regulator of lipid and energy metabolism and regulates the levels of LDL-C, triglycerides and fatty acids in the body. SREBP controls lipid levels by controlling the expression of genes such as PCSK9, HMG-CoA reductase and ATP citrate lyase. Dysregulation of SREBP activity has been implicated in a number of human metabolic diseases, including hyperlipidemias, such as hypercholesterolemia and hypertriglyceridemia, and chronic liver diseases, including NASH and hepatocellular carcinoma. Modulators of SREBP activity could have therapeutic benefit in treating these SREBP-mediated diseases.

As shown in the figure below, we designed the CAT-2000 molecules to inhibit the maturation of SREBP and reduce the expression of key proteins involved in LDL-C and triglyceride metabolism.

The CAT-2000 Molecules Inhibit SREBP



SREBP regulates cholesterol levels by controlling expression of PCSK9, a protein that controls the clearance of LDL-C from circulation through the reduction of the amount of the LDL receptor on the surface of the liver; HMG-CoA reductase, an enzyme that plays a central role in the synthesis of LDL-C in the liver; ATP citrate lyase, an enzyme in the LDL-C synthetic pathway; and Niemann-Pick C1-like 1, or NPC1L1, which is the critical mediator of cholesterol absorption in the gastrointestinal tract epithelial cells as well as in liver cells. These four proteins are important in regulating cholesterol levels because they control cholesterol clearance, synthesis and absorption.

SREBP regulates triglyceride levels by controlling the expression of apolipoprotein C3, or ApoC3, angiopoietin-like protein 3, or Angptl3, and angiopoietin-like protein 4, or Angptl4, which inhibit the activity of lipoprotein lipase, or LPL, an enzyme responsible for the breakdown of triglycerides in the blood. SREBP regulates fatty acid levels by controlling the expression of fatty acid synthase and acetyl-CoA carboxylase, enzymes that play a central role in the synthesis of fatty acids and regulate fatty acid oxidation. We believe that inhibiting SREBP activity will lead to an inhibition of fatty acid synthesis and an increase in fatty acid oxidation, and will increase LPL enzyme activity to accelerate clearance of triglycerides.

SREBP activity has also been implicated in a number of other metabolic processes that may provide further therapeutic applications for our CAT-2000 series of compounds. We believe that inhibition of SREBP in the liver has the potential to enhance insulin signaling and increase glucose metabolism without increasing liver fat content, which may be useful in the treatment of type 2 diabetes. We also believe that inhibition of SREBP has the potential to inhibit fatty acid synthesis and activate fatty acid oxidation to reduce liver triglyceride content, which may be useful in the treatment of fatty liver diseases. In addition, SREBP is believed to regulate Palatin-like phospholipase domain-containing protein 3, or PNPLA3, which is an enzyme found in cells that may play a role in cellular energy storage and metabolism, as well as a specific mutation of PNPLA3 that is associated with liver fat accumulation and increased risk of chronic liver diseases. As a result, we believe that the CAT-2000 series of compounds has the potential to be effective in the treatment of liver diseases in which patients may be identified by the occurrence of this specific mutation of PNPLA3, such as NASH and hepatocellular carcinoma.

CAT-2054

We are initially developing CAT-2054 for the treatment of patients with hypercholesterolemia, or elevated LDL-C, for whom existing treatments are insufficient. By modulating the SREBP pathway, CAT-2054 may inhibit production of important cholesterol metabolism proteins, such as PCSK9, HMG-CoA reductase, ATP citrate lyase and NPC1L1. In a clinical trial of our first generation SREBP modulator, CAT-2003, we observed statistically significant reductions in LDL-C, which we believe demonstrate the impact of SREBP modulation on cholesterol metabolism. Because the liver is the primary regulator of cholesterol metabolism, we specifically designed the SMART linker in CAT-2054 to deliver more of the intact conjugate to the liver than CAT-2003. We believe that CAT-2054, if approved, has the potential to be the first therapy to simultaneously modulate cholesterol synthesis, clearance and absorption. We submitted an IND to the FDA for CAT-2054 in November 2014. In January 2015, we initiated a Phase 1 clinical trial to assess the safety, tolerability and pharmacokinetics of CAT-2054 in healthy volunteers. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the trial. If the final results of this clinical trial show safety, tolerability and plasma exposure, we intend to initiate a Phase 2a clinical trial 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.

Hypercholesterolemia Market Overview

Hypercholesterolemia is a major risk factor for cardiovascular disease, or CVD, a leading cause of mortality and morbidity in the United States. Hypercholesterolemia is a complex disease involving redundant biological pathways that are tightly regulated and have built-in feedback mechanisms. Current treatment guidelines recognize lowering of LDL-C as a primary target for reducing the risk of CVD.

Several of the lipid-lowering therapies currently available or in development target proteins in the SREBP pathway:

- *Statins.* Statins are typically prescribed as first-line therapy for reducing LDL-C based on their efficacy, established safety and proven benefit in reducing cardiovascular event risk. Statins inhibit HMG-CoA reductase. Crestor® (rosuvastatin), the largest remaining branded prescription statin, generated worldwide sales of $5.5 billion for the 12-month period ended December 2014.

- *Cholesterol Absorption Inhibitors.* Ezetimibe is a cholesterol absorption inhibitor that targets NPC1L1, reducing LDL-C by inhibiting cholesterol absorption in the small intestine. It may be used alone (marketed as Zetia® or Ezetrol®), for example in statin-intolerant patients, or together with statins, such as in ezetimibe/simvastatin (marketed as Vytorin® and Inegy®), when statins alone do not adequately control cholesterol. Zetia and the combination product Vytorin together generated worldwide sales of $4.2 billion for the 12-month period ended December 2014.

- *Monoclonal antibodies against PCSK9 and inhibitors of ATP citrate lyase.* In addition to the marketed therapies, several companies are developing other agents that target the synthesis and clearance of LDL-C. Monoclonal antibodies against PCSK9 are injectable product candidates that are being evaluated as potential therapies to lower LDL-C. ATP citrate lyase inhibitors target cholesterol synthesis in the liver but at an earlier step of the pathway than statins. To date, none of these agents has received U.S. or European marketing approval.

Despite the availability of these classes of drugs that lower LDL-C, many patients are unable to achieve their LDL-C goals using the marketed therapies. A 2011 report of the Centers for Disease Control and Prevention estimated that, of the 34 million adults in the United States receiving treatment for high LDL-C, 11 million had uncontrolled LDL-C. The limitations of the efficacy of some existing therapies, including statins, may be partly the result of feedback mechanisms in the SREBP pathway, which ensure that cellular cholesterol levels are maintained at levels required for normal cellular function. For example, doubling the dose of a statin is accompanied by only an incremental 6% lowering of lipids. This non-linear decrease in LDL-C as the statin dose increases is due to feedback mechanisms that are triggered when HMG-CoA reductase is inhibited to a greater extent. As the statin dose is increased, intracellular levels of cholesterol decrease, ultimately resulting in activation of the SREBP pathway. Activated SREBP induces the expression of PCSK9 which promotes the degradation of the LDL receptor, resulting in reduced clearance of LDL-C from circulation. The feedback mechanism ensures that the cell is never completely depleted of cholesterol because cholesterol is required for cellular viability. Thus, high-dose statins trigger a feedback mechanism that counteracts their beneficial effects on lipids.

Several biotechnology and pharmaceutical companies have pursued compounds to inhibit SREBP but we believe that none have reached clinical development. The goal of these programs has been to identify small molecule drugs that can block the activity of SREBP and produce beneficial effects on lipids. Directly reducing active SREBP may have a significant benefit on LDL-C levels in circulation. SREBP modulators may work synergistically with inhibitors of proteins that are downstream of SREBP such as PCSK9, HMG-CoA reductase and ATP citrate lyase. In addition, SREBP modulators may substantially reduce feedback mechanisms that are activated by other classes of LDL-C lowering drugs such as statins and ezetimibe.

CAT-2054 for the Treatment of Hypercholesterolemia

CAT-2054 is a SMART linker conjugate designed to modulate SREBP in the liver and to reduce LDL-C levels in patients with hypercholesterolemia. We designed the SMART linker in CAT-2054 to be more stable to intracellular enzymatic cleavage than the SMART linker in CAT-2003. We have observed in preclinical studies that CAT-2054 was cleaved at a significantly slower rate than CAT-2003, and that significantly greater levels of CAT-2054 reached the liver following oral dosing than with CAT-2003.

This slower rate of cleavage enables more intact CAT-2054 to pass through the portal vein and to the liver, where SREBP controls cholesterol levels. We have observed in *in vitro* studies that, once cleaved in human liver cells, CAT-2054 inhibited the activity of SREBP by blocking its maturation, a conversion from an inactive to an active form. As a result, the amount of mature SREBP protein in the nucleus of the cells is reduced. This inhibition reduces the expression of downstream target genes in the SREBP pathway, including HMG-CoA reductase, PCSK9 and ATP citrate lyase. Based on this mechanism, we believe CAT-2054 may be effective in reducing elevated LDL-C and positively affect other metabolic parameters. If approved, CAT-2054 has the potential to be prescribed in patients whose hypercholesterolemia is inadequately controlled by statins alone or are intolerant to statins, and the potential to be used before injectable PCSK9 monoclonal antibodies.

Following the completion of Phase 2 clinical development, we intend to pursue development and commercialization collaborations with biotechnology and pharmaceutical companies to maximize the value of CAT-2054 as a treatment for hypercholesterolemia.

CAT-2054 Clinical Development

Ongoing Phase 1 Clinical Trial (CAT-2054-101)

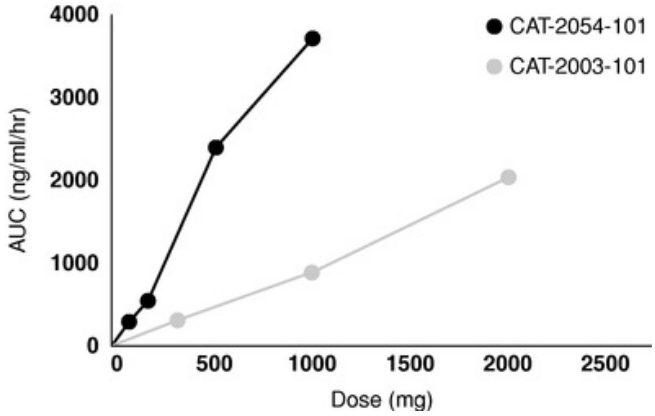
We are conducting a randomized, double-blind, placebo-controlled Phase 1 trial in healthy volunteers at a single center in the United States to assess the safety, tolerability and pharmacokinetics of single and multiple doses of CAT-2054 in both fasting and fed states. The trial includes both coated and uncoated capsule formulations, as well as multiple doses of CAT-2054 with atorvastatin to assess safety and pharmacokinetics of both compounds in combination in preparation for Phase 2 studies. Preliminary data are available for the full range of doses tested in the single and multiple ascending dose portions of the Phase 1 trial and are described below. We expect that full results will be available in the third quarter of 2015.

In the single ascending dose portion of the Phase 1 clinical trial, 38 healthy volunteers were randomized to receive CAT-2054 in capsules at doses ranging from 50 mg to 1000 mg or placebo. When single doses of CAT-2054 were administered under fed and fasted conditions, CAT-2054 was well tolerated and no serious AEs were reported. No safety signals were observed in laboratory, vital sign or electrocardiogram results following CAT-2054 administration. The observed AEs occurring under fed and fasted conditions at doses up to 500 mg were similar for CAT-2054 and placebo. The most common AEs observed in fed and fasting conditions were nausea and diarrhea and all reported AEs were mild. Of the 38 subjects, eight subjects received placebo, two of whom reported diarrhea and two of whom reported nausea. Thirty subjects received CAT-2054, of whom six reported nausea, five reported diarrhea and four reported abdominal pain. Nicotinic acid is known to interact with a specific extracellular receptor, GPR109A, and causes flushing and immediate decreases in free fatty acids, followed by a rebound. We assessed flushing using a subjective questionnaire, and administration of CAT-2054 was not associated with flushing. We also measured free fatty acid levels after administration of CAT-2054 and no differences in free fatty acid levels relative to placebo were observed. Additionally, we did not observe decreases generally associated with nicotinic acid

As shown in the graph below, in preliminary data from the single ascending dose portion of the Phase 1 clinical trial, we observed that the plasma exposure of CAT-2054 increased with dose, which

was measured using a common statistical method known as area under the curve, or AUC. The plasma exposure of CAT-2054 was greater than the plasma exposure observed for the first generation CAT-2000 product candidate, CAT-2003, in the CAT-2003-101 Phase 1 clinical trial, and consistent with our expectations for the design of the linker and the conjugate.

Plasma Exposure of CAT-2054 and CAT-2003

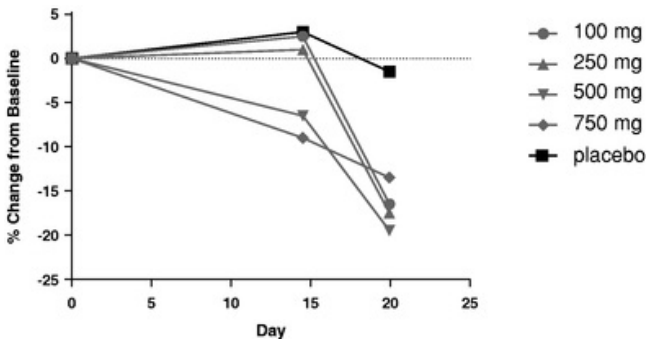


In the ongoing multiple ascending dose portion of the Phase 1 trial, approximately 60 healthy volunteers are being randomized to receive CAT-2054 in soft gelatin capsules at daily doses ranging from 100 mg to 750 mg or placebo for 14 days. Similar to the single ascending dose portion of the trial, the multiple ascending dose portion of the trial was designed to assess safety, tolerability and pharmacokinetics.

When multiple doses of CAT-2054 were administered under fed and fasted conditions, CAT-2054 was well tolerated and no serious AEs were reported. We observed no safety signals in laboratory, vital signs or electrocardiogram results following CAT-2054 administration, and all subjects completed dosing. The most common AE observed was diarrhea. Of the 40 subjects for whom results are available, 32 received CAT-2054 among the four dosing cohorts. Eight of the subjects who received CAT-2054 reported diarrhea, four reported headache and three reported abdominal cramping. All reported AEs were mild. Based on a subjective questionnaire, administration of CAT-2054 was not associated with flushing.

We also measured lipid biomarkers in the healthy volunteers enrolled in the Phase 1 trial. As shown in the graph below, in preliminary data from the multiple ascending dose portion of the Phase 1 trial, decreases in LDL-C were observed at the end of the 14-day dosing period at doses of 500 and 750 mg. Decreases in LDL-C of up to 20% were observed at day 21, which were statistically significant compared to baseline for all dose levels. We did not observe statistically significant changes in PCSK9 in this Phase 1 trial in healthy adults. Based on the results of this trial, we believe that the magnitude of LDL-C reduction with CAT-2054 may increase with continued dosing beyond 14 days. Based on our preclinical studies, we believe that patients with elevated PCSK9 levels reflective of activated SREBP, such as those on statins, may experience greater LDL-C reductions with CAT-2054.

Median LDL-C Changes*



* p<0.05 (all dose levels, day 21)

Planned Phase 2a Clinical Trial

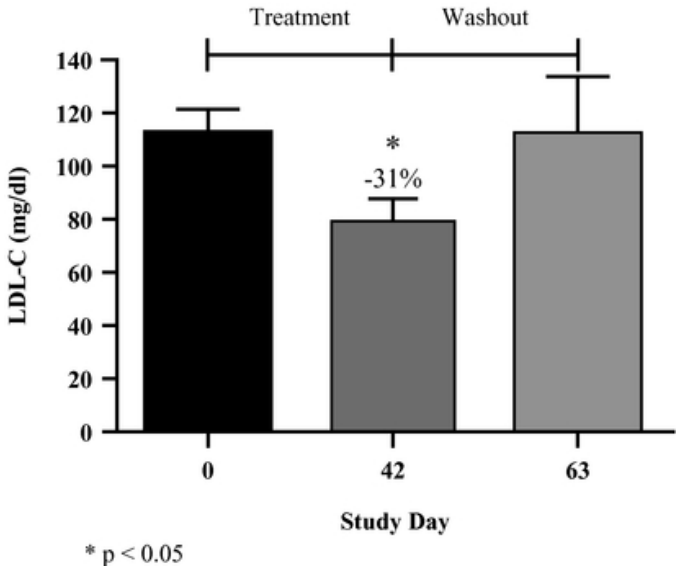
We expect to initiate a randomized, double-blind, placebo-controlled Phase 2a trial in the fourth quarter of 2015 at multiple sites in the United States in patients with hypercholesterolemia. In this clinical trial, patients will be treated for four weeks, with 25 to 30 patients in each arm. Depending on the full results of the Phase 1 trial, we intend to compare multiple dose levels of CAT-2054 in a variety of populations, such as patients on statins or those with elevated lipids, triglycerides and glucose. We expect the primary endpoint to be reduction in levels of LDL-C. We also plan to assess the safety and tolerability of CAT-2054, as well as the activity of CAT-2054 on other metabolic parameters, including triglycerides, glucose and glycosylated hemoglobin, or HbA1c, which is a measure of glucose levels over time. We anticipate that we will report data from this trial 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.

Preclinical Data for CAT-2054

Based on a comprehensive program of preclinical testing of CAT-2054, including several *in vitro* analyses and *in vivo* studies in animal models, we believe that CAT-2054 may be effective in reducing elevated LDL-C and have positive effects on other metabolic parameters. Key findings from our preclinical program included the following:

- CAT-2054 reduced LDL-C by week six in rhesus monkeys that were maintained on a high fat, high cholesterol diet. We observed no effect on food consumption or body weight. We dosed the animals with CAT-2054 at 500 mg by capsule once daily for six weeks. As shown in the graph below, at the end of the treatment period, we observed a statistically significant reduction of 31% in LDL-C levels relative to baseline. The effect of CAT-2054 on plasma LDL-C levels was most pronounced in the monkeys with the highest baseline LDL-C levels. Additionally, we observed that LDL-C levels returned to near baseline after a washout period following the end of dosing with CAT-2054.

CAT-2054 Effect on LDL-C in
Rhesus Monkeys on a High Cholesterol Diet

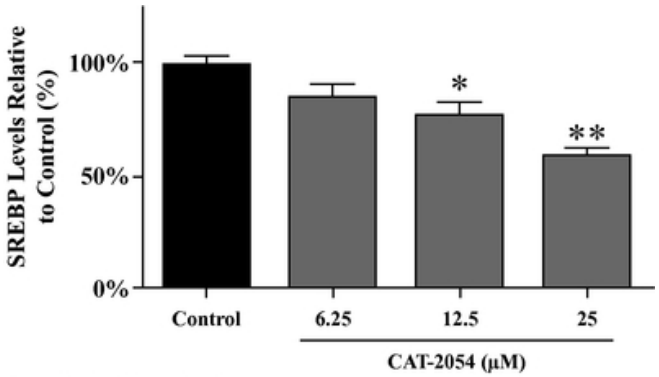


- CAT-2054 significantly reduced fasting plasma LDL-C in cynomolgus macaque monkeys that had developed age-related spontaneous dyslipidemia, which were maintained on a normal diet. In this study, we dosed the animals with CAT-2054 at 100 mg/kg by oral gavage, once daily for four weeks. We observed no effect on body weight. The mean reduction in fasting LDL-C after 14 days of treatment with CAT-2054 was 21%. The effect of CAT-2054 on plasma LDL-C levels was most pronounced in the monkeys with the highest baseline LDL-C levels. CAT-2054 treatment essentially returned LDL-C to normal levels in these monkeys without significantly decreasing LDL-C below the normal threshold.

- In *in vitro* studies, we observed that treatment of a human liver cell line with CAT-2054 reduced the amount of mature SREBP protein and that this reduction was greater than what we observed with approximately equivalent amounts of EPA and nicotinic acid administered either

alone or in combination. The graph below shows the reduction in the level of SREBP protein compared to control.

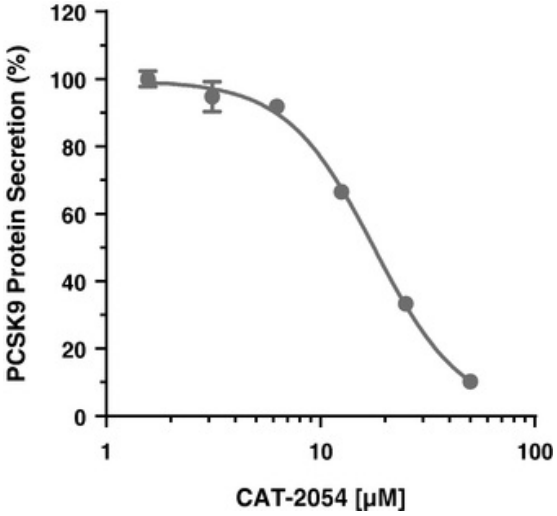
CAT-2054 Effect on Levels of the Mature SREBP Protein



* $p < 0.05$, ** $p < 0.01$

- In an *in vitro* study, we observed that treatment of a human liver cell line with CAT-2054 reduced the secretion of PCSK9 protein. As shown in the graph below, the reduction in PCSK9 protein secretion was dependent on dose of CAT-2054 with higher doses resulting in greater reductions. We also observed the bioactive components of CAT-2054, EPA and nicotinic acid, did not have a significant effect on PCSK9 secretion when administered to cells either individually or in combination at similar concentrations.

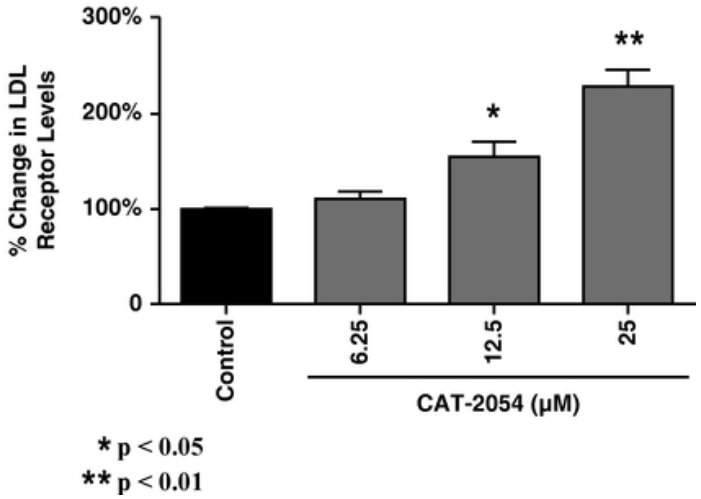
CAT-2054 Effect on Levels of PCSK9 Protein Secretion



- In an *in vitro* study, we observed that CAT-2054 induced an increase in LDL receptor protein levels on the surface of a human liver cell line. As shown in the graph below, the increase in

LDL receptor protein was dependent on the dose of CAT-2054, with higher doses resulting in greater increases.

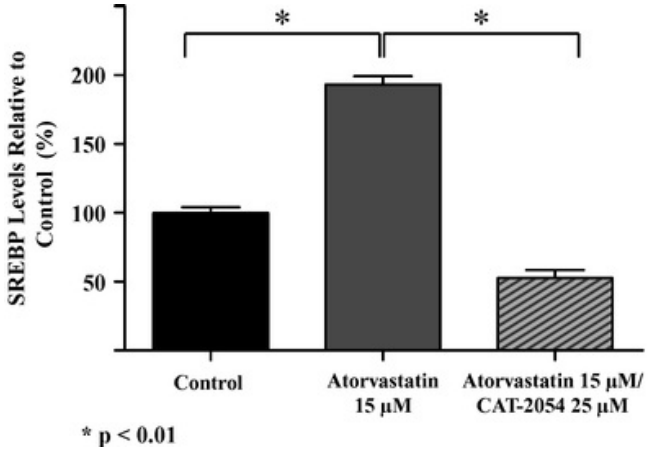
CAT-2054 Effect on Levels of the Cell Surface LDL Receptor



*** p < 0.05**
**** p < 0.01**

- In *in vitro* studies, we have observed that treatment of a human liver cell line with the statin atorvastatin caused an approximately two-fold increase in the amount of mature SREBP. As shown in the graph below, CAT-2054 inhibited the activation of SREBP2, a form of SREBP that controls the expression of genes involved in LDL-C synthesis and clearance in the liver, in the presence of atorvastatin. As expected, due to feedback mechanisms in the SREBP pathway, treatment with atorvastatin alone increased the activation of SREBP2. These data suggest that CAT-2054 may inhibit SREBP2 maturation and subsequent SREBP2-mediated gene transcription in the presence of a statin.

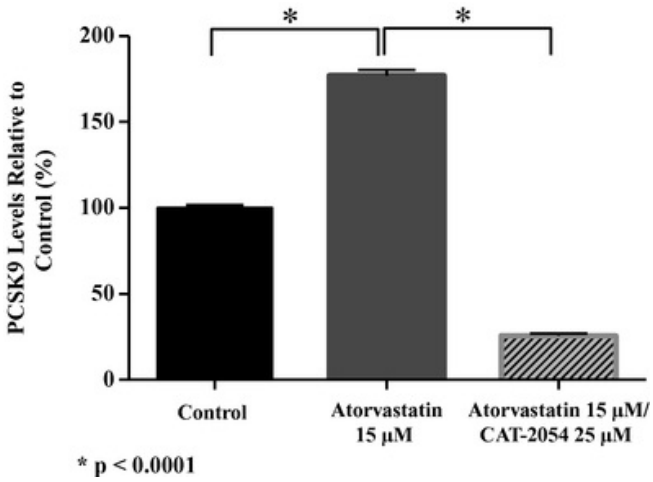
CAT-2054 Effect on SREBP2 Activation in the Presence of a Statin



*** p < 0.01**

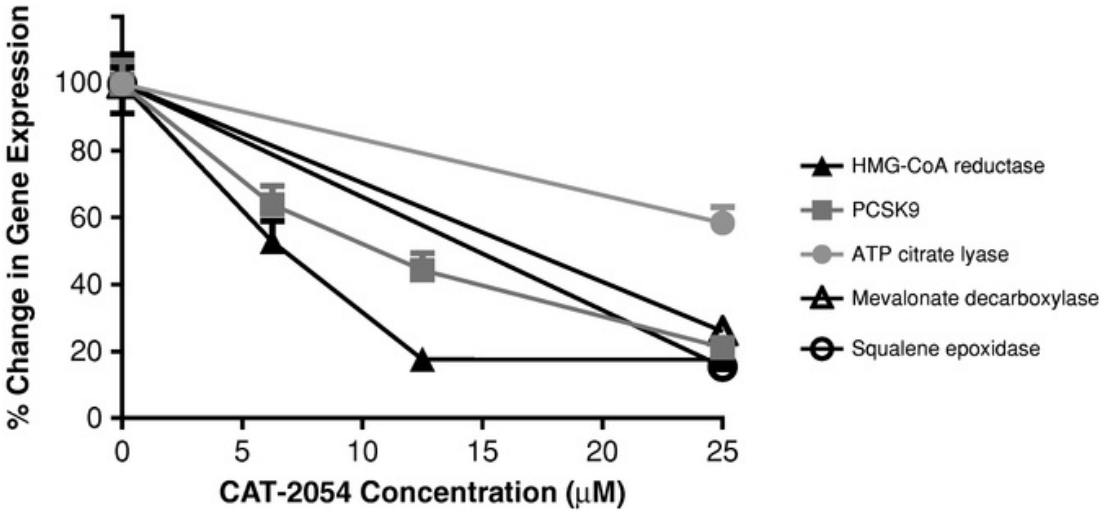
- In *in vitro* studies, we have observed that treatment of a human liver cell line with the statin atorvastatin caused an increase in the amount of secreted PCSK9. As shown in the graph below, CAT-2054 abrogated the statin-induced increase in PCSK9 secretion.

CAT-2054 Effect on PCSK9 Levels in the Presence of a Statin



- In an *in vitro* study, we observed that after a 24-hour incubation, treatment of a human liver cell line with CAT-2054 inhibited the expression of multiple SREBP2 target genes, including PCSK9 and four genes involved in cholesterol synthesis: HMG-CoA reductase, ATP citrate lyase, Mevalonate decarboxylase and Squalene expoxidase. These results are illustrated in the graph below.

CAT-2054 Effect on Expression of Genes Involved in Cholesterol Metabolism



CAT-2003

CAT-2003 is our first generation product candidate in the CAT-2000 series. We engineered CAT-2003 as an orally administered SMART linker conjugate of EPA and nicotinic acid to modulate the SREBP pathway. We designed CAT-2003 to target triglyceride levels in the blood and studied it for the treatment of MFC and rSHTG, diseases with niche patient populations with hypertriglyceridemia. We submitted an IND to the FDA for CAT-2003 in September 2012. We have completed three Phase 2a trials in patient populations with elevated triglyceride or hypertriglyceridemia in which we observed positive effects of CAT-2003 on triglycerides, LDL-C and glucose. We also observed gastrointestinal side effects. These side effects were reduced, but not eliminated, through the use of a coated soft gelatin capsule formulation with modified release characteristics.

While we have chosen to prioritize the development of CAT-2054 over CAT-2003, we believe that the clinical trial data for CAT-2003 support the utility of our SMART linker technology and the potential to treat lipid and metabolic disorders by modulating the SREBP pathway. We intend to pursue collaborations to conduct exploratory evaluation of CAT-2003 in other serious diseases that involve alterations in the SREBP pathway, such as NASH and hepatocellular carcinoma, either to develop CAT-2003 as a product candidate or to support our development efforts for CAT-2054.

Effect of CAT-2003 in Hypertriglyceridemias

We have completed three Phase 2a clinical trials of CAT-2003 in patients with elevated triglycerides and two Phase 1 clinical trials in healthy volunteers. In the Phase 2a clinical trials, CAT-2003 reduced elevated triglycerides, including in patients treated with other triglyceride and lipid lowering therapies. CAT-2003 also demonstrated in Phase 2a clinical trials beneficial effects on other lipid and cardio-metabolic parameters, such as LDL-C and blood glucose levels. In our clinical trials, CAT-2003 showed no observed trends in laboratory values, vital signs, electrocardiogram or physical examination at up to 12 weeks of patient dosing. Mild to moderate gastrointestinal tolerability issues were observed with CAT-2003 at higher doses with an uncoated soft gelatin capsule and were improved but not eliminated with a coated soft gelatin capsule formulation.

CAT-2003—Completed Clinical Trials

Phase 1 Clinical Trial in Healthy Volunteers (CAT-2003-101)

We conducted a Phase 1 trial in healthy volunteers at a single center in the United States to assess the safety, tolerability and pharmacokinetics of single and multiple doses of CAT-2003 in both fasting and fed states. We also measured CAT-2003 effects on post-prandial triglycerides and other lipid parameters. All subjects were administered 500 mg immediate release niacin in the screening period so effects on flushing could be compared to CAT-2003. In this Phase 1 trial, there were a total of 99 subjects, 79 of whom received CAT-2003.

Single Ascending Dose: 41 healthy volunteers were randomized to receive CAT-2003 in soft gelatin capsules at doses ranging from 300 mg to 2000 mg or placebo. With single doses of CAT-2003 administered under fed and fasted conditions, the most commonly reported AEs were diarrhea, which was reported in 11 subjects, nausea, which was reported in five subjects, and abdominal distention, which was reported in three subjects. The majority of AEs were mild in severity and there were no serious AEs reported. In subjects administered CAT-2003, reports of gastrointestinal AEs were higher in the fed state than in the fasted state. We observed no safety signals in laboratory, vital signs or electrocardiogram results following CAT-2003 administration in either the fasted or fed state.

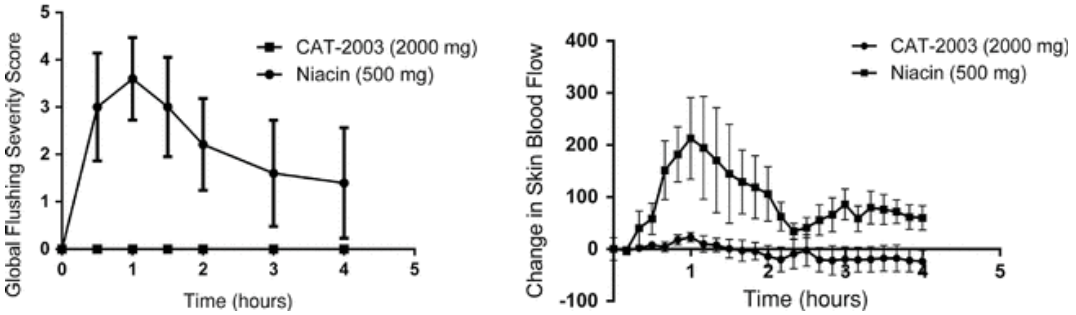
CAT-2003 was rapidly absorbed in plasma, with mean maximum and overall plasma exposure generally increasing with CAT-2003 dose. Nicotinic acid was not detected in plasma at levels above background, consistent with intracellular cleavage of CAT-2003 and intracellular delivery of the component bioactives. Exposure of CAT-2003 was similar in the fasted and fed states.

We measured postprandial triglycerides in healthy volunteers after single doses of 300 mg, 1000 mg and 2000 mg CAT-2003 and placebo were administered after a standardized high-fat meal. In subjects receiving the 300 mg dose of CAT-2003, the peak postprandial increase in plasma triglycerides was reduced by approximately 50% on average compared to subjects receiving placebo, while in subjects receiving the 1000 and 2000 mg doses of CAT-2003, the peak postprandial increase in plasma triglycerides was reduced by approximately 80% on average compared to subjects receiving placebo.

We measured flushing in subjects screened for susceptibility to niacin-induced flushing. Flushing was measured using a validated patient-reported questionnaire and also by laser Doppler after single doses of 300 mg, 1000 mg, and 2000 mg of CAT-2003 pre-dose and four hours post-dose, the general timeframe during which niacin-related flushing would be expected to be observed. No subject dosed with CAT-2003 experienced flushing at any dose. Results for the highest CAT-2003 dose, 2000 mg, are shown in the graphs below.

CAT-2003 Effect on Flushing in Subjects Screened for Niacin-Induced Flushing

Measured by Questionnaire ***Measured by Laser Doppler***



Multiple Ascending Dose: 58 healthy volunteers were administered CAT-2003 in soft gelatin capsules at doses ranging from 500 mg to 1500 mg or placebo daily for 14 days. In addition to the safety, tolerability and pharmacokinetics of CAT-2003, we assessed the activity of CAT-2003 on lipid parameters including fasting and postprandial triglycerides following 14 days of dosing.

CAT-2003 administered for 14 days demonstrated no safety signals in laboratory values, vital signs, electrocardiogram or physical examination that were considered to be drug-related AEs. No serious AEs were reported. The most common AEs were gastrointestinal, including diarrhea, which was reported in 32 subjects, nausea, which was reported in 26 subjects, vomiting, which was reported in 12 subjects, and headache, which was reported in 11 subjects. The majority of subjects at a total daily dose of 1000 mg had gastrointestinal AEs. Overall, most AEs were mild in intensity but moderate intensity gastrointestinal AEs were reported, particularly at daily doses of at least 1000 mg.

CAT-2003 was rapidly absorbed in plasma, with mean maximum and overall plasma exposure generally increasing with CAT-2003 dose. Nicotinic acid was not detected in plasma at levels above background, consistent with intracellular cleavage of CAT-2003 and intracellular delivery of the component bioactives.

We observed that postprandial triglyceride levels were reduced after two weeks of treatment with CAT-2003. Both 500 mg and 1000 mg total daily doses of CAT-2003 substantially suppressed the expected increase in postprandial lipids after the first meal of the day as compared to the typical increase in postprandial triglycerides observed in the placebo group. We did not assess the statistical significance of these data and did not measure postprandial lipids after a high-fat meal in subjects receiving 1500 mg total daily doses of CAT-2003.

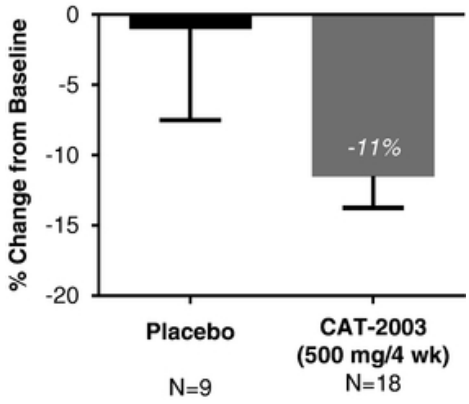
Phase 2a Clinical Trial in Patients with Hyperlipidemia (CAT-2003-201)

We conducted a Phase 2a randomized, double-blind, placebo-controlled clinical trial in 72 patients with moderate hypertriglyceridemia with baseline triglyceride levels between 200 and 500 mg/dL and in 27 patients with hypercholesterolemia, defined as LDL-C levels between 100 mg/dL and 190 mg/dL and triglycerides less than 200 mg/dL, while on a statin. We enrolled patients at 15 sites in the United States and Canada. Patients were treated for 28 days with CAT-2003 at doses of 300 mg once daily, 500 mg once daily or 300 mg twice daily, or placebo. Of the total 99 patients, 71 were treated with CAT-2003.

We observed a median 16% reduction in fasting triglycerides in patients receiving 500 mg CAT-2003. Median triglyceride reductions in the placebo, 300 mg once daily and 300 mg twice daily dosing cohorts were less than 5%. However, in a pre-specified subgroup of patients with a baseline fasting triglyceride value greater than 350 mg/dL, we observed a median 27% decrease in fasting triglycerides from baseline in all CAT-2003-treated patients, and a median 44% decrease in fasting triglycerides in patients receiving 500 mg CAT-2003. The greater effect of CAT-2003 in patients with higher baseline triglycerides is consistent with preclinical data supporting the mechanism of action of CAT-2003 in enhancing LPL activity.

As shown in the graph below in the cohort of 27 patients with hypercholesterolemia, we observed that CAT-2003 reduced LDL-C levels by a median 11% in patients who received a 500 mg once daily dose on concomitant moderate-dose statin therapy.

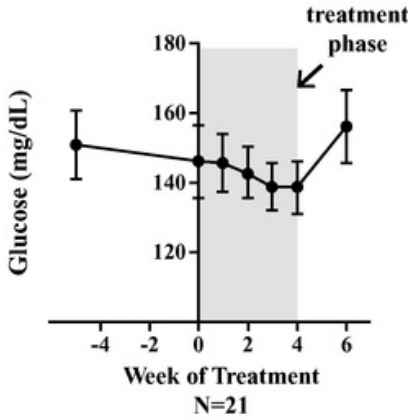
CAT-2003 Effect on LDL Cholesterol in Patients with Hypercholesterolemia



p<0.01 change from baseline
p=0.03 relative to placebo group

As shown in the graph below, in the 21 patients with Type 2 diabetes enrolled in the trial and randomized to CAT-2003, we observed reductions in fasting glucose. In addition, we observed a statistically significant reduction of 0.2% in HbA1c following four weeks of treatment, with a p-value of less than 0.01.

CAT-2003 Effect on Fasting Glucose Levels in Patients with Type 2 Diabetes



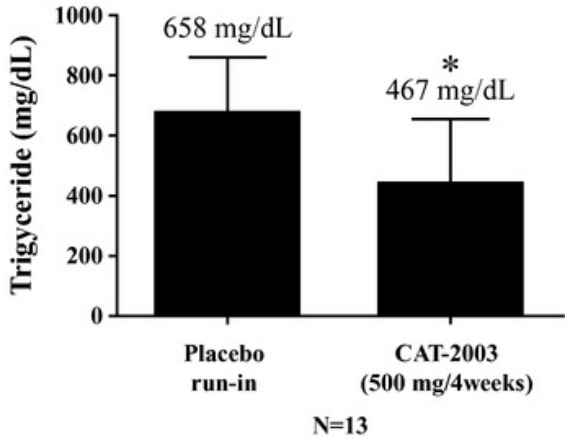
p: not significant for glucose

No serious AEs were reported. The most common AEs reported were gastrointestinal, with nausea, diarrhea, vomiting and abdominal distention the most frequently reported. Twenty-three patients reported nausea, 17 reported diarrhea, nine reported vomiting and five reported abdominal distention and most of the AEs were mild or moderate in severity. Four patients discontinued treatment because of gastrointestinal AEs and several received dose reductions because of AEs. We observed no safety signals in laboratory, vital signs or electrocardiogram results.

Phase 2a Clinical Trial in Patients with SHTG and rSHTG (CAT-2003-202)

We conducted a randomized, single-blind Phase 2a trial in 14 patients with severe hypertriglyceridemia and rSHTG, defined as fasting triglycerides greater than 500 mg/dL and either treatment naive or currently taking other triglyceride-lowering therapies, to evaluate the effect of CAT-2003 on triglyceride levels. Thirteen of the 14 patients enrolled completed dosing. This clinical trial enrolled patients at four sites in the United States. A placebo run-in for two weeks was followed by treatment with 500 mg of CAT-2003 dosed once-daily for four weeks. As illustrated in the graph below, we observed a reduction in fasting triglycerides from a median of 658 mg/dL after the placebo run-in to a median of 467 mg/dL following four weeks of CAT-2003 treatment. We observed a comparable reduction in fasting triglycerides from a median of 760 mg/dL after the placebo run-in to a median of 452 mg/dL following four weeks of CAT-2003 treatment in the four patients on concomitant fibrate or statin therapy. In the four patients with Type 2 diabetes, three of whom were also receiving metformin, we observed decreases in HbA1c. No serious AEs were reported. The most common AEs reported were gastrointestinal, with nausea and diarrhea the most frequently reported. Five patients reported nausea and four patients reported diarrhea and all of the AEs were mild in severity. We observed no safety signals in laboratory, vital signs or electrocardiogram results.

CAT-2003 Effect on Fasting Triglycerides in Patients with Hypertriglyceridemia



Median-interquartile ranges
***p<0.02**

Phase 1 Clinical Trial of the CAT-2003 Coated Capsule Formulation (CAT-2003-102)

To improve gastrointestinal tolerability observed with the uncoated soft gelatin capsule, we reformulated CAT-2003 in a gelatin capsule with a pH-sensitive polymer coating and modified release characteristics. We then conducted a Phase 1 trial in healthy volunteers to examine the safety, tolerability and pharmacokinetics of seven days of treatment with the CAT-2003 coated capsule formulation. This trial enrolled 48 subjects at one site in the United States. In this trial, the coated capsule formulation of CAT-2003 substantially reduced gastrointestinal side effects, particularly nausea and vomiting, over the seven-day treatment period while retaining the desired pharmacokinetic and pharmacodynamic profile.

Phase 2a Clinical Trial in Patients with Chylomicronemia (CAT-2003-203)

We conducted a two-part 12-week single-blind Phase 2a clinical trial of CAT-2003 in patients with chylomicronemia syndromes and rSHTG at two sites in Canada. Patients with familial chylomicronemia, or FCS, were enrolled based on previous diagnosis, and patients with multi-factorial chylomicronemia, or MFC, and rSHTG, were enrolled based on history of fasting triglycerides greater than 880 mg/dL, or, if on stable dose of fibrate therapy, documented fasting triglycerides greater than 440 mg/dL. All patients enrolled in the trial participated in a run-in period, during which patients followed a low fat diet and received placebo, followed by a 12-week CAT-2003 treatment phase. In the first part, 12 patients were dosed with the uncoated CAT-2003 capsules and in the second part seven patients were dosed with the coated capsules.

Preliminary data from the first part of the clinical trial of patients with MFC and rSHTG indicate that CAT-2003 reduced fasting triglycerides by a median of 40% at a dose of 500 mg once daily. Meaningful reductions in triglycerides were not observed in patients with FCS. We observed no safety signals in laboratory, vital signs or electrocardiogram results. The patients enrolled in the trial experienced mild to moderate gastrointestinal AEs at some point. These included nausea in nine patients, diarrhea in nine patients and vomiting in six patients. In some cases these AEs led to dose reduction or discontinuation.

In the second part of the trial, we evaluated the effect of the coated capsule formulation of CAT-2003 on gastrointestinal tolerability compared to the uncoated capsule formulation. Preliminary data demonstrated improvement in nausea events, with three patients reporting nausea, but no vomiting events. Diarrhea was observed in seven patients. As a result of these AEs, four patients received dose reductions and two discontinued.

CAT-2003 Preclinical Development

We have observed that CAT-2003 reduced fasting or post-prandial triglycerides in several *in vivo* models of hypertriglyceridemia. In some of these studies, we tested and observed that EPA and niacin, the individual bioactives, administered at doses that correspond to the top dose of CAT-2003 did not reduce triglycerides significantly. In a study using a mouse model, we also observed that CAT-2003 reduced liver inflammation and liver fat content.

We have investigated the mechanism of action of CAT-2003 based on the abilities of EPA and niacin to modulate triglyceride and cholesterol synthesis through SREBP modulation. In human cell lines, we have observed that CAT-2003 reduced the amount of mature SREBP protein and levels of ApoC3, Angptl3 and Angptl4 to a greater extent than either EPA or niacin alone or in combination, and also increased LPL activity in cells.

CAT-4001

CAT-4001 is a SMART linker conjugate of monomethyl fumarate and DHA designed to modulate the Nrf2 and NF-κB pathways. We are developing CAT-4001 initially for the treatment of severe, rare neurodegenerative diseases, such as Friedreich's ataxia and ALS, two diseases of the central nervous system in which the Nrf2 and NF-κB pathways have been implicated.

We designed CAT-4001 to combine the potentially beneficial activities of monomethyl fumarate and DHA on the Nrf2 and NF-κB pathways. Nrf2 is a transcription factor that regulates cellular response to oxidative stress. NF-κB is a transcription factor that controls cellular responses to stress and inflammation. Oxidative stress and neuroinflammation are believed to play a central role in a number of neurodegenerative diseases, including Friedreich's ataxia and ALS. In addition, monomethyl fumarate is the circulating form of the active ingredient of Tecfidera, an FDA-approved treatment for multiple sclerosis, another neurodegenerative disease. We believe that this known therapeutic effectiveness of monomethyl fumarate offers further support for the potential for CAT-4001 to be developed for the treatment of neurodegenerative diseases.

We observed in preclinical studies that CAT-4001 modulated the NF-κB pathway and the Nrf2 pathway. In cellular assays and in animal models, we observed that the activity produced by CAT-4001 was greater than that produced by the individual components, monomethyl fumarate and DHA, either alone or in combination at approximately equivalent amounts to those contained in the CAT-4001 conjugate.

Based on its mechanism of action, we believe that CAT-4001 has the potential to be a disease modifying agent in certain neurodegenerative diseases, such as Friedreich's ataxia or ALS. We plan to conduct additional preclinical evaluation of CAT-4001 in 2015, and if the results are positive we intend to advance CAT-4001 into IND-enabling studies in 2016.

Friedreich's Ataxia

Friedreich's ataxia is a rare genetic disease that causes nervous system damage and compromises motor coordination. Friedreich's ataxia is caused by a defect in the frataxin gene, which regulates iron levels in the mitochondria. In the majority of cases, the genetic defect in Friedreich's ataxia causes a reduction in the production of the frataxin protein and iron levels in mitochondria become poorly regulated. In Friedreich's ataxia, iron overload in mitochondria affects metabolism, causing oxidative

stress and ultimately damaging mitochondrial DNA. Progressive degeneration of central and peripheral nervous systems in Friedreich's ataxia patients causes impaired gait and coordination, muscle loss and fatigue. Disease progression varies, but generally, the patient is confined to a wheelchair within 10 to 20 years after the appearance of the first symptoms. Patients may become completely incapacitated in later stages of the disease.

Nrf2 regulates mitochondrial function to control cellular energy metabolism. Activation of Nrf2 increases the mitochondrial use of fatty acids and glucose, two molecules that work as cellular fuel, and increases the formation of new mitochondria. Preclinical studies indicate that genetic or pharmacologic Nrf2 activation positively regulates mitochondrial function and energy production. This activity may translate into improved physical functioning and reduced fatigue in patients with Friedreich's ataxia.

Friedreich's ataxia affects both males and females and there are approximately 6,000 patients with Friedreich's ataxia in the United States and 20,000 in the European Union.

Amyotrophic Lateral Sclerosis

ALS, sometimes called Lou Gehrig's disease or classical motor neuron disease, is a rapidly progressive, fatal neurological disease that attacks the nerve cells responsible for controlling voluntary muscles. Eventually, muscle weakness and atrophy occurs. People with ALS lose the ability to stand and walk, and use their hands and arms. In later stages of the disease, individuals have difficulty breathing as the muscles of the respiratory system weaken. Although ventilation support can enable breathing and prolong survival, it does not affect the progression of ALS. Most people with ALS die from respiratory failure, usually within three to five years of diagnosis.

According to the ALS Association, approximately 5,600 people in the United States are diagnosed with ALS each year. The incidence of ALS is two per 100,000 people, and it is estimated that as many as 30,000 Americans may have the disease at any given time. ALS occurs throughout the world and affects all racial, ethnic and socioeconomic groups.

Sales and Marketing

Given our stage of development, we have not yet established a commercial organization or distribution capabilities, nor have we entered into any collaboration or co-promotion arrangements. We plan to build focused capabilities in the United States and Canada to commercialize development programs, such as CAT-1004 for DMD, where we believe that the medical specialists for the indications are sufficiently concentrated to allow us to effectively promote the product with a targeted sales team. We also intend to enter into strategic relationships with biotechnology and pharmaceutical companies where realizing the full value of our development programs will require access to broader geographic markets or the pursuit of broader patient populations or indications. In addition, we intend to expand the drug development applications of our SMART linker technology platform through selective collaborations with leading biotechnology and pharmaceutical companies.

Manufacturing and Supply

Each of our SMART linker conjugate product candidates is a small molecule compound manufactured from component raw materials, for each of the bioactives and for the linker. The omega-3 fatty acid materials that we use as bioactives are purified from natural sources by established pharmaceutical intermediate manufacturers. The other bioactive and linker raw materials that we use are readily available from established pharmaceutical intermediate manufacturers who synthesize them using well understood, conventional chemistries. The components are conjugated to form the SMART linker product candidate using well understood, conventional chemistries.

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, collaborators to manufacture commercial quantities of our products, if approved.

Competition

The development and commercialization of new drugs is highly competitive. If we successfully develop and commercialize any of our product candidates, we and any future collaborators will face competition from pharmaceutical and biotechnology companies worldwide. Many of the entities developing and marketing potentially competing products have significantly greater financial resources and expertise than we do in research and development, manufacturing, preclinical testing, conducting clinical trials, obtaining regulatory approvals and marketing. Our commercial opportunity will be reduced or eliminated if our competitors develop and commercialize products that are more effective, have fewer side effects, are more convenient or are less expensive than any products that we may develop.

The key competitive factors affecting the success of our product candidates, if approved, are likely to be their efficacy, safety, convenience, price and the availability of coverage and reimbursement from government and other third-party payors.

CAT-1004 for Duchenne Muscular Dystrophy

There are currently no therapies approved for the treatment of DMD in the United States. Although not approved for the treatment of DMD, corticosteroid therapy is often prescribed to treat the inflammation underlying DMD and to delay loss of ambulation. Marathon Pharmaceuticals has announced that it is conducting clinical trials and preclinical studies to support approval of deflazacort, a corticosteroid, in DMD and that it anticipates filing an NDA for deflazacort with the FDA in 2016.

A number of companies are developing therapies to treat DMD in patients with specific mutations in the dystrophin gene. PTC Therapeutics has received conditional approval for Translarna™ in the European Union for DMD patients with nonsense mutations and has begun a rolling NDA submission for marketing approval in the United States. BioMarin Pharmaceuticals and Sarepta Therapeutics have product candidates in clinical development based on a different scientific approach, which is referred to as exon-skipping. BioMarin has submitted an NDA for marketing approval for drisapersen for DMD in the United States. Sarepta Therapeutics is conducting Phase 3 clinical trials of its lead product candidate eteplirsen and plans to submit the final component of the NDA by mid-2015. Based on the prevalence of the specific mutations that these product candidates are designed to address, they would be expected to be effective in an aggregate of approximately 26% of DMD patients.

Other companies have alternative therapeutic approaches to the treatment of DMD in late stage clinical development. Santhera Pharmaceuticals has announced positive effects on respiratory function in a Phase 3 clinical trial of idebenone (Raxone® in the European Union and Catena® in the United States). Santhera has announced that it plans to seek regulatory approval for the treatment of DMD in Europe and the United States. Eli Lilly is conducting a Phase 3 trial of the product tadalafil (Cialis®), which is currently approved for marketing for the treatment of erectile dysfunction, to assess whether Cialis will increase blood flow to muscles and delay the loss of ambulatory function in patients with DMD. A number of companies have products in earlier stages of clinical development for DMD, including Akashi Therapeutics, Bristol Myers Squibb, Pfizer, Phrixus Pharmaceuticals, Summit Plc and Taiho Pharmaceuticals. If successfully developed, some of these alternative therapeutic approaches may be applicable to all DMD patients.

CAT-2054 for 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.* The most advanced of these product candidates are the monoclonal antibodies alirocumab, being developed by Sanofi and Regeneron Pharmaceuticals, and evolocumab, being developed by Amgen. Both agents have completed Phase 3 clinical trials and are in the process of registration with United States and European regulatory authorities. Other PCSK9 inhibitors in clinical development include Pfizer's bococizumab, which is currently in Phase 3 clinical trials, and Eli Lilly's LY3015014, which has completed a Phase 2 clinical trial. In addition, Alnylam Pharmaceuticals has announced the filing of a Clinical Trial Application with the U.K. Medicines and Healthcare Products Regulatory Agency and initiation of a Phase 1 clinical trial of ALN-PCSsc, which targets PCSK9.

- *Cholesterol ester transfer protein (CETP) inhibitors.* CETP inhibitors are intended to reduce the risk of atherosclerosis. There are two CETP inhibitors in Phase 3 clinical trials, Merck's MK-0859 and Eli Lilly's LY2484595. Dezima Pharma's TA-8995 has completed a Phase 2b clinical trial.

Esperion is developing ETC-1002, an inhibitor of ATP citrate lyase that is currently in Phase 2b clinical trials, and Madrigal Pharmaceuticals' is developing MGL-3196, an inhibitor of thyroid hormone receptors that has completed Phase 1 clinical trials in healthy volunteers.

Intellectual Property

We strive to protect the proprietary technologies that we believe are important to our business, including pursuing and maintaining patent protection intended to cover the composition of matter of our product candidates, their methods of use, related technologies and other inventions that are important to our business. In addition to patent protection, we also rely on trade secrets to protect aspects of our business that are not amenable to, or that we do not consider appropriate for, patent protection, including certain aspects of our SMART linker technology platform.

Our commercial success depends in part upon our ability to obtain and maintain patent and other proprietary protection for commercially important technologies, inventions and know-how related to our business, defend and enforce our intellectual property rights, in particular, our patent rights, preserve the confidentiality of our trade secrets and operate without infringing valid and enforceable intellectual property rights of others.

The patent positions for biotechnology and pharmaceutical companies like us are generally uncertain and can involve complex legal, scientific and factual issues. In addition, the coverage claimed in a patent application can be significantly reduced before a patent is issued, and its scope can be reinterpreted and even challenged after issuance. As a result, we cannot guarantee that any of our product candidates will be protectable or remain protected by enforceable patents. We cannot predict whether the patent applications we are currently pursuing will issue as patents in any particular jurisdiction or whether the claims of any issued patents will provide sufficient proprietary protection from competitors. Any patents that we hold may be challenged, circumvented or invalidated by third parties.

As of April 30, 2015, our patent estate included 9 issued U.S. patents and over 25 pending U.S. patent applications, 10 issued foreign patents and 100 pending foreign patent applications.

With regard to CAT-1004, we have two issued U.S. patents and one allowed U.S. patent application with composition of matter and method of use claims directed to CAT-1004 and its use. The issued U.S. patents and the allowed U.S. patent application, when issued, are expected to expire in

2029, without taking a potential patent term extension into account. In addition, we have patents that have been granted in Australia, China, Mexico and New Zealand, which are expected to expire in 2029, without taking potential patent term extensions into account, and at least 20 pending patent applications in various other countries and regions in North America, South America, Europe, and Asia, which, if issued, are expected to expire in 2029, without taking potential patent term extensions into account.

With regard to CAT-2003 and CAT-2054, we have two issued U.S. patents with composition of matter and method of use claims directed to CAT-2003 and CAT-2054 and their use. These U.S. patents are scheduled to expire in 2030 and 2031, without taking potential patent term extensions into account. In addition, we have patents that have been granted in Australia, Mexico and New Zealand, which are expected to expire in 2030, without taking potential patent term extensions into account and at least 20 pending applications in various other countries and regions including North and South America, Europe, and Asia, which, if issued, are expected to expire in 2030, without taking patent term extensions into account. In addition, we have a pending U.S. patent application directed to CAT-2054, which, if issued, is expected to expire in 2033, without taking a potential patent term extension into account. We have at least 10 counterpart patent applications pending in various countries and regions in North America, South America, Europe and Asia, which, if issued, are expected to expire in 2033, without taking potential patent term extensions into account.

With regard to CAT-4001, we have one granted U.S. patent with composition of matter and method of use claims directed to CAT-4001 and its use. This U.S. patent is scheduled to expire in 2031, without taking a potential patent term extension into account. We have at least 20 counterpart patent applications pending in various other countries and regions in North America, South America, Europe and Asia, which, if issued, are expected to expire in 2031, without taking potential patent term extensions into account.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the earliest date of filing a non-provisional patent application.

In the United States, the term of a patent covering an FDA-approved drug may be eligible for a patent term extension under the Hatch-Waxman Act as compensation for the loss of patent term during the FDA regulatory review process. The period of extension may be up to five years beyond the expiration of the patent, but cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval. Only one patent among those eligible for an extension may be extended. Similar provisions are available in Europe and in certain other jurisdictions to extend the term of a patent that covers an approved drug. It is possible that issued U.S. patents covering CAT-1004, CAT-2003, CAT-2054 and CAT-4001 may be entitled to patent term extensions. If our product candidates receive FDA approval, we intend to apply for patent term extensions, if available, to extend the term of patents that cover the approved product candidates. We also intend to seek patent term extensions in any jurisdictions where they are available, however, there is no guarantee that the applicable authorities, including the FDA, will agree with our assessment of whether such extensions should be granted, and even if granted, the length of such extensions.

In addition to patent protection, we also rely on trade secret protection for our proprietary information that is not amenable to, or that we do not consider appropriate for, patent protection, including, for example, certain aspects of our manufacturing processes and conjugate selection methodologies. However, trade secrets can be difficult to protect. Although we take steps to protect our proprietary information, including restricting access to our premises and our confidential information, as well as entering into agreements with our employees, consultants, advisors and potential collaborators, third parties may independently develop the same or similar proprietary information or may otherwise gain access to our proprietary information. As a result, we may be unable to meaningfully protect our trade secrets and proprietary information.

Government Regulation and Product Approvals

Government authorities in the United States, at the federal, state and local level, and in other countries and jurisdictions, including the European Union, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, packaging, storage, recordkeeping, labeling, advertising, promotion, distribution, marketing, post-approval monitoring and reporting, and import and export of pharmaceutical products. The processes for obtaining regulatory approvals in the United States and in foreign countries and jurisdictions, along with subsequent compliance with applicable statutes and regulations and other regulatory authorities, require the expenditure of substantial time and financial resources.

Review and Approval of Drugs in the United States

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and implementing regulations. The failure to comply with applicable U.S. requirements at any time during the product development process, approval process or after approval may subject an applicant and/or sponsor to a variety of administrative or judicial sanctions, including refusal by the FDA to approve pending applications, withdrawal of an approval, imposition of a clinical hold, issuance of warning letters and other types of letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement of profits, or civil or criminal investigations and penalties brought by the FDA and the Department of Justice or other governmental entities.

An applicant seeking approval to market and distribute a new drug product in the United States must typically undertake the following:

- completion of preclinical laboratory tests, animal studies and formulation studies in compliance with the FDA's good laboratory practice, or GLP, regulations;
- submission to the FDA of an IND, which must take effect before human clinical trials may begin;
- approval by an independent institutional review board, or IRB, representing each clinical site before each clinical trial may be initiated;
- performance of adequate and well-controlled human clinical trials in accordance with good clinical practices, or GCP, to establish the safety and efficacy of the proposed drug product for each indication;
- preparation and submission to the FDA of an NDA;
- review of the product by an FDA advisory committee, where appropriate or if applicable;
- satisfactory completion of one or more FDA inspections of the manufacturing facility or facilities at which the product, or components thereof, are produced to assess compliance with current Good Manufacturing Practices, or cGMP, requirements and to assure that the facilities, methods and controls are adequate to preserve the product's identity, strength, quality and purity;
- satisfactory completion of FDA audits of clinical trial sites to assure compliance with GCPs and the integrity of the clinical data;
- payment of user fees and securing FDA approval of the NDA; and
- compliance with any post-approval requirements, including Risk Evaluation and Mitigation Strategies, or REMS, and post-approval studies required by the FDA.

Preclinical Studies

Preclinical studies include laboratory evaluation of the purity and stability of the manufactured drug substance or active pharmaceutical ingredient and the formulated drug or drug product, as well as *in vitro* and animal studies to assess the safety and activity of the drug for initial testing in humans and to establish a rationale for therapeutic use. The conduct of preclinical studies is subject to federal regulations and requirements, including GLP regulations. The results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and plans for clinical trials, among other things, are submitted to the FDA as part of an IND. Some long-term preclinical testing, such as animal tests of reproductive adverse events and carcinogenicity, may continue after the IND is submitted.

Human Clinical Trials in Support of an NDA

Clinical trials involve the administration of the investigational product to human subjects under the supervision of qualified investigators in accordance with GCP requirements, which include, among other things, the requirement that all research subjects provide their informed consent in writing before their participation in any clinical trial. Clinical trials are conducted under written study protocols detailing, among other things, the inclusion and exclusion criteria, the objectives of the study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated. A protocol for each clinical trial and any subsequent protocol amendments must be submitted to the FDA as part of the IND. An IND automatically becomes effective 30 days after receipt by the FDA, unless before that time the FDA raises concerns or questions related to a proposed clinical trial and places the trial on clinical hold. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical trial can begin. The FDA can place an IND on clinical hold at any point in development, and depending upon the scope of the hold, clinical trial(s) may not restart until resolution of the outstanding concerns to the FDA's satisfaction.

In addition, an IRB representing each institution participating in the clinical trial must review and approve the plan for any clinical trial before it commences at that institution, and the IRB must conduct a continuing review and reapprove the study at least annually. The IRB must review and approve, among other things, the study protocol and informed consent information to be provided to study subjects. An IRB must operate in compliance with FDA regulations. Information about certain clinical trials must be submitted within specific timeframes to the National Institutes of Health for public dissemination on their ClinicalTrials.gov website.

Human clinical trials are typically conducted in three sequential phases, which may overlap or be combined:

- ***Phase 1.*** The drug is initially introduced into healthy human subjects or, in certain indications such as cancer, patients with the target disease or condition and tested for safety, dosage tolerance, absorption, metabolism, distribution, excretion and, if possible, to gain an early indication of its effectiveness and to determine optimal dosage.

- ***Phase 2.*** The drug is administered to a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance and optimal dosage.

- ***Phase 3.*** The drug is administered to an expanded patient population, generally at geographically dispersed clinical trial sites, in well-controlled clinical trials to generate enough data to statistically evaluate the efficacy and safety of the product for approval, to establish the overall risk-benefit profile of the product and to provide adequate information for the labeling of the product.

Progress reports detailing the results of the clinical trials must be submitted at least annually to the FDA and more frequently if serious adverse events occur. In addition, IND safety reports must be submitted to the FDA for any of the following: serious and unexpected suspected adverse reactions; findings from other studies or animal or *in vitro* testing that suggest a significant risk in humans exposed to the drug; and any clinically important increase in the case of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, or at all. Furthermore, the FDA or the sponsor may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research subjects are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution, or an institution it represents, if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients. The FDA will typically inspect one or more clinical sites to assure compliance with GCP and the integrity of the clinical data submitted.

Submission of an NDA to the FDA

Assuming successful completion of required clinical testing and other requirements, the results of the preclinical studies and clinical trials, together with detailed information relating to the product's chemistry, manufacture, controls and proposed labeling, among other things, are submitted to the FDA as part of an NDA requesting approval to market the drug product for one or more indications. Under federal law, the submission of most NDAs is additionally subject to an application user fee, currently exceeding $2.1 million, and the sponsor of an approved NDA is also subject to annual product and establishment user fees, currently exceeding $104,000 per product and $554,000 per establishment. These fees are typically increased annually.

The FDA conducts a preliminary review of an NDA within 60 days of its receipt and informs the sponsor by the 74th day after the FDA's receipt of the submission to determine whether the application is sufficiently complete to permit substantive review. The FDA may request additional information rather than accept an NDA for filing. In this event, the application must be resubmitted with the additional information. The resubmitted application is also subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review. The FDA has agreed to specified performance goals in the review process of NDAs. Most such applications are meant to be reviewed within ten months from the date of filing, and most applications for "priority review" products are meant to be reviewed within six months of filing. The review process may be extended by the FDA for three additional months to consider new information or clarification provided by the applicant to address an outstanding deficiency identified by the FDA following the original submission.

Before approving an NDA, the FDA typically will inspect the facility or facilities where the product is or will be manufactured. These pre-approval inspections may cover all facilities associated with an NDA submission, including drug component manufacturing (such as active pharmaceutical ingredients), finished drug product manufacturing, and control testing laboratories. The FDA will not approve an application unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. Additionally, before approving an NDA, the FDA will typically inspect one or more clinical sites to assure compliance with GCP.

In addition, as a condition of approval, the FDA may require an applicant to develop a REMS. REMS use risk minimization strategies beyond the professional labeling to ensure that the benefits of the product outweigh the potential risks. To determine whether a REMS is needed, the FDA will consider the size of the population likely to use the product, seriousness of the disease, expected benefit of the product, expected duration of treatment, seriousness of known or potential adverse events, and whether the product is a new molecular entity. REMS can include medication guides,

physician communication plans for healthcare professionals, and elements to assure safe use, or ETASU. ETASU may include, but are not limited to, special training or certification for prescribing or dispensing, dispensing only under certain circumstances, special monitoring, and the use of patient registries. The FDA may require a REMS before approval or post-approval if it becomes aware of a serious risk associated with use of the product. The requirement for a REMS can materially affect the potential market and profitability of a product.

The FDA is required to refer an application for a novel drug to an advisory committee or explain why such referral was not made. Typically, an advisory committee is a panel of independent experts, including clinicians and other scientific experts, that reviews, evaluates and provides a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions.

Fast Track, Breakthrough Therapy and Priority Review Designations

The FDA is authorized to designate certain products for expedited review if they are intended to address an unmet medical need in the treatment of a serious or life-threatening disease or condition. These programs are fast track designation, breakthrough therapy designation and priority review designation.

Specifically, the FDA may designate a product for fast track review if it is intended, whether alone or in combination with one or more other drugs, for the treatment of a serious or life-threatening disease or condition, and it demonstrates the potential to address unmet medical needs for such a disease or condition. For fast track products, sponsors may have greater interactions with the FDA and the FDA may initiate review of sections of a fast track product's NDA before the application is complete. This rolling review may be available if the FDA determines, after preliminary evaluation of clinical data submitted by the sponsor, that a fast track product may be effective. The sponsor must also provide, and the FDA must approve, a schedule for the submission of the remaining information and the sponsor must pay applicable user fees. However, the FDA's time period goal for reviewing a fast track application does not begin until the last section of the NDA is submitted. In addition, the fast track designation may be withdrawn by the FDA if the FDA believes that the designation is no longer supported by data emerging in the clinical trial process.

Second, in 2012, Congress enacted the Food and Drug Administration Safety and Innovation Act, or FDASIA. This law established a new regulatory scheme allowing for expedited review of products designated as "breakthrough therapies." A product may be designated as a breakthrough therapy if it is intended, either alone or in combination with one or more other drugs, to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the product may demonstrate substantial improvement over existing therapies on one or more clinically significant endpoints, such as substantial treatment effects observed early in clinical development. The FDA may take certain actions with respect to breakthrough therapies, including holding meetings with the sponsor throughout the development process; providing timely advice to the product sponsor regarding development and approval; involving more senior staff in the review process; assigning a cross-disciplinary project lead for the review team; and taking other steps to design the clinical trials in an efficient manner.

Third, the FDA may designate a product for priority review if it is a drug that treats a serious condition and, if approved, would provide a significant improvement in safety or effectiveness. The FDA determines, on a case- by-case basis, whether the proposed drug represents a significant improvement when compared with other available therapies. Significant improvement may be illustrated by evidence of increased effectiveness in the treatment of a condition, elimination or substantial reduction of a treatment-limiting drug reaction, documented enhancement of patient compliance that

may lead to improvement in serious outcomes, and evidence of safety and effectiveness in a new subpopulation. A priority designation is intended to direct overall attention and resources to the evaluation of such applications, and to shorten the FDA's goal for taking action on a marketing application from ten months to six months.

Accelerated Approval Pathway

The FDA may grant accelerated approval to a drug for a serious or life-threatening condition that provides meaningful therapeutic advantage to patients over existing treatments based upon a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit. The FDA may also grant accelerated approval for such a condition when the product has an effect on an intermediate clinical endpoint that can be measured earlier than an effect on irreversible morbidity or mortality, or IMM, and that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity or prevalence of the condition and the availability or lack of alternative treatments. Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval.

For the purposes of accelerated approval, a surrogate endpoint is a marker, such as a laboratory measurement, radiographic image, physical sign or other measure that is thought to predict clinical benefit, but is not itself a measure of clinical benefit. Surrogate endpoints can often be measured more easily or more rapidly than clinical endpoints. An intermediate clinical endpoint is a measurement of a therapeutic effect that is considered reasonably likely to predict the clinical benefit of a drug, such as an effect on IMM. The FDA has limited experience with accelerated approvals based on intermediate clinical endpoints, but has indicated that such endpoints generally may support accelerated approval where the therapeutic effect measured by the endpoint is not itself a clinical benefit and basis for traditional approval, if there is a basis for concluding that the therapeutic effect is reasonably likely to predict the ultimate clinical benefit of a drug.

The accelerated approval pathway is most often used in settings in which the course of a disease is long and an extended period of time is required to measure the intended clinical benefit of a drug, even if the effect on the surrogate or intermediate clinical endpoint occurs rapidly. Thus, accelerated approval has been used extensively in the development and approval of drugs for treatment of a variety of cancers in which the goal of therapy is generally to improve survival or decrease morbidity and the duration of the typical disease course requires lengthy and sometimes large trials to demonstrate a clinical or survival benefit.

The accelerated approval pathway is usually contingent on a sponsor's agreement to conduct, in a diligent manner, additional post-approval confirmatory studies to verify and describe the drug's clinical benefit. As a result, a drug candidate approved on this basis is subject to rigorous post-marketing compliance requirements, including the completion of Phase 4 or post-approval clinical trials to confirm the effect on the clinical endpoint. Failure to conduct required post-approval studies, or confirm a clinical benefit during post-marketing studies, would allow the FDA to withdraw the drug from the market on an expedited basis. All promotional materials for drug candidates approved under accelerated regulations are subject to prior review by the FDA.

The FDA's Decision on an NDA

On the basis of the FDA's evaluation of the NDA and accompanying information, including the results of the inspection of the manufacturing facilities, the FDA may issue an approval letter or a complete response letter. An approval letter authorizes commercial marketing of the product with specific prescribing information for specific indications. A complete response letter generally outlines the deficiencies in the submission and may require substantial additional testing or information in order

for the FDA to reconsider the application. If and when those deficiencies have been addressed to the FDA's satisfaction in a resubmission of the NDA, the FDA will issue an approval letter. The FDA has committed to reviewing such resubmissions in two or six months depending on the type of information included. Even with submission of this additional information, the FDA ultimately may decide that the application does not satisfy the regulatory criteria for approval.

If the FDA approves a product, it may limit the approved indications for use for the product, require that contraindications, warnings or precautions be included in the product labeling, require that post-approval studies, including Phase 4 clinical trials, be conducted to further assess the drug's safety after approval, require testing and surveillance programs to monitor the product after commercialization, or impose other conditions, including distribution restrictions or other risk management mechanisms, including REMS, which can materially affect the potential market and profitability of the product. The FDA may prevent or limit further marketing of a product based on the results of post-market studies or surveillance programs. After approval, many types of changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to further testing requirements and FDA review and approval.

Post-Approval Requirements

Drugs manufactured or distributed pursuant to FDA approvals are subject to pervasive and continuing regulation by the FDA, including, among other things, requirements relating to recordkeeping, periodic reporting, product sampling and distribution, advertising and promotion and reporting of adverse experiences with the product. After approval, most changes to the approved product, such as adding new indications or other labeling claims, are subject to prior FDA review and approval. There also are continuing, annual user fee requirements for any marketed products and the establishments at which such products are manufactured, as well as new application fees for supplemental applications with clinical data.

In addition, drug manufacturers and other entities involved in the manufacture and distribution of approved drugs are required to register their establishments with the FDA and state agencies, and are subject to periodic unannounced inspections by the FDA and these state agencies for compliance with cGMP requirements. Changes to the manufacturing process are strictly regulated and often require prior FDA approval before being implemented. FDA regulations also require investigation and correction of any deviations from cGMP and impose reporting and documentation requirements upon the sponsor and any third-party manufacturers that the sponsor may decide to use. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance.

Once an approval is granted, the FDA may withdraw the approval if compliance with regulatory requirements and standards is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product, including adverse events of unanticipated severity or frequency, or with manufacturing processes, or failure to comply with regulatory requirements, may result in revisions to the approved labeling to add new safety information; imposition of post-market studies or clinical trials to assess new safety risks; or imposition of distribution or other restrictions under a REMS program. Other potential consequences include, among other things:

- restrictions on the marketing or manufacturing of the product, complete withdrawal of the product from the market or product recalls;
- fines, warning letters or holds on post-approval clinical trials;
- refusal of the FDA to approve pending NDAs or supplements to approved NDAs, or suspension or revocation of product license approvals;

- product seizure or detention, or refusal to permit the import or export of products; or
- injunctions or the imposition of civil or criminal penalties.

The FDA strictly regulates marketing, labeling, advertising and promotion of products that are placed on the market. Drugs may be promoted only for the approved indications and in accordance with the provisions of the approved label. The FDA and other agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses, and a company that is found to have improperly promoted off-label uses may be subject to significant liability.

In addition, the distribution of prescription pharmaceutical products is subject to the Prescription Drug Marketing Act, or PDMA, which regulates the distribution of drugs and drug samples at the federal level, and sets minimum standards for the registration and regulation of drug distributors by the states. Both the PDMA and state laws limit the distribution of prescription pharmaceutical product samples and impose requirements to ensure accountability in distribution.

Abbreviated New Drug Applications for Generic Drugs

In 1984, with passage of the Hatch-Waxman Amendments to the FDCA, Congress authorized the FDA to approve generic drugs that are the same as drugs previously approved by the FDA under the NDA provisions of the statute. To obtain approval of a generic drug, an applicant must submit an abbreviated new drug application, or ANDA, to the agency. In support of such applications, a generic manufacturer may rely on the preclinical and clinical testing previously conducted for a drug product previously approved under an NDA, known as the reference-listed drug, or RLD.

Specifically, in order for an ANDA to be approved, the FDA must find that the generic version is identical to the RLD with respect to the active ingredients, the route of administration, the dosage form, and the strength of the drug. At the same time, the FDA must also determine that the generic drug is "bioequivalent" to the innovator drug. Under the statute, a generic drug is bioequivalent to a RLD if "the rate and extent of absorption of the drug do not show a significant difference from the rate and extent of absorption of the listed drug..."

Upon approval of an ANDA, the FDA indicates whether the generic product is "therapeutically equivalent" to the RLD in its publication "Approved Drug Products with Therapeutic Equivalence Evaluations," also referred to as the "Orange Book." Physicians and pharmacists consider a therapeutic equivalent generic drug to be fully substitutable for the RLD. In addition, by operation of certain state laws and numerous health insurance programs, the FDA's designation of therapeutic equivalence often results in substitution of the generic drug without the knowledge or consent of either the prescribing physician or patient.

Under the Hatch-Waxman Amendments, the FDA may not approve an ANDA until any applicable period of non-patent exclusivity for the RLD has expired. The FDCA provides a period of five years of non-patent data exclusivity for a new drug containing a new chemical entity. For the purposes of this provision, a new chemical entity, or NCE, is a drug that contains no active moiety that has previously been approved by the FDA in any other NDA. An active moiety is the molecule or ion responsible for the physiological or pharmacological action of the drug substance. In cases where such NCE 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, in which case the applicant may submit its application four years following the original product approval.

The FDCA also provides for a period of three years of exclusivity if the NDA includes reports of one or more new clinical investigations, other than bioavailability or bioequivalence studies, that were conducted by or for the applicant and are essential to the approval of the application. This three-year exclusivity period often protects changes to a previously approved drug product, such as a new dosage form, route of administration, combination or indication. Three-year exclusivity would be available for a

drug product that contains a previously approved active moiety, provided the statutory requirement for a new clinical investigation is satisfied. Unlike five-year NCE exclusivity, an award of three-year exclusivity does not block the FDA from accepting ANDAs seeking approval for generic versions of the drug as of the date of approval of the original drug product. The FDA typically makes decisions about awards of data exclusivity shortly before a product is approved.

Hatch-Waxman Patent Certification and the 30-Month Stay

Upon approval of an NDA or a supplement thereto, NDA sponsors are required to list with the FDA each patent with claims that cover the applicant's product or an approved method of using the product. Each of the patents listed by the NDA sponsor is published in the Orange Book. When an ANDA applicant files its application with the FDA, the applicant is required to certify to the FDA concerning any patents listed for the reference product in the Orange Book, except for patents covering methods of use for which the ANDA applicant is not seeking approval. To the extent that the Section 505(b)(2) applicant is relying on studies conducted for an already approved product, the applicant is required to certify to the FDA concerning any patents listed for the approved product in the Orange Book to the same extent that an ANDA applicant would.

Specifically, the applicant must certify with respect to each patent that:

- the required patent information has not been filed;
- the listed patent has expired;
- the listed patent has not expired, but will expire on a particular date and approval is sought after patent expiration; or
- the listed patent is invalid, unenforceable or will not be infringed by the new product.

A certification that the new product will not infringe the already approved product's listed patents or that such patents are invalid or unenforceable is called a Paragraph IV certification. If the applicant does not challenge the listed patents or indicates that it is not seeking approval of a patented method of use, the ANDA application will not be approved until all the listed patents claiming the referenced product have expired (other than method of use patents involving indications for which the ANDA applicant is not seeking approval).

If the ANDA applicant has provided a Paragraph IV certification to the FDA, the applicant must also send notice of the Paragraph IV certification to the NDA and patent holders once the ANDA has been accepted for filing by the FDA. The NDA and patent holders may then initiate a patent infringement lawsuit in response to the notice of the Paragraph IV certification. The filing of a patent infringement lawsuit within 45 days after the receipt of a Paragraph IV certification automatically prevents the FDA from approving the ANDA until the earlier of 30 months after the receipt of the Paragraph IV notice, expiration of the patent, or a decision in the infringement case that is favorable to the ANDA applicant.

Pediatric Studies and Exclusivity

Under the Pediatric Research Equity Act of 2003, an NDA or supplement thereto must contain data that are adequate to assess the safety and effectiveness of the drug product for the claimed indications in all relevant pediatric subpopulations, and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. With enactment of the FDASIA in 2012, sponsors must also submit pediatric study plans prior to the assessment data. Those plans must contain an outline of the proposed pediatric study or studies the applicant plans to conduct, including study objectives and design, any deferral or waiver requests, and other information required by regulation. The applicant, the FDA, and the FDA's internal review committee must then review the

information submitted, consult with each other, and agree upon a final plan. The FDA or the applicant may request an amendment to the plan at any time.

The FDA may, on its own initiative or at the request of the applicant, grant deferrals for submission of some or all pediatric data until after approval of the product for use in adults, or full or partial waivers from the pediatric data requirements. Additional requirements and procedures relating to deferral requests and requests for extension of deferrals are contained in FDASIA. Unless otherwise required by regulation, the pediatric data requirements do not apply to products with orphan designation.

Pediatric exclusivity is another type of non-patent marketing exclusivity in the United States and, if granted, provides for the attachment of an additional six months of marketing protection to the term of any existing regulatory exclusivity, including the non-patent and orphan exclusivity. This six-month exclusivity may be granted if an NDA sponsor submits pediatric data that fairly respond to a written request from the FDA for such data. The data do not need to show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by the FDA within the statutory time limits, whatever statutory or regulatory periods of exclusivity or patent protection cover the product are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve another application.

Orphan Drug Designation and Exclusivity

Under the Orphan Drug Act, the FDA may designate a drug product as an "orphan drug" if it is intended to treat a rare disease or condition (generally meaning that it affects fewer than 200,000 individuals in the United States, or more in cases in which there is no reasonable expectation that the cost of developing and making a drug product available in the United States for treatment of the disease or condition will be recovered from sales of the product). A company must request orphan product designation before submitting an NDA. If the request is granted, the FDA will disclose the identity of the therapeutic agent and its potential use. Orphan product designation does not convey any advantage in or shorten the duration of the regulatory review and approval process.

If a product with orphan status receives the first FDA approval for the disease or condition for which it has such designation or for a select indication or use within the rare disease or condition for which it was designated, the product generally will be receiving orphan product exclusivity. Orphan product exclusivity means that the FDA may not approve any other applications for the same product for the same indication for seven years, except in certain limited circumstances. Competitors may receive approval of different products for the indication for which the orphan product has exclusivity and may obtain approval for the same product but for a different indication. If a drug or drug product designated as an orphan product ultimately receives marketing approval for an indication broader than what was designated in its orphan product application, it may not be entitled to exclusivity.

Patent Term Restoration and Extension

A patent claiming a new drug product may be eligible for a limited patent term extension under the Hatch-Waxman Act, which permits a patent restoration of up to five years for patent term lost during product development and the FDA regulatory review. The restoration period granted is typically one-half the time between the effective date of an IND and the submission date of an NDA, plus the time between the submission date of an NDA and the ultimate approval date. Patent term restoration cannot be used to extend the remaining term of a patent past a total of 14 years from the product's approval date. Only one patent applicable to an approved drug product is eligible for the extension, and the application for the extension must be submitted prior to the expiration of the patent in

question. A patent that covers multiple drugs for which approval is sought can only be extended in connection with one of the approvals. The U.S. Patent and Trademark Office reviews and approves the application for any patent term extension or restoration in consultation with the FDA.

Review and Approval of Drug Products in the European Union

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Procedures Governing Approval of Drug Products in the European Union

Pursuant to the European Clinical Trials Directive, a system for the approval of clinical trials in the European Union has been implemented through national legislation of the member states. Under this system, an applicant must obtain approval from the competent national authority of a European Union member state in which the clinical trial is to be conducted. Furthermore, the applicant may only start a clinical trial after a competent ethics committee has issued a favorable opinion. Clinical trial application must be accompanied by an investigational medicinal product dossier with supporting information prescribed by the European Clinical Trials Directive and corresponding national laws of the member states and further detailed in applicable guidance documents.

To obtain marketing approval of a product under European Union regulatory systems, an applicant must submit a marketing authorization application, or MAA, either under a centralized or decentralized procedure. The centralized procedure provides for the grant of a single marketing authorization by the European Commission that is valid for all European Union member states. The centralized procedure is compulsory for specific products, including for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, advanced therapy products and products with a new active substance indicated for the treatment of certain diseases. For products with a new active substance indicated for the treatment of other diseases and products that are highly innovative or for which a centralized process is in the interest of patients, the centralized procedure may be optional.

Under the centralized procedure, the Committee for Medicinal Products for Human Use, or the CHMP, established at the EMA is responsible for conducting the initial assessment of a product. The CHMP is also responsible for several post-authorization and maintenance activities, such as the assessment of modifications or extensions to an existing marketing authorization. Under the centralized procedure in the European Union, the maximum timeframe for the evaluation of an MAA is 210 days, excluding clock stops, when additional information or written or oral explanation is to be provided by the applicant in response to questions of the CHMP. Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is of major interest from the point of view of public health and in particular from the viewpoint of therapeutic innovation. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days.

The decentralized procedure is available to applicants who wish to market a product in various European Union member states where such product has not received marketing approval in any European Union member states before. The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state designated by the applicant, known as the reference member state. Under this procedure, an applicant submits an application based on identical dossiers and related materials, including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment report and drafts of the related materials within 210 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report and related materials, each concerned member state must decide whether to approve the assessment report and related materials.

If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points are subject to a dispute resolution mechanism and may eventually be referred to the European Commission, whose decision is binding on all member states.

Data and Market Exclusivity in the European Union

In the European Union, new chemical entities qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the European Union from referencing the innovator's data to assess a generic (abbreviated) application for eight years, after which generic marketing authorization can be submitted, and the innovator's data may be referenced, but not approved for two years. The overall ten-year period will be extended to a maximum of eleven years if, during the first eight years of those ten years, the marketing authorization holder obtains an authorization for one or more new therapeutic indications which, during the scientific evaluation prior to their authorization, are held to bring a significant clinical benefit in comparison with existing therapies. Even if a compound is considered to be a new chemical entity and the sponsor is able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the product if such company can complete a full MAA with a complete database of pharmaceutical test, preclinical tests and clinical trials and obtain marketing approval of its product.

In order to market any product outside of the United States, a company must also comply with numerous and varying regulatory requirements of other countries and jurisdictions regarding quality, safety and efficacy and governing, among other things, clinical trials, marketing authorization, commercial sales and distribution of drug products. Whether or not it obtains FDA approval for a product, the company would need to obtain the necessary approvals by the comparable foreign regulatory authorities before it can commence clinical trials or marketing of the product in those countries or jurisdictions. The approval process ultimately varies between countries and jurisdictions and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries and jurisdictions might differ from and be longer than that required to obtain FDA approval. Regulatory approval in one country or jurisdiction does not ensure regulatory approval in another, but a failure or delay in obtaining regulatory approval in one country or jurisdiction may negatively impact the regulatory process in others.

Periods of Authorization and Renewals

Marketing authorization is valid for five years in principle and the marketing authorization may be renewed after five years on the basis of a re-evaluation of the risk-benefit balance by the EMA or by the competent authority of the authorizing member state. To this end, the marketing authorization holder must provide the EMA or the competent authority with a consolidated version of the file in respect of quality, safety and efficacy, including all variations introduced since the marketing

authorization was granted, at least six months before the marketing authorization ceases to be valid. Once renewed, the marketing authorization is valid for an unlimited period, unless the European Commission or the competent authority decides, on justified grounds relating to pharmacovigilance, to proceed with one additional five-year renewal. Any authorization which is not followed by the actual placing of the drug on the European Union market (in case of centralized procedure) or on the market of the authorizing member state within three years after authorization ceases to be valid (the so-called sunset clause).

Orphan Drug Designation and Exclusivity

Regulation 141/2000 provides that a drug shall be designated as an orphan drug if its sponsor can establish: that the product is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting not more than five in ten thousand persons in the European Community when the application is made, or that the product is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition in the European Community and that without incentives it is unlikely that the marketing of the drug in the European Community would generate sufficient return to justify the necessary investment. For either of these conditions, the applicant must demonstrate that there exists no satisfactory method of diagnosis, prevention or treatment of the condition in question that has been authorized in the European Community or, if such method exists, the drug will be of significant benefit to those affected by that condition.

Regulation 847/2000 sets out criteria and procedures governing designation of orphan drugs in the European Union. Specifically, an application for designation as an orphan product can be made any time prior to the filing of an application for approval to market the product. Marketing authorization for an orphan drug leads to a ten-year period of market exclusivity. This period may, however, be reduced to six years if, at the end of the fifth year, it is established that the product no longer meets the criteria for orphan drug designation, for example because the product is sufficiently profitable not to justify market exclusivity. Market exclusivity can be revoked only in very selected cases, such as consent from the marketing authorization holder, inability to supply sufficient quantities of the product, demonstration of "clinically relevant superiority" by a similar medicinal product, or, after a review by the Committee for Orphan Medicinal Products, requested by a member state in the fifth year of the marketing exclusivity period (if the designation criteria are believed to no longer apply). Medicinal products designated as orphan drugs pursuant to Regulation 141/2000 shall be eligible for incentives made available by the European Community and by the member states to support research into, and the development and availability of, orphan drugs.

Pharmaceutical Coverage, Pricing and Reimbursement

Significant uncertainty exists as to the coverage and reimbursement status of products approved by the FDA and other government authorities. Sales of products will depend, in part, on the extent to which third-party payors, including government health programs in the United States such as Medicare and Medicaid, commercial health insurers and managed care organizations, provide coverage, and establish adequate reimbursement levels for, such products. The process for determining whether a payor will provide coverage for a product may be separate from the process for setting the price or reimbursement rate that the payor will pay for the product once coverage is approved. Third-party payors are increasingly challenging the prices charged for medical products and services and imposing controls to manage costs. Third-party payors may limit coverage to specific products on an approved list, or formulary, which might not include all of the approved products for a particular indication.

In order to secure coverage and reimbursement for any product that might be approved for sale, a company may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost-effectiveness of the product, in addition to the costs required to obtain FDA

or other comparable regulatory approvals. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Third-party reimbursement may not be sufficient to maintain price levels high enough to realize an appropriate return on investment in product development.

The containment of healthcare costs also has become a priority of federal, state and foreign governments and the prices of drugs have been a focus in this effort. Governments have shown significant interest in implementing cost-containment programs, including price controls, restrictions on reimbursement and requirements for substitution of generic products. Adoption of price controls and cost-containment measures, and adoption of more restrictive policies in jurisdictions with existing controls and measures, could further limit our net revenue and results.

Outside the United States, ensuring adequate coverage and payment for our product candidates will face challenges. Pricing of prescription pharmaceuticals is subject to governmental control in many countries. Pricing negotiations with governmental authorities can extend well beyond the receipt of regulatory marketing approval for a product and may require us to conduct a clinical trial that compares the cost effectiveness of our product candidates or products to other available therapies. The conduct of such a clinical trial could be expensive and result in delays in our commercialization efforts.

In the European Union, pricing and reimbursement schemes vary widely from country to country. Some countries provide that drug products may be marketed only after a reimbursement price has been agreed. Some countries may require the completion of additional studies that compare the cost-effectiveness of a particular drug candidate to currently available therapies. For example, the European Union provides options for its member states to restrict the range of drug products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. European Union member states may approve a specific price for a drug product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the drug product on the market. Other member states allow companies to fix their own prices for drug products, but monitor and control company profits. The downward pressure on health care costs in general, particularly prescription drugs, has become intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert competitive pressure that may reduce pricing within a country. Any country that has price controls or reimbursement limitations for drug products may not allow favorable reimbursement and pricing arrangements.

Healthcare Law and Regulation

Healthcare providers, physicians and third-party payors play a primary role in the recommendation and prescription of drug products that are granted marketing approval. Arrangements with third-party payors and customers are subject to broadly applicable fraud and abuse and other healthcare laws and regulations. Such restrictions under applicable federal and state healthcare laws and regulations, include the following:

- the federal Anti-Kickback Statute, which 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 either the referral of an individual for, or the purchase, order or recommendation of, any good or service, for which payment may be made, in whole or in part, under a federal healthcare program such as Medicare and Medicaid;

- the federal civil and criminal false claims laws, including the civil False Claims Act, and civil monetary penalties laws, which prohibit individuals or entities from, among other things, knowingly presenting, or causing to be presented, to the federal government, claims for payment that are false or fraudulent or making a false statement to avoid, decrease or conceal an obligation to pay money to the federal government;

- the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created federal criminal laws that prohibit, among other things, knowingly and willingly executing, or attempting to execute, a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;

- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act and its implementing regulations, which also imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security and transmission of individually identifiable health information;

- the federal false statements statute, which prohibits knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false statement in connection with the delivery of or payment for healthcare benefits, items or services;

- the federal transparency requirements under the Patient Protection and Affordable Care Act, as amended by the Health Care Education Reconciliation Act, or the Affordable Care Act, which requires certain manufacturers of drugs, devices, biologics and medical supplies to report to the Department of Health and Human Services information related to payments and other transfers of value to physicians and teaching hospitals and physician ownership and investment interests; and

- analogous state and foreign laws and regulations, such as state anti-kickback and false claims laws, which may apply to healthcare items or services that are reimbursed by non-governmental third-party payors, including private insurers.

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 in addition to requiring drug manufacturers to report information related to payments to physicians and other health care providers or marketing expenditures. State and foreign 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 preempted by HIPAA, thus complicating compliance efforts.

Healthcare Reform

There have been a number of federal and state proposals during the last few years regarding the pricing of pharmaceutical and biopharmaceutical products, government control and other changes to the healthcare system in the United States.

By way of example, the United States and state governments continue to propose and pass legislation designed to reduce the cost of healthcare. In March 2010, the United States Congress enacted the Affordable Care Act, which, among other things, includes changes to the coverage and payment for drug products under government health care programs. The Affordable Care Act:

- expanded manufacturers' rebate liability under the Medicaid Drug Rebate Program by increasing the minimum rebate for both branded and generic drugs and revising the definition of "average manufacturer price," or AMP, for calculating and reporting Medicaid drug rebates on outpatient prescription drug prices;

- addressed a new methodology by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected;

- expanded the types of entities eligible for the 340B drug discount program; and

- established the Medicare Part D coverage gap discount program by requiring manufacturers to provide a 50% point-of-sale-discount off the negotiated price of applicable brand drugs to eligible beneficiaries during their coverage gap period as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D.

Other legislative changes have been proposed and adopted in the United States 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, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers 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. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare.

Employees

As of April 30, 2015, we had 34 employees, 23 of whom were primarily engaged in research and development activities. A total of 17 employees have Ph.D. degrees. None of our employees is represented by a labor union and we believe our relations with our employees are good.

Facilities

Our offices are located in Cambridge, Massachusetts and consist of approximately 15,000 square feet of leased office and laboratory space. The lease expires in June 2017.

Legal Proceedings

We are not currently a party to any material legal proceedings.

MANAGEMENT

The following table sets forth the name, age as of May 1, 2015 and position of each of our executive officers and directors.

Name	Age	Position
Jill C. Milne, Ph.D.	47	President, Chief Executive Officer and Director
Ian C. Sanderson	54	Chief Financial Officer and Treasurer
Michael Jirousek, Ph.D.	56	Chief Scientific Officer
Joanne M. Donovan, M.D., Ph.D.	58	Chief Medical Officer
Rick Modi	46	Chief Business Officer
Michael Ross, Ph.D.(1)(3)	65	Chairman of the Board of Directors
Nicholas Galakatos, Ph.D.(2)(3)	57	Director
Jean George(3)	57	Director
Ron Laufer, M.D.(1)	48	Director
Kenneth Bate(1)(2)	64	Director

(1) Member of the Audit Committee

(2) Member of the Compensation Committee

(3) Nominating and Corporate Governance Committee

Jill C. Milne, Ph.D., is a co-founder of our company and has served as a member of our board of directors and as our President and Chief Executive Officer since June 2008. Prior to co-founding our company, Dr. Milne worked as head of discovery biology at Sirtris Pharmaceuticals, a biotechnology company, from 2004 to 2008, when it was acquired by GlaxoSmithKline. From 1998 to 2004, Dr. Milne worked at Pfizer Global Research and Development, where she served as the worldwide head of the Drug Pfinder Program and head of the Enzyme Target Group at the Pfizer Discovery Technology Center in Cambridge, Massachusetts. Prior to joining Pfizer, she was an American Cancer Society postdoctoral fellow in the department of biological chemistry and molecular pharmacology at Harvard Medical School from 1995 to 1998. Dr. Milne holds a Ph.D. from Harvard University and a B.A. in biological chemistry from Wellesley College. We believe that Dr. Milne is qualified to serve on our board of directors because of her extensive leadership experience in the life sciences industry and her extensive knowledge of our company based on her role as co-founder and Chief Executive Officer.

Ian C. Sanderson has served as our Chief Financial Officer since December 2013 and our Treasurer since January 2014. Prior to joining Catabasis, Mr. Sanderson worked as a senior advisor at JSB-Partners, L.P., a global life sciences advisory firm specializing in strategic partnering and mergers and acquisitions transactions, from August 2012 to August 2013. From 1992 to August 2012, Mr. Sanderson worked at at Cowen and Company, LLC, a financial services firm, in positions of increasing responsibility, ultimately as a managing director and senior research analyst. Prior to Cowen, Mr. Sanderson worked at Houlihan Lokey Howard and Zukin, a financial services firm, from 1989 to 1992, at Cambridge Associates, an investment consulting firm, from 1985 to 1987, and at U.S. Surgical Corporation (now Covidien), a global healthcare products company, from 1983 to 1985. Mr. Sanderson holds an M.B.A. from The Wharton School of the University of Pennsylvania and a B.A. in political economy from Williams College.

Michael Jirousek, Ph.D., is a co-founder of our company and has served as our Chief Scientific Officer since June 2008. From 2006 to 2008, Dr. Jirousek served as senior vice president of research at Sirtris Pharmaceuticals. From 2001 to 2006, Dr. Jirousek served as Senior Director and Head of the Diabetes Therapeutic Area for Pfizer Inc., a pharmaceutical company. From 1998 to 2001, Dr. Jirousek was at Abbott Laboratories, serving as a Metabolic Department Head, and prior to that he worked at Eli Lilly as a Scientist and Program Leader from 1993 to 1998 and at American Home Products

Cyanamid as a Medicinal Chemist from 1991 to 1993. Dr. Jirousek was a Post-Doctoral fellow at Harvard University from 1989 to 1991 and holds a Ph.D. in Chemistry from Case Western University and a B.S. in Chemistry from Kent State University.

Joanne M. Donovan, M.D., Ph.D., has served as our Chief Medical Officer since July 2011. In addition, since 1989, she worked as a staff physician at the VA Boston Healthcare System, where she was formerly Chief of Gastroenterology. Dr. Donovan has held an appointment at Harvard Medical School since 1990, most recently as associate clinical professor of medicine. From 1998 to July 2011, Dr. Donovan served in positions of increasing responsibility, ultimately as vice president of clinical development, at Genzyme Corporation, a publicly traded biotechnology company, which she joined through its acquisition of GelTex. Dr. Donovan holds a Ph.D. in medical engineering and medical physics from the Massachusetts Institute of Technology, an M.D. from Harvard Medical School and an S.B. from the Massachusetts Institute of Technology. She completed residency training in internal medicine and a fellowship in gastroenterology at the Brigham and Women's Hospital.

Rick Modi has served as our Chief Business Officer since January 2015. Prior to joining Catabasis, Mr. Modi worked as the Senior Vice President of Global Marketing at InterMune, Inc., which was acquired by Hoffman-La Roche AG in 2014, from July 2013 to January 2015. From February 2008 to July 2013, Mr. Modi worked at MedImmune, LLC, a wholly-owned biologics research and development subsidiary of AstraZenca plc, in positions of increasing responsibility, ultimately as the Vice President of Corporate Strategy and Portfolio Management. From January 2002 to February 2008, Mr. Modi worked at Janssen Biotech, Inc. (formerly Centocor Biotech, Inc.), a wholly-owned biotechnology subsidiary of Johnson & Johnson, serving in several positions, including the Associate Director of Global Market Development. Mr. Modi holds an M.B.A. from The Wharton School of the University of Pennsylvania and a B.S. in pharmacy from the University of Iowa.

Michael Ross, Ph.D., has served as a member of our board of directors since April 2010 and as Chairman since October 2010. Since 2002, Dr. Ross has served as a Managing Partner at SV Life Sciences Advisers, LLC, a venture capital firm that he joined as a venture partner in 2001. Previously, Dr. Ross served as the Chief Executive Officer of CyThera, Carta Proteomics, MetaXen and Arris Pharmaceutical. Earlier in his career, Dr. Ross was employed at Genentech, serving in several roles, including Vice President of Development and later Vice President of Medicinal and Biomolecular Chemistry. Dr. Ross currently serves on the board of directors of Ophthotech Corporation as well as the boards of directors of several private companies, including Adimab and Sutro Biopharma, and the Board of Overseers of the Thayer School of Engineering at Dartmouth College. Dr. Ross received an A.B. from Dartmouth College and a Ph.D. in chemistry from the California Institute of Technology and completed post doctorate training in molecular biology at Harvard University. We believe that Dr. Ross is qualified to serve on our board of directors because of his extensive executive leadership experience and knowledge of the life sciences industry and his service on the board of directors of other life sciences companies.

Nicholas Galakatos, Ph.D., has served as a member of our board of directors and chair of the compensation committee since February 2012. Dr. Galakatos is a co-founder of Clarus Ventures, a health care and life science venture capital firm, where he has served as Managing Director since its inception in 2005. Dr. Galakatos has also served as a General Partner of MPM Asset Management II LLC since 2000 and as a General Partner of BioVentures III GP, LP since 2002. From 1997 to 2000, Dr. Galakatos served as Vice President, New Business and a member of the Management Team at Millennium Pharmaceuticals. He was a founder of Millennium Predictive Medicine and TransForm Pharmaceuticals, where he was also the Chairman and founding chief executive officer. Dr. Galakatos is a director of Portola Pharmaceuticals, NanoString Technologies and Ophthotech Corporation, and formerly was the Lead Director of Affymax and a director of Critical Therapeutics and Aveo Pharmaceuticals. Dr. Galakatos received a B.A. in chemistry from Reed College and a Ph.D. in organic chemistry from the Massachusetts Institute of Technology, and performed postdoctoral studies in

molecular biology at Harvard Medical School. We believe that Dr. Galakatos is qualified to serve on our board of directors because of his extensive experience as a venture capital investor and a director of several public companies.

Jean George has served as a member of our board of directors since October 2013. Since February 2002, she has been a Managing Director at Advanced Technology Ventures, a venture capital fund, where she currently serves as the East Coast lead partner for healthcare investments. Since March 2012, Ms. George has served as Managing Director at LSV Capital Management, a venture capital firm. Ms. George currently serves as a member of the board of directors of the public companies Acceleron Pharma, Calithera Biosciences and Zeltiq Aesthetics, and previously served as a member of the board of directors of Portola Pharmaceuticals from 2005 to 2013. Ms. George also serves on the boards of the private companies Hydra Biosciences and Thrasos Innovation. Ms. George holds a B.S. in Biology from the University of Maine and an M.B.A. from Simmons College Graduate School of Management. Because of Ms. George's extensive investment and financial experience, we believe she is able to add valuable expertise in guiding the strategic direction of our board of directors.

Ron Laufer, M.D., has served as a member of our board of directors since April 2011. Dr. Laufer has worked as the Senior Managing Director at MedImmune Ventures, Inc. since 2010. He served as an adjunct professor of business administration at the Kelley School of Business at Indiana University from 2009 to 2014. From 2007 to 2008, he served as a managing director at Visium Asset Management, a healthcare-focused investment firm, and co-founded Lilly Ventures, the venture capital arm of Eli Lilly & Company, in 2001. Dr. Laufer currently serves as a member of the boards of directors of the private companies Adheron Therapeutics, G1 Therapeutics, NeuProtect and VentiRx Pharmaceuticals. Dr. Laufer received his B.Sci., M.D. and M.P.H. from Hebrew University, and his M.B.A. from the Harvard Business School. We believe that Dr. Laufer's management experience and more than 18 years of experience in life sciences make him a valuable contributor to our board of directors.

Kenneth Bate has served as a member of our board of directors since January 2014. Mr. Bate is currently an independent consultant. From May 2009 until January 2012, Mr. Bate was the President and Chief Executive Officer of Archemix, a privately held biotechnology company. From January 2007 to April 2009, Mr. Bate was President and Chief Executive Officer of NitroMed, a public pharmaceutical company. From March 2006 until January 2007, Mr. Bate was Chief Operating Officer and Chief Financial Officer of NitroMed. From January 2005 to March 2006, Mr. Bate was employed at JSB-Partners, a firm that he co-founded. From 2002 to January 2005, Mr. Bate was head of commercial operations and Chief Financial Officer at Millennium Pharmaceuticals. Mr. Bate has served as a member of the Board of Directors of Cubist Pharmaceuticals, Inc., a public biopharmaceutical company, since June 2003 and as its non-executive Chair since March 2011. Mr. Bate is a director of four other public biopharmaceutical companies, AVEO Pharmaceuticals, BioMarin Pharmaceuticals, Genocea Biosciences and Epizyme Pharmaceuticals. During the last five years, Mr. Bate also served as a director of NitroMed and Coley Pharmaceutical Group, a biopharmaceutical company, which was a public company during the period of Mr. Bate's service. He holds a B.A. in Chemistry from Williams College and an M.B.A. from The Wharton School of the University of Pennsylvania. We believe Mr. Bate's qualifications to serve on our board of directors include his operating, finance, commercial, transactional and senior management experience in the industry, as well as his experience serving on the boards of directors of other public companies in the life sciences industry.

Board Composition and Election of Directors

Board Composition

Our board of directors currently consists of six members. Our directors hold office until their successors have been elected and qualified or until the earlier of their resignation or removal.

Our certificate of incorporation and bylaws that will become effective upon the closing of this offering provide that the authorized number of directors may be changed only by resolution of our board of directors. Our certificate of incorporation and bylaws will also provide that our directors may be removed only for cause by the affirmative vote of the holders of 75% of our shares of capital stock present in person or by proxy and entitled to vote, and that any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, may be filled only by vote of a majority of our directors then in office.

In accordance with the terms of our certificate of incorporation and bylaws that will become effective upon the closing of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms. Upon the closing of this offering, the members of the classes will be divided as follows:

- the class I directors will be Jill C. Milne and Ron Laufer, and their term will expire at the annual meeting of stockholders to be held in 2016;
- the class II directors will be Nicholas Galakatos and Jean George, and their term will expire at the annual meeting of stockholders to be held in 2017; and
- the class III directors will be Kenneth Bate and Michael Ross, and their term will expire at the annual meeting of stockholders to be held in 2018.

Upon the expiration of the term of a class of directors, directors in that class will be eligible to be elected for a new three-year term at the annual meeting of stockholders in the year in which their term expires.

Director Independence

Applicable NASDAQ Stock Market, or NASDAQ, rules require a majority of a listed company's board of directors to be comprised of independent directors within one year of listing. In addition, the NASDAQ rules require that, subject to specified exceptions, each member of a listed company's audit, compensation and nominating and corporate governance committees be independent under the Securities Exchange Act of 1934, as amended, or the Exchange Act. Audit committee members must also satisfy the independence criteria set forth in Rule 10A-3 under the Exchange Act, and compensation committee members must also satisfy the independence criteria set forth in Rule 10C-1 under the Exchange Act. Under applicable NASDAQ rules, a director will only qualify as an "independent director" if, in the opinion of the listed company's board of directors, that person does not have a relationship that would interfere with the exercise of independent judgment in carrying out the responsibilities of a director. In order to be considered independent for purposes of Rule 10A-3, a member of an audit committee of a listed company may not, other than in his or her capacity as a member of the audit committee, the board of directors, or any other board committee, accept, directly or indirectly, any consulting, advisory, or other compensatory fee from the listed company or any of its subsidiaries or otherwise be an affiliated person of the listed company or any of its subsidiaries. In order to be considered independent for purposes of Rule 10C-1, the board must consider, for each member of a compensation committee of a listed company, all factors specifically relevant to determining whether a director has a relationship to such company which is material to that director's ability to be independent from management in connection with the duties of a compensation committee member, including, but not limited to: the source of compensation of the director, including any consulting advisory or other compensatory fee paid by such company to the director; and whether the director is affiliated with the company or any of its subsidiaries or affiliates.

In March 2015, our board of directors undertook a review of the composition of our board of directors and its committees and the independence of each director. Based upon information requested from and provided by each director concerning his or her background, employment and affiliations,

including family relationships, our board of directors has determined that each of our directors, with the exception of Dr. Milne, is an "independent director" as defined under applicable NASDAQ rules, including, in the case of all the members of our audit committee, the independence criteria set forth in Rule 10A-3 under the Exchange Act, and in the case of all the members of our compensation committee, the independence criteria set forth in Rule 10C-1 under the Exchange Act. In making such determination, our board of directors considered the relationships that each such non-employee director has with our company and all other facts and circumstances that our board of directors deemed relevant in determining his or her independence, including the beneficial ownership of our capital stock by each non-employee director. Dr. Milne is not an independent director under these rules because she is our President and Chief Executive Officer.

There are no family relationships among any of our directors or executive officers.

Board Committees

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee. Each of these committees will operate under a charter that has been approved by our board of directors. The composition of each committee will be effective as of the date of this prospectus.

Audit Committee

The members of our audit committee are Kenneth Bate, Ron Laufer and Michael Ross, and Mr. Bate is the chair of the audit committee. Effective at the time of this offering, our audit committee's responsibilities will include:

- appointing, approving the compensation of, and assessing the independence of our registered public accounting firm;
- overseeing the work of our independent registered public accounting firm, including through the receipt and consideration of reports from that firm;
- reviewing and discussing with management and our independent registered public accounting firm our annual and quarterly financial statements and related disclosures;
- monitoring our internal control over financial reporting, disclosure controls and procedures and code of business conduct and ethics;
- overseeing our internal audit function, if any;
- overseeing our risk assessment and risk management policies;
- establishing policies regarding hiring employees from our independent registered public accounting firm and procedures for the receipt and retention of accounting related complaints and concerns;
- meeting independently with our internal auditing staff, if any, our independent registered public accounting firm and management;
- reviewing and approving or ratifying any related person transactions; and
- preparing the audit committee report required by Securities and Exchange Commission, or SEC, rules.

All audit and non-audit services, other than *de minimis* non-audit services, to be provided to us by our independent registered public accounting firm must be approved in advance by our audit committee.

Our board of directors has determined that Kenneth Bate is an "audit committee financial expert" as defined in applicable SEC rules and that each of the members of our audit committee possesses the financial sophistication required for audit committee members under NASDAQ rules. We believe that the composition of our audit committee will meet the requirements for independence under current NASDAQ and SEC rules and regulations.

Compensation Committee

The members of our compensation committee are Nicholas Galakatos and Kenneth Bate, and Dr. Galakatos is the chair of the compensation committee. Effective at the time of this offering, our compensation committee's responsibilities will include:

- reviewing and approving, or making recommendations to our board of directors with respect to, the compensation of our chief executive officer and our other executive officers;
- overseeing an evaluation of our senior executives;
- overseeing and administering our cash and equity incentive plans;
- reviewing and making recommendations to our board of directors with respect to director compensation and management succession planning;
- reviewing and discussing annually with management our "Compensation Discussion and Analysis" disclosure if and to the extent then required by SEC rules; and
- preparing the compensation committee report if and to the extent then required by SEC rules.

We believe that the composition of our compensation committee will meet the requirements for independence under current NASDAQ and SEC rules and regulations.

Nominating and Corporate Governance Committee

The members of our nominating and corporate governance committee are Michael Ross, Nicholas Galakatos and Jean George, and Dr. Ross is the chair of the nominating and corporate governance committee. Effective at the time of this offering, our nominating and corporate governance committee's responsibilities will include:

- identifying individuals qualified to become members of our board of directors;
- recommending to our board of directors the persons to be nominated for election as directors and to each of our board's committees;
- reviewing and making recommendations to our board with respect to our board leadership structure and board committee structure;
- making recommendations to our board with respect to accepting director resignations;
- reviewing and making recommendations to our board with respect to management succession planning;
- developing and recommending to our board of directors corporate governance principles; and
- overseeing an annual evaluation of our board of directors and an annual review of succession planning for senior executives.

We believe that the composition of our nominating and corporate governance committee will meet the requirements for independence under current NASDAQ and SEC rules and regulations.

Compensation Committee Interlocks and Insider Participation

None of our executive officers serves, or in the past year has served, as a member of the board of directors or compensation committee, or other committee serving an equivalent function, of any other entity that has one or more of its executive officers serving as a member of our board of directors or our compensation committee. None of the members of our compensation committee is, or has ever been, an officer or employee of our company.

Code of Business Conduct and Ethics

We have adopted a written code of business conduct and ethics that applies to our directors, officers and employees, including our principal executive officer, principal financial officer principal accounting officer or controller, or persons performing similar functions. Following this offering, we will post a copy of the code on the Corporate Governance section of our website, which is located at www.catabasis.com. If we make any substantive amendments to, or grant any waivers from, the code of business conduct and ethics for any officer or director, we will disclose the nature of such amendment or waiver on our website or in a current report on Form 8-K.

EXECUTIVE COMPENSATION

This section describes the material elements of compensation awarded to, earned by or paid to each of our named executive officers in 2014. Our named executive officers for 2014 are Jill C. Milne, Ian C. Sanderson and Michael Jirousek. This section also provides qualitative information regarding the manner and context in which compensation is awarded to and earned by our executive officers and is intended to place in perspective the data presented in the tables and narrative that follow.

Summary Compensation Table

The following table sets forth information regarding compensation awarded to, earned by or paid to our named executive officers during 2014.

Name and principal position	Year	Salary ($)	Bonus ($)(1)	Option Awards ($)(2)	All Other Compensation ($)(3)	Total ($)
Jill C. Milne, Ph.D.(4) *President and Chief Executive Officer*	2014	375,000	120,000	302,264	1,290	798,554
Ian C. Sanderson *Chief Financial Officer and Treasurer*	2014	311,554	56,080	564,315	1,310	933,259
Michael Jirousek, Ph.D. *Chief Scientific Officer*	2014	329,197	97,936	161,208	1,290	589,631

(1) The amounts reported in the "Bonus" column represent discretionary annual cash bonuses awarded to our named executive officers.

(2) The amounts reported in the "Option Awards" column reflect the aggregate grant date fair value of share-based compensation awarded during the year computed in accordance with the provisions of Financial Accounting Standards Board Accounting Standard Codification, or ASC, Topic 718. See Note 12 to our financial statements appearing at the end of this prospectus regarding assumptions underlying the valuation of equity awards.

(3) The amounts reported in the "All Other Compensation" column reflect, for each named executive officer, the cost to us of life insurance premiums paid for the named executive officer.

(4) Dr. Milne also serves as a member of our board of directors but does not receive any additional compensation for her service as a director.

Narrative to Summary Compensation Table

In 2014, we paid annual base salaries of $375,000 to Dr. Milne, $311,554 to Mr. Sanderson and $329,197 to Dr. Jirousek. We use base salaries to recognize the experience, skills, knowledge and responsibilities required of all our employees, including our named executive officers. None of our named executive officers is currently party to an employment agreement or other agreement or arrangement that provides for automatic or scheduled increases in base salary.

We do not have a formal performance-based bonus plan. From time to time, our board of directors has approved discretionary annual cash bonuses to our named executive officers with respect to their prior year performance. Dr. Milne, Mr. Sanderson and Dr. Jirousek earned cash bonuses of $120,000, $56,080 and $97,936, respectively, for services performed during 2014.

Although we do not have a formal policy with respect to the grant of equity incentive awards to our executive officers, or any formal equity ownership guidelines applicable to them, we believe that equity grants provide our executives with a strong link to our long-term performance, create an ownership culture and help to align the interests of our executives and our stockholders. In addition, we believe that equity grants with a time-based vesting feature promote executive retention because this feature incents our executive officers to remain in our employment during the vesting period. Accordingly, our board of directors periodically reviews the equity incentive compensation of our named executive officers and from time to time may grant equity incentive awards to them in the form of stock options. In 2014, we granted Dr. Milne an option to purchase 58,365 shares of our common stock, Mr. Sanderson options to purchase an aggregate of 108,965 shares of our common stock and Dr. Jirousek an option to purchase 31,128 shares of our common stock.

Outstanding Equity Awards at Year End

The following table sets forth information regarding outstanding equity awards held by our named executive officers as of December 31, 2014, which consisted entirely of stock options:

Name	Number of Securities Underlying Unexercised Options Exercisable (#)	Number of Securities Underlying Unexercised Options Unexercisable (#)	Option Exercise Price ($/share)	Option Expiration Date
Jill C. Milne, Ph.D.	79,009(1)	5,267	1.67	2/28/2021
	16,719(2)	5,573	2.31	12/17/2022
	19,621(3)	12,856	2.31	12/17/2022
	13,706(4)	14,899	2.31	4/16/2023
	—(5)	58,365	6.81	3/18/2024
Ian C. Sanderson	22,662(6)	61,012	6.81	3/18/2024
	—(5)	25,291	6.81	3/18/2024
Michael Jirousek, Ph.D.	79,009(1)	5,267	1.67	2/28/2021
	16,719(2)	5,573	2.31	12/17/2022
	19,621(3)	12,856	2.31	12/17/2022
	13,706(4)	14,899	2.31	4/16/2023
	—(5)	31,128	6.81	3/18/2024

(1) This option was granted on March 1, 2011 and vested as to 25% of the shares on March 1, 2012 with the remaining 75% of the shares vesting in equal monthly installments thereafter through March 1, 2015.

(2) This option was granted on December 18, 2012 and vested as to 25% of the shares on December 1, 2012 with the remaining 75% of the shares vesting in equal monthly installments thereafter through December 1, 2015.

(3) This option was granted on December 18, 2012 and vested as to 25% of the shares on July 10, 2013 with the remaining 75% of the shares vesting in equal monthly installments thereafter through July 10, 2016.

(4) This option was granted on April 17, 2013 and vested as to 25% of the shares on January 18, 2014 with the remaining 75% of the shares vesting in equal monthly installments thereafter through January 18, 2017.

(5) This option was granted on March 19, 2014 and vested as to 25% of the shares on January 1, 2015 with the remaining 75% of the shares vesting in equal monthly installments thereafter through January 1, 2018.

(6) This option was granted on March 19, 2014 and vested as to 25% of the shares on November 1, 2014 with the remaining 75% of the shares vesting in equal monthly installments thereafter through November 1, 2017.

Agreements with Our Named Executive Officers

We have entered into written employment agreements or offer letters with each of our named executive officers. These agreements set forth the terms of the named executive officer's compensation, including his or her initial base salary, severance and an annual cash bonus opportunity. In addition, the agreements provide that the named executive officers are eligible to participate in company-sponsored benefit programs that are available generally to all of our employees. In connection with the commencement of their employment with us, our named executive officers executed our standard invention and non-disclosure agreement and non-competition and non-solicitation agreement.

The employment agreement with Dr. Milne provides that she is eligible to receive an annual cash bonus, as determined by the board of directors in its sole discretion, with a target of 40% of her annual base salary earned in such particular calendar year, which percentage shall be subject to adjustment from time to time by the board of directors in its sole discretion. The board of directors determines the amount of the bonus, if any, based on its assessment of Dr. Milne's performance and that of the company against appropriate goals established annually by the board of directors after consultation with Dr. Milne. Mr. Sanderson's offer letter provides for an annual cash bonus of up to 30% of his annual base salary, as determined in the sole discretion of the board of directors.

In May 2015, we entered into a letter agreement with Dr. Jirousek that provides for him to continue as our Chief Scientific Officer until September 30, 2015. Pursuant to such letter agreement, which superseded the existing employment agreement between Dr. Jirousek and us, upon the termination of his employment, Dr. Jirousek will be appointed as a member of our Scientific Advisory Board for a term ending on September 30, 2016. Dr. Jirousek will also receive the benefits described below under "Potential Payment upon Termination or Change of Control," and he will provide specified transition services.

Potential Payments upon Termination or Change in Control

Upon execution and effectiveness of a separation agreement and release of all claims, each named executive officer is entitled to severance payments if his or her employment is terminated under specified circumstances pursuant to the terms of his or her employment agreement or offer letter, subject to providing a release of claims. Severance payments to the officers could be delayed for six months in certain circumstances for compliance with Section 409A of the Internal Revenue Code of 1986, as amended, or the Code. On any termination of employment, the executive officer will receive any base salary and bonus earned but not paid through the date of termination and any vacation time accrued but not used to that date and any business expenses incurred but not un-reimbursed on the date of termination.

If we terminate Dr. Milne's or Dr. Jirousek's employment without "cause" or such named executive officer terminates his or her employment with us for "good reason," each as defined in accordance with the terms of his or her employment agreement, we will be obligated to pay, in addition to the aforementioned payments:

- an amount equal to up to 100% of his or her annual base salary, payable in equal installments in accordance with our standard payroll practices, for a period of 12 months;
- a bonus payment in an amount equal to 50% of the average annual bonus paid to the executive officer over the three calendar years preceding the year of termination, prorated for the portion of the calendar year he or she worked in the year of termination; and
- premiums for continuation health coverage under COBRA for up to 12 months.

In the event Dr. Milne or Dr. Jirousek commences any employment substantially similar to his or her employment with the company (based upon responsibility, reporting level, or compensation), any

remaining portion of the base salary payment will cease to be payable on the date 60 days after the commencement of such new employment.

Within 12 months following a change of control, under the terms of the employment agreements, if Dr. Milne's or Dr. Jirousek's employment is terminated by us or our successor without cause or by such executive officer for good reason, then all of Dr. Milne's or Dr. Jirousek's remaining unvested stock options and restricted stock will automatically vest 15 days following the execution and effectiveness of a separation agreement and release of all claims, and we will be obligated to pay an amount equal to his or her annual base salary, payable as a lump sum, a bonus payment equal to 50% of the average annual bonus paid to the executive officer over the three calendar years preceding the year of termination, and premiums for continuation health coverage under COBRA for up to 12 months.

If we terminate Mr. Sanderson's employment without "cause" as defined in his offer letter, we will be obligated to pay an amount equal to his monthly base salary, payable in installments in accordance with our standard payroll practice, for a period of nine months. In the event Mr. Sanderson commences any employment substantially similar to his employment with the company (based upon responsibility, reporting level, or compensation) within such nine-month period, any remaining payment will be reduced such that the number of months of base salary will be equal to that number of months between the date of termination and the date of commencement of such new employment.

Stock Option and Other Compensation Plans

The three equity incentive plans described in this section are our amended and restated 2008 equity incentive plan, as amended to date, or the 2008 plan, our 2015 stock incentive plan, or the 2015 plan and our 2015 employee stock purchase plan, or the 2015 ESPP. Prior to this offering, we granted awards to eligible participants under the 2008 plan. Following the closing of this offering, we expect to grant awards to eligible participants only under the 2015 plan and the 2015 ESPP.

Amended and Restated 2008 Equity Incentive Plan

The 2008 plan provides for the grant of incentive stock options, nonqualified stock options, restricted stock awards, stock appreciation rights and performance units and performance share awards. Our employees, directors, and consultants are eligible to receive awards under our 2008 plan; however, incentive stock options may only be granted to our employees. Our board of directors administers the 2008 plan.

The 2008 plan provides that a maximum of 1,843,154 shares of our common stock are authorized for issuance under the plan. The 2008 plan does not have a fixed expiration date, however, no incentive stock options may be granted under the 2008 plan after December 30, 2018 and our board of directors may amend, suspend or terminate the 2008 plan at any time.

Upon the occurrence of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we shall equitably adjust (or make substitute awards, if applicable), in the manner determined by our board of directors:

- the number and class of securities available under the 2008 plan;
- the number and class of securities and exercise price per share of each outstanding option;
- the number of shares subject to and the repurchase price per share subject to each outstanding restricted stock award; and
- the terms of each other outstanding award under the 2008 plan.

Upon the occurrence of a merger or consolidation of our company with or into another entity as a result of which all of our common stock is converted into or exchanged for the right to receive cash, securities or other property or is cancelled; an exchange of all of our common stock for cash, securities or other property pursuant to a share exchange transaction; or a liquidation or dissolution of our company, our board of directors may, on such terms as our board of directors determines, take any one or more of the following actions pursuant to the 2008 plan, as to some or all outstanding awards, other than restricted stock awards:

- provide that awards shall be assumed, or substantially equivalent awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof);
- upon written notice to a plan participant, provide that the participant's unexercised awards will terminate immediately prior to the consummation of such transaction unless exercised by the participant within a specified period;
- provide that outstanding awards shall become exercisable, realizable, or deliverable, or restrictions applicable to an award shall lapse, in whole or in part prior to or upon such transaction;
- in the event of a transaction under the terms of which holders of common stock will receive upon consummation thereof a cash payment for each share surrendered in the transaction, make or provide for a cash payment to a plan participant;
- provide that, in connection with a liquidation of dissolution of the company, awards shall convert into the right to receive liquidation proceeds; or
- any combination of the foregoing.

Our board of directors is not obligated under the 2008 plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

Upon the occurrence of any corporate transaction described above, other than our liquidation or dissolution, our repurchase and other rights under each outstanding restricted stock award will continue for the benefit of our successor and will, unless our board of directors determines otherwise, apply to the cash, securities or other property which our common stock was converted into or exchanged for in the transaction in the same manner and to the same extent as they applied to the common stock subject to the restricted stock award. Upon our liquidation or dissolution, except to the extent specifically provided to the contrary in the restricted stock award agreement or any other agreement between the plan participant and us, all restrictions and conditions on all restricted stock awards then outstanding will automatically be deemed terminated or satisfied.

Our board of directors, in its sole discretion, may accelerate the exercisability of any option or time at which any restrictions shall lapse or be removed from any restricted stock award, as the case may be.

As of April 30, 2015, there were options to purchase 1,478,731 shares of our common stock outstanding under the 2008 plan, at a weighted-average exercise price of $5.46 per share, and options to purchase 160,194 shares of our common stock had been exercised. Effective as of immediately prior to the closing of this offering, we will no longer grant stock options or other awards under the 2008 plan.

2015 Stock Incentive Plan

In May 2015 our board of directors adopted, and our stockholders have approved, the 2015 plan, to become effective in connection with this offering. The 2015 plan provides for the grant of incentive stock options, nonstatutory stock options, stock appreciation rights, awards of restricted stock, restricted

stock units, other stock-based awards. Upon the closing of this offering, the number of shares of our common stock that will be reserved for issuance under the 2015 plan will be the sum of 1,068,287 shares plus the number of shares reserved for issuance under the 2008 plan that remain available for future issuance immediately prior to the closing of this offering. Following the closing of this offering, the number of shares reserved for issuance under the 2015 plan will increase by (1) the number of shares of our common stock subject to outstanding awards under our 2008 plan upon the closing of this offering that expire, terminate or are otherwise surrendered, cancelled, forfeited or repurchased by us at their original issuance price pursuant to a contractual repurchase right and (2) 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 our common stock, 4% of the number of shares of our common stock outstanding on the first day of the fiscal year and an amount determined by our board of directors. Our employees, officers, directors, consultants and advisors will be eligible to receive awards under the 2015 plan; however, incentive stock options may only be granted to our employees.

Pursuant to the terms of the 2015 plan, our board of directors (or a committee delegated by our board of directors) administers the 2015 plan and, subject to any limitations set forth in the 2015 plan, will select the recipients of awards and determine:

- the number of shares of common stock covered by options and the dates upon which those options become exercisable;
- the type of options to be granted;
- the exercise price of options, which price must be at least equal to the fair market value of our common stock on the date of grant;
- the duration of options, which may not be in excess of ten years;
- the methods of payment of the exercise price of options; and
- the number of shares of our common stock subject to and the terms of any stock appreciation rights, awards of restricted stock, restricted stock units, other stock-based awards and the terms and conditions of such awards, including the issue price, conditions for repurchase, repurchase price and performance conditions (though the measurement price of stock appreciation rights must be at least equal to the fair market value of our common stock on the date of grant and the duration of such awards may not be in excess of ten years), if any.

If our board of directors delegates authority to an executive officer to grant awards under the 2015 plan, the executive officer will have the power to make awards to all of our employees, except executive officers. Our board of directors will fix the terms of the awards to be granted by such executive officer, including the exercise price of such awards (or a formula for establishing such price), and the maximum number of shares subject to awards that such executive officer may make.

In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of our common stock other than an ordinary cash dividend, we are required by the 2015 plan to make equitable adjustments (or make substitute awards, if applicable), in a manner determined by our board, to:

- the number and class of securities available under the 2015 plan;
- the share counting rules under the 2015 plan;
- the number and class of securities and exercise price per share of each outstanding option;

- the share and per-share provisions and measurement price of each outstanding stock appreciation right;
- the number of shares and the repurchase price per share subject to each outstanding restricted stock award or restricted stock unit award; and
- the share and per-share related provisions and purchase price, if any, of any outstanding other stock-based award.

Upon a merger or other reorganization event (as defined in our 2015 plan), our board of directors, may, on such terms as our board determines (except to the extent specifically provided otherwise in an applicable award agreement or other agreement between the participant and us), take any one or more of the following actions pursuant to the 2015 plan, as to some or all outstanding awards, other than restricted stock awards:

- provide that all outstanding awards will be assumed or substantially equivalent awards will be substituted by the successor corporation (or an affiliate thereof);
- upon written notice to a participant, provide that the participant's unvested and/or unexercised options or other awards will terminate immediately prior to the consummation of such transaction unless exercised by the participant;
- provide that outstanding awards will become exercisable, realizable or deliverable, or restrictions applicable to an award will lapse, in whole or in part, prior to or upon the reorganization event;
- in the event of a reorganization event pursuant to which holders of our common stock will receive a cash payment for each share surrendered in the reorganization event, make or provide for a cash payment to the participants with respect to each award held by a participant equal to (1) the number of shares of our common stock subject to the vested portion of the award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such reorganization event) multiplied by (2) the excess, if any, of the cash payment for each share surrendered in the reorganization event over the exercise, measurement or purchase price of such award and any applicable tax withholdings, in exchange for the termination of such award;
- provide that, in connection with a liquidation or dissolution, awards convert into the right to receive liquidation proceeds (if applicable, net of exercise, measurement or purchase price thereof and any applicable tax withholdings); or
- any combination of the foregoing.

Our board of directors is not obligated by the 2015 plan to treat all awards, all awards held by a participant, or all awards of the same type, identically.

In the case of certain restricted stock units, no assumption or substitution is permitted, and the restricted stock units will instead be settled in accordance with the terms of the applicable restricted stock unit agreement.

Upon the occurrence of a reorganization event other than a liquidation or dissolution, the repurchase and other rights under each outstanding restricted stock award will continue for the benefit of the successor company and will, unless our board of directors may otherwise determine, apply to the cash, securities or other property which our common stock is converted into or exchanged for pursuant to the reorganization event, unless our board provided for the termination or deemed satisfaction of such repurchase or other rights under the restricted stock award agreement or any other agreement between the participant and us. Upon the occurrence of a reorganization event involving a liquidation or dissolution, all restrictions and conditions on each outstanding restricted stock award will automatically be deemed terminated or satisfied, unless otherwise provided in the agreement evidencing the restricted stock award or in any other agreement between the participant and us.

Our board of directors may at any time provide that any award under the 2015 plan shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

Except with respect to certain actions requiring stockholder approval under the Internal Revenue Code or the rules of the NASDAQ Stock Market, our board of directors may amend, modify or terminate any outstanding award under the 2015 plan, including but not limited to, substituting therefor another award of the same or a different type, changing the date of exercise or realization, and converting an incentive stock option into a nonstatutory stock option, subject to certain participant consent requirements. Unless our stockholders approve such action, the 2015 plan provides that we may not (except as otherwise permitted in connection with a change in capitalization or reorganization event):

- amend any outstanding stock option or stock appreciation right granted under the 2015 plan to provide an exercise or measurement price per share that is lower than the then-current exercise or measurement price per share of such outstanding award;
- cancel any outstanding option or stock appreciation right (whether or not granted under the 2015 plan) and grant in substitution therefor new awards under the 2015 plan (other than substitute awards permitted in connection with a merger or consolidation of an entity with us or our acquisition of property or stock of another entity) covering the same or a different number of shares of our common stock and having an exercise or measurement price per share lower than the then-current exercise or measurement price per share of the cancelled award;
- cancel in exchange for a cash payment any outstanding option or stock appreciation right with an exercise or measurement price per share above the then-current fair market value of our common stock; or
- take any other action that constitutes a "repricing" within the meaning of the rules of the NASDAQ Stock Market.

No award may be granted under the 2015 plan after 10 years from the effective date of this offering. Our board of directors may amend, suspend or terminate the 2015 plan at any time, except that stockholder approval will be required to comply with applicable law or stock market requirements.

2015 Employee Stock Purchase Plan

In June 2015, our board of directors adopted, and our stockholders have approved, the 2015 ESPP, to become effective upon the closing of this offering. The 2015 ESPP will be administered by our board of directors or by a committee appointed by our board of directors. The 2015 ESPP initially provides participating employees with the opportunity to purchase up to an aggregate of 182,352 shares of our common stock. The number of shares of our common stock reserved for issuance under the 2015 ESPP will automatically increase on the first day of each fiscal year, commencing on January 1, 2016 and ending on December 31, 2026, in an amount equal to the least of (i) 364,705 shares of our common stock, (ii) 1% of the total number of shares of our common stock outstanding on the first day of the applicable year, and (iii) an amount determined by our board of directors.

All of our employees or employees of any designated subsidiary, as defined in the 2015 ESPP, are eligible to participate in the 2015 ESPP, provided that:

- such person is customarily employed by us or a designated subsidiary for more than 20 hours a week and for more than five months in a calendar year;
- such person has been employed by us or by a designated subsidiary for at least six months prior to enrolling in the 2015 ESPP; and

- such person was our employee or an employee of a designated subsidiary on the first day of the applicable offering period under the 2015 ESPP.

No employee may purchase shares of our common stock under the 2015 ESPP and any of our other employee stock purchase plans in excess of $25,000 of the fair market value of our common stock (as of the date of the option grant) in any calendar year. In addition, no employee may purchase shares of our common stock under the 2015 ESPP that would result in the employee owning 5% or more of the total combined voting power or value of our stock or the stock of any of our subsidiaries.

We expect to make one or more offerings to our eligible employees to purchase stock under the 2015 ESPP beginning at such time as our board of directors may determine. Each offering will consist of a six-month offering period during which payroll deductions will be made and held for the purchase of our common stock at the end of the offering period. Our board of directors may, at its discretion, choose a different period of not more than 12 months for offerings.

On the commencement date of each offering period, each eligible employee may authorize up to a maximum of 15% of his or her compensation to be deducted by us during the offering period. Each employee who continues to be a participant in the 2015 ESPP on the last business day of the offering period will be deemed to have exercised an option to purchase from us the number of whole shares of our common stock that his or her accumulated payroll deductions on such date will pay for, not in excess of the maximum numbers set forth above. Under the terms of the 2015 ESPP, the purchase price shall be determined by our board of directors for each offering period and will be at least 85% of the applicable closing price of our common stock. If our board of directors does not make a determination of the purchase price, the purchase price will be 85% of the lesser of the closing price of our common stock on the first business day of the offering period or on the last business day of the offering period.

An employee who is not a participant on the last day of the offering period is not entitled to purchase shares under the 2015 ESPP, and the employee's accumulated payroll deductions will be refunded. An employee's rights under the 2015 ESPP terminate upon voluntary withdrawal from an offering under the 2015 ESPP at any time, or when the employee ceases employment for any reason.

We will be required to make equitable adjustments to the number and class of securities available under the 2015 ESPP, the share limitations under the 2015 ESPP, and the purchase price for an offering period under the 2015 ESPP to reflect stock splits, reverse stock splits, stock dividends, recapitalizations, combinations of shares, reclassifications of shares, spin-offs and other similar changes in capitalization or events or any dividends or distributions to holders of our common stock other than ordinary cash dividends.

In connection with a merger or other reorganization event, as defined in the 2015 ESPP, our board of directors or a committee of our board of directors may take any one or more of the following actions as to outstanding options to purchase shares of our common stock under the 2015 ESPP on such terms as our board or committee determines:

- provide that options shall be assumed, or substantially equivalent options shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof);

- upon written notice to employees, provide that all outstanding options will be terminated immediately prior to the consummation of such reorganization event and that all such outstanding options will become exercisable to the extent of accumulated payroll deductions as of a date specified by our board or committee in such notice, which date shall not be less than ten days preceding the effective date of the reorganization event;

- upon written notice to employees, provide that all outstanding options will be cancelled as of a date prior to the effective date of the reorganization event and that all accumulated payroll deductions will be returned to participating employees on such date;

- in the event of a reorganization event under the terms of which holders of our common stock will receive upon consummation thereof a cash payment for each share surrendered in the reorganization event, change the last day of the offering period to be the date of the consummation of the reorganization event and make or provide for a cash payment to each employee equal to (1) the cash payment for each share surrendered in the reorganization event times the number of shares of our common stock that the employee's accumulated payroll deductions as of immediately prior to the reorganization event could purchase at the applicable purchase price, where the acquisition price is treated as the fair market value of our common stock on the last day of the applicable offering period for purposes of determining the purchase price and where the number of shares that could be purchased is subject to the applicable limitations under the 2015 ESPP minus (2) the result of multiplying such number of shares by the purchase price; and/or

- provide that, in connection with our liquidation or dissolution, options shall convert into the right to receive liquidation proceeds (net of the purchase price thereof).

Our board of directors may at any time, and from time to time, amend or suspend the 2015 ESPP or any portion thereof. We will obtain stockholder approval for any amendment if such approval is required by Section 423 of the Internal Revenue Code. Further, our board of directors may not make any amendment that would cause the 2015 ESPP to fail to comply with Section 423 of the Internal Revenue Code. The 2015 ESPP may be terminated at any time by our board of directors. Upon termination, we will refund all amounts in the accounts of participating employees.

401(k) Retirement Plan

We maintain a 401(k) retirement plan that is intended to be a tax-qualified defined contribution plan under Section 401(k) of the Code. In general, all of our employees are eligible to participate, beginning on the first day of the month following commencement of their employment. The 401(k) plan includes a salary deferral arrangement pursuant to which participants may elect to reduce their current compensation by up to the statutorily prescribed limit, equal to $18,000 in 2015, and have the amount of the reduction contributed to the 401(k) plan.

Limitations on Liability and Indemnification

Our certificate of incorporation, which will become effective upon the closing of this offering, limits the personal liability of directors for breach of fiduciary duty to the maximum extent permitted by the General Corporation Law of the State of Delaware and provides that no director will have personal liability to us or to our stockholders for monetary damages for breach of fiduciary duty or other duty as a director. However, these provisions do not eliminate or limit the liability of any of our directors:

- for any breach of the director's duty of loyalty to us or our stockholders;

- for acts or omissions not in good faith or that involve intentional misconduct or a knowing violation of law;

- for voting for or assenting to unlawful payments of dividends, stock repurchases or other distributions; or

- for any transaction from which the director derived an improper personal benefit.

Any amendment to or repeal of these provisions will not eliminate or reduce the effect of these provisions in respect of any act, omission or claim that occurred or arose prior to such amendment or repeal. If the General Corporation Law of the State of Delaware is amended to provide for further limitations on the personal liability of directors of corporations, then the personal liability of our

directors will be further limited to the greatest extent permitted by the General Corporation Law of the State of Delaware.

In addition, our certificate of incorporation, which will become effective upon the closing of this offering, provides that we must indemnify our directors and officers and we must advance expenses, including attorneys' fees, to our directors and officers in connection with legal proceedings, subject to very limited exceptions.

We maintain a general liability insurance policy that covers specified liabilities of our directors and officers arising out of claims based on acts or omissions in their capacities as directors or officers. In addition, we have entered into indemnification agreements with certain of our directors, and we intend to enter into indemnification agreements with all of our directors and executive officers prior to the completion of this offering. These indemnification agreements may require us, among other things, to indemnify each such director or officer for some expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by him in any action or proceeding arising out of his or her service as one of our directors or officers.

Some of our non-employee directors may, through their relationships with their employers, be insured or indemnified against specified liabilities incurred in their capacities as members of our board of directors.

Insofar as indemnification for liabilities arising under the Securities Act of 1933, or the Securities Act, may be permitted to directors, executive officers or persons controlling us, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

Rule 10b5-1 Sales Plans

Our directors and executive officers may adopt written plans, known as Rule 10b5-1 plans, in which they will contract with a broker to buy or sell shares of our common stock on a periodic basis. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the director or officer when entering into the plan, without further direction from the director or officer. The director or officer may amend or terminate the plan in some circumstances. Our directors and executive officers may also buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

Director Compensation

During 2014, we paid Mr. Bate an annual retainer of $30,000 for service on our board of directors. In addition, on March 19, 2014, we granted Mr. Bate an option to purchase 9,883 shares of our common stock, at an exercise price of $6.81 per share, which vests over four years, with 25% of the shares having vested on January 1, 2015 and the remainder vesting in equal monthly installments thereafter. This stock option had a grant date fair value of $58,848, computed in accordance with ASC Topic 718, which combined with Mr. Bate's annual retainer, resulted in total compensation of $88,848 for Mr. Bate for 2014. See Note 12 to our financial statements appearing at the end of this prospectus regarding assumptions underlying the valuation of equity awards. None of our other non-employee directors has received any compensation from us, although we reimburse our non-employee directors for reasonable travel and out-of-pocket expenses incurred in connection with attending board of director and committee meetings. With the exception of the stock option granted to Mr. Bate described above, there were no outstanding equity awards held by our non-employee directors as of December 31, 2014.

We do not pay any compensation to our President and Chief Executive Officer in connection with her service on our board of directors. The compensation that we pay to our President and Chief Executive Officer is discussed earlier in this "Executive Compensation" section.

In March 2015, our board of directors approved a director compensation program to be effective at the time of this offering.

Under this director compensation program, we will pay our non-employee directors a cash retainer for service on the board of directors and for service on each committee on which the director is a member. The chairman of each committee will receive higher retainers for such service. These fees are payable in arrears in four equal quarterly installments on the last day of each quarter, provided that the amount of such payment will be prorated for any portion of such quarter that the director is not serving on our board of directors and no fee shall be payable in respect of any period prior to the closing of this offering. The fees paid to non-employee directors for service on the board of directors and for service on each committee of the board of directors on which the director is a member are as follows:

	Member Annual Fee	Chairman Additional Annual Fee
Board of Directors	$ 35,000	$ 25,000
Audit Committee	7,500	15,000
Compensation Committee	5,000	10,000
Nominating and Corporate Governance Committee	3,500	7,000

We also will continue to reimburse our non-employee directors for reasonable travel and out-of-pocket expenses incurred in connection with attending our board of director and committee meetings.

In addition, under our director compensation program to be effective at the time of this offering, each non-employee director serving on our board of directors upon the closing of this offering and each non-employee director elected to our board of directors after the closing of this offering will receive an option to purchase 11,094 shares of our common stock. Each of these options will vest as to one-third of the shares of our common stock underlying such option on each anniversary of the grant date until the third anniversary of the grant date, subject to the non-employee director's continued service as a director. All options issued to our non-employee directors under our director compensation program will become exercisable in full upon a change in control of our company. Further, on the date of the first board meeting held after each annual meeting of stockholders, each non-employee director that has served on our board of directors for at least six months will receive an option to purchase 4,437 shares of our common stock. Each of these options will vest in full on the one-year anniversary of the grant date unless otherwise provided at the time of grant, subject to the non-employee director's continued service as a director, and will become exercisable in full upon a change in control of our company. The exercise price of these options will equal the fair market value of our common stock on the date of grant.

TRANSACTIONS WITH RELATED PERSONS

Since January 1, 2012, we have engaged in the following transactions in which the amount involved exceeded $120,000 and any of our directors or executive officers or beneficial holders of more than 5% of any class of our voting securities, or any immediate family member of the foregoing persons, had a material interest. We believe that all of these transactions were on terms comparable to terms that could have been obtained from unrelated third parties.

Series A Preferred Stock Financing

In closings that occurred in July 2012, January 2013 and June 2013, we issued and sold an aggregate of 25,636,951 shares of our series A preferred stock at a price per share of $0.70, for an aggregate purchase price of $17.9 million. The following table sets forth the number of shares of our series A preferred stock purchased by our directors, executive officers and 5% stockholders and their respective affiliates and the aggregate purchase price paid for such shares.

Name	Shares of Series A Preferred Stock Purchased	Aggregate Purchase Price
SV Life Sciences Fund V, L.P.	8,160,866	$ 5,712,606
SV Life Sciences Fund V Strategic Partners, L.P.	172,467	120,727
Clarus Lifesciences II, L.P.	8,000,000	5,600,000
MedImmune Ventures, Inc.	5,333,333	3,733,333
Advanced Technology Ventures VIII, L.P.	3,333,333	2,333,333
George Milne(1)	302,115	211,481
Jill C. Milne	35,714	25,000
Michael Jirousek	35,714	25,000
Total	25,373,542	$ 17,761,480

(1) George Milne is the father-in-law of Jill C. Milne, our President and Chief Executive Officer.

Series B Preferred Stock Financing

In October 2013, we issued and sold an aggregate of 34,129,571 shares of our series B preferred stock at a price per share of $0.9503, for an aggregate purchase price of $32.4 million. The following table sets forth the number of shares of our series B preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

Name	Shares of Series B Preferred Stock Purchased	Aggregate Purchase Price
SV Life Sciences Fund V, L.P.	7,961,792	$ 7,566,091
SV Life Sciences Fund V Strategic Partners, L.P.	168,259	159,897
Clarus Lifesciences II, L.P.	7,899,973	7,507,344
MedImmune Ventures, Inc.	3,156,898	3,000,000
Advanced Technology Ventures VIII, L.P.	3,156,897	2,999,999
Lightstone Ventures, L.P.	6,539,602	6,214,584
Lightstone Ventures (A), L.P.	1,352,643	1,285,417
Total	30,236,064	$ 28,733,332

In March 2015, we issued and sold an aggregate of 13,062,965 additional shares of our series B preferred stock at a price per share of $0.9503, for an aggregate purchase price of $12.4 million. The following table sets forth the number of shares of our series B preferred stock purchased by our directors, executive officers and 5% stockholders and their affiliates and the aggregate purchase price paid for such shares.

Name	Shares of Series B Preferred Stock Purchased	Aggregate Purchase Price
SV Life Sciences Fund V, L.P.	1,859,593	$ 1,767,171
SV Life Sciences Fund V Strategic Partners, L.P.	39,299	37,346
Clarus Lifesciences II, L.P.	1,828,998	1,738,097
MedImmune Ventures, Inc.	1,084,882	1,030,963
Advanced Technology Ventures VIII, L.P.	753,495	716,046
Lightstone Ventures, L.P.	442,646	420,647
Lightstone Ventures (A), L.P.	60,312	57,315
Jill C. Milne	105,229	99,999
George Milne	333,105	316,550
Rick Modi	52,614	49,999
Joanne M. Donovan	5,261	5,000
Michael Jirousek	5,261	5,000
Total	6,570,695	$ 6,244,133

Investor Rights Agreement

We are a party to an amended and restated investor rights agreement, dated as of March 17, 2015, with holders of our preferred stock, including some of our directors and 5% stockholders and their affiliates and entities affiliated with our officers and directors. The amended and restated investor rights agreement provides these holders the right, following the completion of this offering, to demand that we file a registration statement or request that their shares be covered by a registration statement that we are otherwise filing. In addition, the holders of warrants to purchase shares of our preferred stock have rights under those warrants to become party to the amended and restated investor rights agreement following exercise of the warrants, following which they will have, with respect to the shares acquired on exercise of the warrants, the same rights to require us to register the shares as the other investor parties to the amended and restated investor rights agreement. See "Description of Capital Stock—Registration Rights" for additional information regarding these registration rights.

Employment Agreements

See the "Executive Compensation—Agreements with Our Named Executive Officers" section of this prospectus for a further discussion of these arrangements.

Indemnification Agreements

Our certificate of incorporation that will become effective upon the closing of this offering provides that we will indemnify our directors and officers to the fullest extent permitted by Delaware law. In addition, we have entered into indemnification agreements with each of our directors. See "Executive Compensation—Limitation of Liability and Indemnification" for additional information regarding these agreements.

Policies and Procedures for Related Person Transactions

Our board of directors has adopted written policies and procedures, which will become effective at the time of this offering, for the review of any transaction, arrangement or relationship in which our company is a participant, the amount involved exceeds $120,000 and one of our executive officers, directors, director nominees or 5% stockholders, or their immediate family members, each of whom we refer to as a "related person," has a direct or indirect material interest.

If a related person proposes to enter into such a transaction, arrangement or relationship, which we refer to as a "related person transaction," the related person must report the proposed related person transaction to our Chief Financial Officer. The policy calls for the proposed related person transaction to be reviewed and approved by our audit committee. Whenever practicable, the reporting, review and approval will occur prior to entry into the transaction. If advance review and approval is not practicable, the committee will review, and, in its discretion, may ratify the related person transaction. The policy also permits the chairman of the audit committee to review and, if deemed appropriate, approve proposed related person transactions that arise between committee meetings, subject to ratification by the committee at its next meeting. Any related person transactions that are ongoing in nature will be reviewed annually.

A related person transaction reviewed under the policy will be considered approved or ratified if it is authorized by the audit committee after full disclosure of the related person's interest in the transaction. As appropriate for the circumstances, the committee will review and consider:

- the related person's interest in the related person transaction;
- the approximate dollar value of the amount involved in the related person transaction;
- the approximate dollar value of the amount of the related person's interest in the transaction without regard to the amount of any profit or loss;
- whether the transaction was undertaken in the ordinary course of our business;
- whether the terms of the transaction are no less favorable to us than terms that could have been reached with an unrelated third party;
- the purpose of, and the potential benefits to us of, the transaction; and
- any other information regarding the related person transaction or the related person in the context of the proposed transaction that would be material to investors in light of the circumstances of the particular transaction.

The audit committee may approve or ratify the transaction only if the committee determines that, under all of the circumstances, the transaction is in our best interests. The committee may impose any conditions on the related person transaction that it deems appropriate.

The policy provides that transactions involving compensation of executive officers shall be reviewed and approved by the compensation committee in the manner specified in its charter.

PRINCIPAL STOCKHOLDERS

The following table sets forth information with respect to the beneficial ownership of our common stock as of April 30, 2015 by:

- each of our directors;
- each of our named executive officers;
- all of our directors and executive officers as a group; and
- each person, or group of affiliated persons, who is known by us to beneficially own more than 5% of our common stock.

The column entitled "Percentage of Shares Beneficially Owned—Before Offering" is based on a total of 9,547,796 shares of our common stock outstanding as of April 30, 2015, assuming the automatic conversion of all outstanding shares of our preferred stock into an aggregate of 9,029,551 shares of our common stock upon the closing of this offering. The column entitled "Percentage of Shares Beneficially Owned—After Offering" is based on 14,547,796 shares of our common stock to be outstanding after this offering, including the 5,000,000 shares of our common stock that we are selling in this offering, but not including any additional shares issuable upon exercise of outstanding options or warrants or any exercise by the underwriters of their over-allotment option.

Beneficial ownership is determined in accordance with the rules and regulations of the SEC and includes voting or investment power with respect to our common stock. Shares of our common stock subject to options and warrants that are currently exercisable or exercisable within 60 days after April 30, 2015 are considered outstanding and beneficially owned by the person holding the options or warrants for the purpose of calculating the percentage ownership of that person but not for the purpose of calculating the percentage ownership of any other person. Except as otherwise noted, the persons and entities in this table have sole voting and investing power with respect to all of the shares of our common stock beneficially owned by them, subject to community property laws, where applicable. Except as otherwise set forth below, the address of the beneficial owner is c/o Catabasis Pharmaceuticals, Inc., One Kendall Square, Bldg. 1400E, Suite B14202, Cambridge, MA 02139.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to $15.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these existing stockholders and any of these existing stockholders could determine to purchase more, less or no shares in this offering. The following table does not reflect any potential purchases by these existing principal stockholders. If any shares are purchased by these stockholders, the number and percentage of shares of our common

stock beneficially owned by them after this offering will differ from those set forth in the following table.

Name of Beneficial Owner	Shares Beneficially Owned	Percentage of Shares Beneficially Owned	
		Before Offering	After Offering
5% Stockholders			
Entities affiliated with SV Life Sciences(1)	2,466,577	25.8%	17.8%
Clarus Lifesciences II, L.P.(2)	2,375,793	24.9	17.2
MedImmune Ventures, Inc.(3)	1,409,213	14.8	10.2
Advanced Technology Ventures VIII, L.P.(4)	978,755	10.3	7.1
Entities affiliated with Lightstone Ventures(5)	653,321	6.8	4.7
Named Executive Officers and Directors			
Jill C. Milne, Ph.D.(6)	428,041	4.4	3.1
Ian C. Sanderson(7)	42,078	*	*
Michael Jirousek, Ph.D.(8)	410,616	4.2	2.9
Michael Ross, Ph.D.(9)	2,466,577	25.8	17.8
Nicholas Galakatos, Ph.D.(10)	2,375,793	24.9	17.2
Jean George(11)	1,632,076	17.1	11.8
Ron Laufer, M.D.(12)	1,409,213	14.8	10.2
Kenneth Bate(13)	3,500	*	*
All Executive Officers and Directors as a Group (10 persons)(14)	8,845,475	88.4	61.8

* Represents beneficial ownership of less than 1% of our outstanding stock.

(1) Consists of 2,415,532 shares of record held by SV Life Sciences Fund V, L.P. ("SVLS V LP") and 51,045 shares of record held by SV Life Sciences Fund V Strategic Partners, L.P. ("SVLS V SPP"). SV Life Sciences Fund V (GP), LP ("SVLS V GP") is the general partner of SVLS V LP and SVLS V SPP. The general partner of SVLS V GP is SVLSF V, LLC. The members of the investment committee of SVLSF V, LLC are Kate Bingham, James Garvey, Eugene D. Hill, III, David Milne and Michael Ross, a member of our board of directors. SVLS V GP, SVLSF V, LLC and each of the individuals comprising the SVLSF V, LLC investment committee may be deemed to share voting, dispositive and investment power over the shares held of record by SVLS V LP and SVLS V SPP. Each of SVLS V GP, SVLSF V, LLC and the individual members of the SVLSF V, LLC investment committee disclaim beneficial ownership of the shares owned directly by SVLS V LP and SVLS V SPP except to the extent of any pecuniary interest therein. The address for the entities is One Boston Place, Suite 3900, 201 Washington Street, Boston, Massachusetts 02108.

(2) Consists of 2,375,793 shares held of record by Clarus Lifesciences II, L.P. ("Clarus"). Clarus Ventures II GP, L.P. (the "GPLP"), as the sole general partner of Clarus, may be deemed to beneficially own certain of the shares held of record by Clarus. The GPLP disclaims beneficial ownership of all shares held of record by Clarus in which the GPLP does not have an actual pecuniary interest. Clarus Ventures II, LLC (the "GPLLC"), as the sole general partner of the GPLP, may be deemed to beneficially own certain of the shares held of record by Clarus. The GPLLC disclaims beneficial ownership of all shares held of record by Clarus in which it does not have an actual pecuniary interest. Each of Nicholas Galakatos, a member of our board of directors, and Denis Henner, Robert Liptak, Nicholas Simon, Michael Steinmetz and Kurt Wheeler, as individual Managing Directors of the GPLLC, may be deemed to beneficially own certain of the shares held of record by Clarus. Each of Messrs. Galakatos, Henner, Liptak, Simon,

Steinmetz and Wheeler disclaims beneficial ownership of all shares held of record by Clarus in which he does not have an actual pecuniary interest. The address for the entities is 101 Main Street, Suite 1210, Cambridge, MA 02142.

(3) Ron Laufer, a member of our board of directors, is the Senior Managing Partner of MedImmune Ventures, Inc., and as a result, Dr. Laufer may be deemed to hold voting, dispositive and investment power over the shares held by MedImmune Ventures, Inc. Dr. Laufer disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. The address of MedImmune Ventures, Inc. is 1 MedImmune Way, Gaithersburg, Maryland 20878.

(4) ATV Associates VIII, LLC ("ATV A VIII") is the General Partner of Advanced Technology Ventures VIII, L.P. ("ATV VIII") and exercises voting and dispositive authority over the shares held by ATV VIII. Voting and dispositive decisions of ATV A VIII are made collectively by Michael A. Carusi, Jean George, a member of our board of directors, Steven N. Baloff, Robert C. Hower and William C. Wiberg (collectively, the "ATV VIII Managing Directors"). ATV A VIII and the ATV VIII Managing Directors disclaim beneficial ownership of the shares held by ATV VIII except to the extent of their pecuniary interest therein. The address for the entities is 500 Boylston Street, Suite 1380, Boston, MA 02116.

(5) Consists of 574,979 shares of common stock held by Lightstone Ventures, L.P. and 78,342 shares of common stock held by Lightstone Ventures (A), L.P. LSV Associates, LLC ("LSV GP") is the General Partner of Lightstone Ventures, L.P. and Lightstone Ventures (A), L.P. (collectively, "LSV") and exercises voting and dispositive authority over the shares held by LSV. Voting and dispositve decisions of LSV GP are made collectively by Michael A. Carusi, Jean George, a member of our board of directors, Ralph E. Christoffersen and Henry A. Plain, Jr. (collectively, the "LSV Managing Directors"). LSV GP and the LSV Managing Directors disclaim beneficial ownership of the shares held by LSV except to the extent of their pecuniary interest therein. The address for the entities is 500 Boylston Street, Suite 1380, Boston, Massachusetts 02116.

(6) Includes (i) 165,416 shares of common stock issuable upon the exercise of options and (ii) 8,753 shares of common stock issuable upon the exercise of warrants, in each case exercisable within 60 days after April 30, 2015.

(7) Consists of 42,078 shares of common stock issuable upon the exercise of options exercisable within 60 days after April 30, 2015.

(8) Includes (i) 155,770 shares of common stock issuable upon the exercise of options and (ii) 8,753 shares of common stock issuable upon the exercise of warrants, in each case exercisable within 60 days after April 30, 2015.

(9) Consists of the shares described in note (1) above. Dr. Ross is a member of the investment committee of SVLSF V, LLC, which is the general partner of SV Life Sciences Fund V (GP), which is the general partner of SV Life Sciences Fund V, L.P. and SV Life Sciences Fund V Strategic Partners, L.P., and, as a result, Dr. Ross may be deemed to share voting, dispositive and investment power over the shares held of record by SV Life Sciences Fund V, L.P. and SV Life Sciences Fund V Strategic Partners L.P. Dr. Ross disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Dr. Ross' address is One Boston Place, Suite 3900, Boston, Massachusetts 02108.

(10) Consists of the shares described in note (2) above. Dr. Galakatos is a Managing Director of Clarus Ventures II, LLC, which the sole general partner of Clarus Ventures II GP, L.P., which is the sole general partner of Clarus Ventures II, L.P., and, as a result, Dr. Galakatos shares investment and voting control over the shares held by Clarus Lifesciences II, L.P. Dr. Galakatos disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Dr. Galakatos' address is 101 Main Street, Suite 1210, Cambridge, MA 02142.

(11) Consists of the shares described in notes (4) and (5) above. Ms. George is an ATV III Managing Director and, as a result, shares voting and dispositive power over the shares held by Advanced Technology Ventures VIII, L.P. Ms. George is also an LSV Managing Director and, as a result, shares voting and dispositive power over the shares held by LSV. Ms. George disclaims beneficial ownership of the shares held by ATV VIII and LSV except to the extent of any pecuniary interest therein. Ms. George's address is 500 Boylston Street, Suite 1380, Boston, MA 02116.

(12) Consists of the shares described in note (3) above. Dr. Laufer is the Senior Managing Partner of MedImmune Ventures, Inc., and as a result, Dr. Laufer may be deemed to hold voting, dispositive and investment power over the shares held by MedImmune Ventures, Inc. Dr. Laufer disclaims beneficial ownership of such shares except to the extent of any pecuniary interest therein. Dr. Laufer's address is c/o MedImmune Ventures, 1 MedImmune Way, Gaithersburg, Maryland 20878.

(13) Consists of 3,500 shares of common stock issuable upon the exercise of options exercisable within 60 days after April 30, 2015.

(14) Includes (i) 439,842 shares of common stock issuable upon the exercise of options and (ii) 17,506 shares of common stock issuable upon the exercise of warrants, in each case exercisable within 60 days after April 30, 2015.

DESCRIPTION OF CAPITAL STOCK

General

Following the closing of this offering, our authorized capital stock will consist of 150,000,000 shares of common stock, par value $0.001 per share, and 5,000,000 shares of preferred stock, par value $0.001 per share, all of which preferred stock will be undesignated. The following description of our capital stock and provisions of our restated certificate of incorporation and amended and restated bylaws are summaries and are qualified by reference to the certificate of incorporation and the bylaws that will be in effect upon the closing of this offering. We have filed copies of these documents as exhibits to our registration statement of which this prospectus forms a part. The descriptions of the common stock and preferred stock reflect changes to our capital structure that will occur upon the closing of this offering.

Common Stock

As of April 30, 2015, we had outstanding 9,547,796 shares of common stock, assuming the automatic conversion of all outstanding shares of our preferred stock into common stock upon the closing of this offering, which were held of record by 27 stockholders.

Holders of our common stock are entitled to one vote for each share held on all matters submitted to a vote of stockholders and do not have cumulative voting rights. An election of directors by our stockholders will be determined by a plurality of the votes cast by the stockholders entitled to vote on the election. Other matters shall be decided by the affirmative vote of our stockholders having a majority in voting power of the votes cast by the stockholders present or represented and voting on such matter, except as otherwise disclosed below. Holders of common stock are entitled to receive proportionately any dividends as may be declared by our board of directors, subject to any preferential dividend rights of outstanding preferred stock.

In the event of our liquidation or dissolution, the holders of common stock are entitled to receive proportionately all assets available for distribution to stockholders after the payment of all debts and other liabilities and subject to the prior rights of any outstanding preferred stock. Holders of common stock have no preemptive, subscription, redemption or conversion rights. The rights, preferences and privileges of holders of common stock are subject to and may be adversely affected by the rights of the holders of shares of any series of preferred stock that we may designate and issue in the future.

Preferred Stock

Under the terms of our certificate of incorporation that will become effective upon the closing of this offering, our board of directors is authorized to issue shares of preferred stock in one or more series without stockholder approval. Our board of directors has the discretion to determine the rights, preferences, privileges and restrictions, including voting rights, dividend rights, conversion rights, redemption privileges and liquidation preferences, of each series of preferred stock.

The purpose of authorizing our board of directors to issue preferred stock and determine its rights and preferences is to eliminate delays associated with a stockholder vote on specific issuances. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions, future financings and other corporate purposes, could have the effect of making it more difficult for a third party to acquire, or could discourage a third party from seeking to acquire, a majority of our outstanding voting stock. Upon the closing of this offering, there will be no shares of preferred stock outstanding, and we have no present plans to issue any shares of preferred stock.

Stock Options

As of April 30, 2015, options to purchase 1,478,731 shares of our common stock at a weighted-average exercise price of $5.46 per share were outstanding, of which options to purchase 657,292 shares of our common stock were exercisable, at a weighted-average exercise price of $2.98 per share.

Warrants

As of April 30, 2015, we had outstanding warrants to purchase shares of our series B preferred stock that upon the closing of this offering will be exercisable for an aggregate of 24,566 shares of our common stock at an exercise price of $12.2114 per share.

As of April 30, 2015, we had outstanding warrants to purchase shares of our common stock that upon the closing of this offering will be exercisable for an aggregate of 34,839 shares of our common stock at an exercise price of $1.67 per share.

Registration Rights

Our amended and restated investor rights agreement, or the Investor Rights Agreement, provides certain holders of our preferred stock, including some of our directors and 5% stockholders and their respective affiliates and entities affiliated with our officers and directors, the right, following the completion of this offering, to require us to register these shares under the Securities Act of 1933, as amended, or the Securities Act, under specified circumstances as described below. In addition, the holders of warrants to purchase shares of our preferred stock have rights under those warrants to become party to the Investor Rights Agreement following exercise of the warrants, following which they will have, with respect to the shares acquired on exercise of the warrants, the same rights to require us to register the shares as the other investor parties to the Investor Rights Agreement. After registration pursuant to these rights, these shares will become freely tradable without restriction under the Securities Act.

Demand Registration Rights

Beginning six months after the closing of this offering, subject to specified limitations set forth in the Investor Rights Agreement, at any time the holders of a majority of then outstanding registrable securities, as defined in the Investor Rights Agreement, acting together, may demand in writing that we register their registrable securities under the Securities Act so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public, net of selling expenses, of least $10.0 million. We are not obligated to file a registration statement pursuant to this demand provision on more than two occasions, subject to specified exceptions.

In addition, at any time after we become eligible to file a registration statement on Form S-3 under the Securities Act, subject to specified limitations, the holders of at least 35% of the registrable securities then outstanding may demand in writing that we register on Form S-3 registrable shares held by them so long as the total amount of registrable shares requested to be registered has an anticipated aggregate offering price to the public, net of selling expenses, of least $1.0 million.

Incidental Registration Rights

If, at any time after the closing of this offering, we propose to file a registration statement to register any of our securities under the Securities Act, either for our own account or for the account of any of our stockholders that are not holders of registrable shares, solely for cash and on a form that would also permit the registration of registrable shares, the holders of our registrable shares are entitled to notice of registration and, subject to specified exceptions, we will be required to register the registrable shares then held by them that they request that we register.

Expenses

Pursuant to the Investor Rights Agreement, we are required to pay all registration expenses, including registration fees, printing expenses, fees and disbursements of our counsel and accountants and reasonable fees and disbursements of one counsel representing the selling stockholders, other than any underwriting discounts and commissions, related to any demand or incidental registration. The Investor Rights Agreement contains customary cross-indemnification provisions, pursuant to which we are obligated to indemnify the selling stockholders in the event of material misstatements or omissions in the registration statement attributable to us, and they are obligated to indemnify us for material misstatements or omissions in the registration statement attributable to them.

Anti-Takeover Effects of Delaware Law and Our Charter and Bylaws

Delaware law contains, and upon the completion of this offering our certificate of incorporation and our bylaws will contain, provisions that could have the effect of delaying, deferring or discouraging another party from acquiring control of us. These provisions, which are summarized below, are expected to discourage coercive takeover practices and inadequate takeover bids. These provisions are also designed to encourage persons seeking to acquire control of us to first negotiate with our board of directors.

Staggered Board; Removal of Directors

Upon the completion of this offering, our certificate of incorporation and bylaws will divide our board of directors into three classes with staggered three-year terms. In addition, a director will only be able to be removed for cause and only by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in an annual election of directors. Any vacancy on our board of directors, including a vacancy resulting from an enlargement of our board of directors, will only be able to be filled by vote of a majority of our directors then in office. The classification of our board of directors and the limitations on the removal of directors and filling of vacancies could make it more difficult for a third party to acquire, or discourage a third party from seeking to acquire, control of our company.

Stockholder Action by Written Consent; Special Meetings

Upon the completion of this offering, our certificate of incorporation will provide that any action required or permitted to be taken by our stockholders must be effected at a duly called annual or special meeting of such holders and may not be effected by any consent in writing by such holders. Upon the completion of this offering, our certificate of incorporation and bylaws will also provide that, except as otherwise required by law, special meetings of our stockholders can only be called by our chairman of the board, our Chief Executive Officer or our board of directors.

Advance Notice Requirements for Stockholder Proposals

Upon the completion of this offering, our bylaws will establish an advance notice procedure for stockholder proposals to be brought before an annual meeting of stockholders, including proposed nominations of persons for election to our board of directors. Stockholders at an annual meeting will only be able to consider proposals or nominations specified in the notice of meeting or brought before the meeting by or at the direction of our board of directors or by a stockholder of record on the record date for the meeting who is entitled to vote at the meeting and who has delivered timely written notice in proper form to our secretary of the stockholder's intention to bring such business before the meeting. These provisions could have the effect of delaying until the next stockholder meeting stockholder actions that are favored by the holders of a majority of our outstanding voting securities.

Delaware Business Combination Statute

Upon the completion of this offering, we will be subject to Section 203 of the General Corporation Law of the State of Delaware. Subject to certain exceptions, Section 203 prevents a publicly held Delaware corporation from engaging in a "business combination" with any "interested stockholder" for three years following the date that the person became an interested stockholder, unless the interested stockholder attained such status with the approval of our board of directors or unless the business combination is approved in a prescribed manner. A "business combination" includes, among other things, a merger or consolidation involving us and the "interested stockholder" and the sale of more than 10% of our assets. In general, an "interested stockholder" is any entity or person beneficially owning 15% or more of our outstanding voting stock and any entity or person affiliated with or controlling or controlled by such entity or person.

Amendment of Certificate of Incorporation and Bylaws

The General Corporation Law of the State of Delaware provides generally that the affirmative vote of a majority of the shares entitled to vote on any matter is required to amend a corporation's certificate of incorporation or bylaws, unless a corporation's certificate of incorporation or bylaws, as the case may be, requires a greater percentage. Effective upon the completion of this offering, our bylaws may be amended or repealed by a majority vote of our board of directors or by the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors. In addition, the affirmative vote of the holders of at least 75% of the votes that all of our stockholders would be entitled to cast in any annual election of directors is required to amend or repeal or to adopt any provisions inconsistent with any of the provisions of our certificate of incorporation described above under "—Staggered Board; Removal of Directors" and "—Stockholder Action by Written Consent; Special Meetings."

Exclusive Forum Selection

Effective upon completion of this offering, our certificate of incorporation will provide 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) shall be the sole and exclusive forum for (1) any derivative action or proceeding brought on behalf of our company, (2) 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, (3) any action asserting a claim against our company arising pursuant to any provision of the General Corporation Law of the State of Delaware or our certificate of incorporation or bylaws, or (4) any action asserting a claim against our company governed by the internal affairs doctrine. Although our certificate of incorporation contains the choice of forum provision described above, it is possible that a court could rule that such a provision is inapplicable for a particular claim or action or that such provision is unenforceable.

Listing on The NASDAQ Global Market

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "CATB."

Authorized but Unissued Shares

The authorized but unissued shares of common stock and preferred stock are available for future issuance without stockholder approval, subject to any limitations imposed by the listing requirements of The NASDAQ Global Market. These additional shares may be used for a variety of corporate finance transactions, acquisitions and employee benefit plans. The existence of authorized but unissued and

unreserved common stock and preferred stock could make it more difficult or discourage an attempt to obtain control of us by means of a proxy contest, tender offer, merger or otherwise.

Transfer Agent and Registrar

The transfer agent and registrar for our common stock will be American Stock Transfer & Trust Company.

SHARES ELIGIBLE FOR FUTURE SALE

Prior to this offering, there has been no public market for our common stock, and a liquid trading market for our common stock may not develop or be sustained after this offering. Future sales of substantial amounts of our common stock in the public market, including shares issued upon exercise of outstanding options, or the anticipation of these sales, could adversely affect market prices prevailing from time to time and could impair our ability to raise capital through sales of equity securities.

Based upon the 518,245 shares of our common stock that were outstanding on April 30, 2015, upon the closing of this offering, we will have outstanding 14,547,796 shares of our common stock, after giving effect to the issuance of 5,000,000 shares of our common stock in this offering and the conversion of all outstanding shares of our preferred stock into 9,029,551 shares of common stock upon the closing of this offering, and assuming no exercise by the underwriters of their over-allotment option and no exercise of options or warrants outstanding as of April 30, 2015.

Of the shares to be outstanding immediately after the closing of this offering, we expect that the 5,000,000 shares to be sold in this offering will be freely tradable without restriction under the Securities Act unless purchased by our "affiliates," as that term is defined in Rule 144 under the Securities Act. The remaining 9,547,796 shares of our common stock outstanding after this offering will be "restricted securities" under Rule 144, and we expect that substantially all of these restricted securities will be subject to the 180-day lock-up period under the lock-up agreements as described below. These restricted securities may be sold in the public market only if registered or pursuant to an exemption from registration, such as Rule 144 or Rule 701 under the Securities Act.

Rule 144

In general, under Rule 144, beginning 90 days after the date of this prospectus, any person who is not our affiliate and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, subject to the availability of current public information about us. In addition, under Rule 144, any person who is not our affiliate and has not been our affiliate at any time during the preceding three months and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the date of this prospectus, a person who is our affiliate or who was our affiliate at any time during the preceding three months may sell any unrestricted securities, as well as restricted securities that the person has beneficially owned for at least six months, including the holding period of any prior owner other than one of our affiliates, under Rule 144. Affiliates selling restricted or unrestricted securities may sell a number of shares within any three-month period that does not exceed the greater of:

- 1% of the number of shares of our common stock then outstanding, which will equal approximately 145,478 shares immediately after this offering; and
- the average weekly trading volume of our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a Notice of Proposed Sale of Securities Pursuant to Rule 144 with respect to the sale.

Sales under Rule 144 by our affiliates are also subject to manner of sale provisions and notice requirements and to the availability of current public information about us.

Upon expiration of the 180-day lock-up period described below, approximately 9,547,796 shares of our common stock will be eligible for sale under Rule 144, including shares eligible for resale

immediately upon the closing of this offering as described above. We cannot estimate the number of shares of our common stock that our existing stockholders will elect to sell under Rule 144.

Rule 701

In general, under Rule 701 of the Securities Act, any of our employees, consultants or advisors, other than our affiliates, who purchased shares from us in connection with a qualified compensatory stock plan or other written agreement is eligible to resell these shares 90 days after the date of this prospectus in reliance on Rule 144, but without compliance with the holding period requirements of Rule 144 and without regard to the volume of such sales or the availability of public information about us. Subject to the 180-day lock-up period described below, approximately 339,182 shares of our common stock will be eligible for sale in accordance with Rule 701.

Lock-Up Agreements

We, and each of our executive officers and directors and the holders of substantially all of our outstanding stock have agreed that, without the prior written consent of Citigroup Global Markets Inc. and Cowen and Company, LLC, on behalf of the underwriters, we and they will not, subject to limited exceptions, during the period ending 180 days after the date of this prospectus:

- offer, sell, contract to sell, pledge or otherwise dispose of, or enter into any transaction which is designed to, or might reasonably be expected to, result in the disposition of (whether by actual disposition or effective economic disposition due to cash settlement or otherwise), directly or indirectly, including the filing (or participation in the filing) of a registration statement (other than a registration statement on Form S-8) with the SEC with respect to, any shares of our capital stock or any securities convertible into, or exercisable or exchangeable for, such capital stock;

- establish or increase a put equivalent position or liquidate or decrease a call equivalent position with respect to any shares of our capital stock or any securities convertible into or exercisable or exchangeable for such capital stock, or publicly announce an intention to effect any such transaction; or

- publicly announce an intention to effect any of the foregoing.

Registration Rights

Subject to the lock-up agreements described above, upon the closing of this offering, the holders of an aggregate of 9,029,551 shares of our common stock, along with the holders of warrants to purchase an aggregate of 24,566 shares of common stock, will be entitled to various rights with respect to the registration of these shares under the Securities Act. Registration of these shares under the Securities Act would result in these shares becoming fully tradable without restriction under the Securities Act immediately upon the effectiveness of the registration. See "Description of Capital Stock—Registration Rights" for additional information. Shares covered by a registration statement will be eligible for sale in the public market upon the expiration or release from the terms of lock-up agreements applicable to such shares.

Stock Options and Warrants

As of April 30, 2015, we had outstanding options to purchase 1,478,731 shares of our common stock, of which options to purchase 657,292 shares were vested. Following this offering, we intend to file one or more registration statements on Form S-8 under the Securities Act to register all of the shares of our common stock subject to outstanding options and options and other awards issuable pursuant to the 2015 stock incentive plan, the 2015 employee stock purchase plan and our 2008 equity

incentive plan. Shares covered by these registration statements will then be eligible for sale in the public markets, subject to vesting restrictions, any applicable lock-up agreements described above and Rule 144 limitations applicable to affiliates.

As of April 30, 2015, we had outstanding warrants to purchase shares of our series B preferred stock that upon the closing of this offering will be exercisable for an aggregate of 24,566 shares of our common stock. As of April 30, 2015, we also had outstanding and exercisable warrants to purchase 34,839 shares of common stock. Any shares acquired through the exercise of these warrants will be eligible for sale subject to the lock-up agreements and securities laws described above.

MATERIAL U.S. FEDERAL INCOME AND ESTATE TAX CONSIDERATIONS FOR NON-U.S. HOLDERS OF COMMON STOCK

The following is a general discussion of material U.S. federal income and estate tax considerations relating to ownership and disposition of shares of our common stock by a non-U.S. holder. For purposes of this discussion, the term "non-U.S. holder" means a beneficial owner of our common stock that is not for U.S. federal income tax purposes:

- an individual who is a citizen or resident of the United States;
- a corporation, or other entity treated as a corporation for U.S. federal income tax purposes, created or organized in the United States or under the laws of the United States or of any political subdivision of the United States;
- an estate the income of which is subject to U.S. federal income taxation regardless of its source; or
- a trust if (1) a U.S. court is able to exercise primary supervision over the administration of the trust and one or more U.S. persons have authority to control all substantial decisions of the trust or (2) the trust has a valid election in effect under applicable U.S. Treasury Regulations to be treated as a U.S. person.

This discussion does not address the tax treatment of partnerships or other entities that are pass-through entities for U.S. federal income tax purposes or persons who hold their common stock through partnerships or other pass-through entities. A partner in a partnership or other pass-through entity that will hold our common stock should consult his, her or its own tax advisor regarding the tax consequences of acquiring, holding and disposing of our common stock through a partnership or other pass-through entity, as applicable.

This discussion is based on current provisions of the U.S. Internal Revenue Code of 1986, as amended, which we refer to as the Code, existing and proposed U.S. Treasury Regulations promulgated thereunder, current administrative rulings and judicial decisions, all as in effect as of the date of this prospectus and all of which are subject to change or to differing interpretation, possibly with retroactive effect. Any change could alter the tax consequences to non-U.S. holders described in this prospectus. There can be no assurance that the Internal Revenue Service, or the IRS, will not challenge one or more of the tax consequences described herein.

We assume in this discussion that a non-U.S. holder holds shares of our common stock as a capital asset (generally, property held for investment). This discussion does not address all aspects of U.S. federal income and estate taxation that may be relevant to a particular non-U.S. holder in light of that non-U.S. holder's individual circumstances nor does it address any aspects of U.S. state, local or non-U.S. taxes, the alternative minimum tax, or the Medicare tax on net investment income. This discussion also does not consider any specific facts or circumstances that may apply to a non-U.S. holder and does not address the special tax rules applicable to particular non-U.S. holders, such as:

- financial institutions;
- brokers or dealers in securities;
- tax-exempt organizations;
- pension plans;
- owners that hold our common stock as part of a straddle, hedge, conversion transaction, synthetic security or other integrated investment;
- insurance companies;

- regulated investment companies;
- controlled foreign corporations;
- passive foreign investment companies;
- persons that have a functional currency other than the U.S. dollar; and
- certain U.S. expatriates.

This discussion is for general information only and is not tax advice. Accordingly, all prospective non-U.S. holders should consult their own tax advisors regarding the U.S. federal, state, local and non-U.S. income tax and estate tax consequences of acquiring, holding and disposing of our common stock.

Distributions on Our Common Stock

If we pay distributions on our common stock, those distributions generally will constitute dividends for U.S. federal income tax purposes to the extent paid from our current or accumulated earnings and profits, as determined under U.S. federal income tax principles. If a distribution exceeds our current and accumulated earnings and profits, the excess will be treated as a tax-free return of the non-U.S. holder's investment, up to such holder's tax basis in the common stock. Any remaining excess will be treated as capital gain, subject to the tax treatment described below under the heading "Gain on Disposition of Common Stock." Any such distribution would also be subject to the discussion below under the section titled "Withholding and Information Reporting Requirements—FATCA."

As discussed under "Dividend Policy," we do not expect to pay cash dividends to holders of our common stock in the foreseeable future. In the event we do pay dividends, dividends paid to a non-U.S. holder generally will be subject to withholding of U.S. federal income tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

Dividends that are treated as effectively connected with a trade or business conducted by a non-U.S. holder within the United States, and if an applicable income tax treaty so provides, that are attributable to a permanent establishment or a fixed base maintained by the non-U.S. holder within the United States, are generally exempt from the 30% withholding tax if the non-U.S. holder satisfies applicable certification and disclosure requirements. However, such U.S. effectively connected income, net of specified deductions and credits, is taxed at the same graduated U.S. federal income tax rates applicable to U.S. persons (as defined in the Code). Any U.S. effectively connected income received by a non-U.S. holder that is a corporation may also, under certain circumstances, be subject to an additional "branch profits tax" at a 30% rate or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence.

A non-U.S. holder of our common stock who claims the benefit of an applicable income tax treaty between the United States and such holder's country of residence generally will be required to provide a properly executed IRS Form W-8BEN or W-8BEN-E (or successor form) and satisfy applicable certification and other requirements. Non-U.S. holders are urged to consult their own tax advisors regarding their entitlement to benefits under a relevant income tax treaty.

A non-U.S. holder that is eligible for a reduced rate of U.S. withholding tax under an income tax treaty may obtain a refund or credit of any excess amounts withheld by timely filing a U.S. tax return with the IRS.

Gain on Disposition of Common Stock

A non-U.S. holder generally will not be subject to any U.S. federal income tax on any gain realized upon such non-U.S. holder's sale, exchange or other disposition of shares of our common stock unless:

- the gain is effectively connected with the non-U.S. holder's conduct of a U.S. trade or business, and, if an applicable income tax treaty so provides, the gain is attributable to a permanent establishment or fixed base maintained by the non-U.S. holder in the United States; in these cases, the non-U.S. holder generally will be taxed on a net income basis at the graduated U.S. federal income tax rates applicable to U.S. persons and, if the non-U.S. holder is a corporation, the branch profits tax described above in "Distributions on Our Common Stock," may also apply;

- the non-U.S. holder is a nonresident alien who is present in the United States for 183 days or more in the taxable year of the disposition and certain other requirements are met, in which case the non-U.S. holder will be subject to a 30% tax (or such lower rate as may be specified by an applicable income tax treaty between the United States and such holder's country of residence) on the net gain derived from the disposition, which may be offset by U.S.-source capital losses of the non-U.S. holder, if any; or

- we are, or have been at any time during the five-year period preceding such disposition (or the non-U.S. holder's holding period, if shorter), a "U.S. real property holding corporation," unless our common stock is regularly traded on an established securities market and the non-U.S. holder held no more than 5% of our outstanding common stock, directly or indirectly, during the shorter of the five-year period ending on the date of the disposition or the period that the non-U.S. holder held our common stock. Generally, a corporation is a "U.S. real property holding corporation" if the fair market value of its "U.S. real property interests" (as defined in the Code and applicable regulations) equals or exceeds 50% of the sum of the fair market value of its worldwide real property interests plus its other assets used or held for use in a trade or business.

Although there can be no assurance, we believe that we are not currently, and we do not anticipate becoming, a "U.S. real property holding corporation" for U.S. federal income tax purposes. Further, no assurance can be provided that our common stock will be regularly traded on an established securities market for purposes of the rule described above. If we are determined to be a "U.S. real property holding corporation" and the exception described above does not apply, then a purchaser may withhold 10% of the proceeds payable to a non-U.S. holder from a sale of our common stock and the non-U.S. holder generally will be taxed on its net gain derived from the disposition at the graduated U.S. federal income tax rates applicable to U.S. persons.

Federal Estate Tax

Shares of our common stock that are owned or treated as owned by an individual who is not a citizen or resident of the United States (as specially defined for U.S. federal estate tax purposes) at the time of death are considered U.S. situs assets and will be included in the individual's gross estate for U.S. federal estate tax purposes. Such shares, therefore, may be subject to U.S. federal estate tax, unless an applicable estate tax or other treaty provides otherwise.

Information Reporting and Backup Withholding

We must report annually to the IRS and to each non-U.S. holder the gross amount of the distributions on our common stock paid to such holder and the tax withheld, if any, with respect to such distributions. Non-U.S. holders may have to comply with specific certification procedures to establish that the holder is not a U.S. person (as defined in the Code) in order to avoid backup

withholding at the applicable rate with respect to dividends on our common stock. Generally, a holder will comply with such procedures if it provides a properly executed IRS Form W-8BEN or W-8BEN-E (or other applicable Form W-8) or otherwise meets documentary evidence requirements for establishing that it is a non-U.S. holder, or otherwise establishes an exemption. Dividends paid to non-U.S. holders subject to withholding of U.S. federal income tax, as described above under the heading "Distributions on Our Common Stock," will generally be exempt from U.S. backup withholding.

Information reporting and backup withholding generally will apply to the proceeds of a disposition of our common stock by a non-U.S. holder effected by or through the U.S. office of any broker, U.S. or foreign, unless the holder certifies its status as a non-U.S. holder and satisfies certain other requirements, or otherwise establishes an exemption. Generally, information reporting and backup withholding will not apply to a payment of disposition proceeds to a non-U.S. holder where the transaction is effected outside the United States through a non-U.S. office of a broker. However, for information reporting purposes, dispositions effected through a non-U.S. office of a broker with substantial U.S. ownership or operations generally will be treated in a manner similar to dispositions effected through a U.S. office of a broker. Non-U.S. holders should consult their own tax advisors regarding the application of the information reporting and backup withholding rules to them.

Copies of information returns may be made available to the tax authorities of the country in which the non-U.S. holder resides or is incorporated under the provisions of a specific treaty or agreement.

Backup withholding is not an additional tax. Any amounts withheld under the backup withholding rules from a payment to a non-U.S. holder can be refunded or credited against the non-U.S. holder's U.S. federal income tax liability, if any, provided that an appropriate claim is timely filed with the IRS.

Withholding and Information Reporting Requirements—FATCA

The Foreign Account Tax Compliance Act, or FATCA, generally imposes U.S. federal withholding tax of 30% on payments of dividends of, and gross proceeds from the sale or disposition of, our common stock if paid to a foreign entity unless (i) if the foreign entity is a "foreign financial institution," the foreign entity undertakes certain due diligence, reporting, withholding, and certification obligations, (ii) if the foreign entity is not a "foreign financial institution," the foreign entity identifies certain of its U.S. investors, if any, or (iii) the foreign entity is otherwise exempt under FATCA. Under applicable U.S. Treasury regulations, withholding under FATCA generally (1) applies to payments of dividends on our common stock and (2) will apply to payments of gross proceeds from a sale or other disposition of our common stock made after December 31, 2016. Under certain circumstances, a non-U.S. holder may be eligible for refunds or credits of the tax. Withholding under FATCA generally will not be reduced or limited by bilateral income tax treaties. However, a non-U.S. holder may be exempt from FATCA withholding under an applicable intergovernmental agreement between the U.S. and a foreign government relating to the implementation of FATCA, provided that the non-U.S. holder and the foreign government comply with the terms of the agreement. Non-U.S. holders should consult their own tax advisors regarding the possible implications of FATCA on their investment in our common stock, and the entities through which they hold our common stock, including, without limitation, the process and deadlines for meeting the applicable requirements to prevent the imposition of this 30% withholding tax under FATCA.

UNDERWRITING

Citigroup Global Markets Inc. and Cowen and Company, LLC are acting as joint book-running managers of this offering and as representatives of the underwriters named below. Subject to the terms and conditions stated in the underwriting agreement dated the date of this prospectus, the underwriters named below have severally agreed to purchase, and we have agreed to sell to them, the number of shares of our common stock indicated below:

Underwriter	Number of Shares
Citigroup Global Markets Inc.	2,050,000
Cowen and Company, LLC	1,650,000
Oppenheimer & Co. Inc.	650,000
Wedbush Securities Inc.	650,000
Total	5,000,000

The underwriting agreement provides that the obligations of the underwriters to purchase the shares of our common stock included in this offering are subject to approval of legal matters by counsel and to other conditions. The underwriters are obligated to purchase all of the shares of our common stock (other than those covered by the over-allotment option described below) if they purchase any of the shares.

Shares of our common stock sold by the underwriters to the public will initially be offered at the initial public offering price set forth on the cover page of this prospectus. Any shares of our common stock sold by the underwriters to securities dealers may be sold at a discount from the initial public offering price not to exceed $0.5040 per share. After the initial offering of the shares of our common stock, if all the shares of our common stock are not sold at the initial public offering price, the underwriters may change the offering price and the other selling terms. The representatives have advised us that the underwriters do not intend to make sales to discretionary accounts.

If the underwriters sell more shares of our common stock than the total number set forth in the table above, we have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase up to 750,000 additional shares of our common stock at the initial public offering price less the underwriting discount. The underwriters may exercise the option solely for the purpose of covering over-allotments, if any, in connection with this offering. To the extent the option is exercised, each underwriter must purchase a number of additional shares of our common stock approximately proportionate to that underwriter's initial purchase commitment set forth in the table above. Any shares of our common stock issued or sold under the option will be issued and sold on the same terms and conditions as the other shares of our common stock that are the subject of this offering.

Certain of our existing principal stockholders have indicated an interest in purchasing an aggregate of up to approximately $15.0 million in shares of our common stock in this offering at the initial public offering price. However, because indications of interest are not binding agreements or commitments to purchase, the underwriters could determine to sell more, less or no shares to any of these potential purchasers and any of these potential purchasers could determine to purchase more, less or no shares in this offering.

We, our officers and directors and substantially all of our stockholders have agreed that, subject to specified limited exceptions, for a period of 180 days from the date of this prospectus, we and they will not, without the prior written consent of Citigroup Global Markets Inc. and Cowen and Company, LLC, offer, sell, contract to sell, pledge or otherwise dispose of, or hedge any shares of our capital stock or any securities convertible into, or exercisable or exchangeable for, our capital stock. Citigroup Global

Markets Inc. and Cowen and Company, LLC in their sole discretion may release any of the securities subject to these lock-up agreements at any time, which, in the case of officers and directors, shall be with notice.

Prior to this offering, there has been no public market for our shares. Consequently, the initial public offering price for the shares of our common stock was determined by negotiations between us and the representatives. Among the factors considered in determining the initial public offering price were our results of operations, our current financial condition, our future prospects, our markets, the economic conditions in and future prospects for the industry in which we compete, our management, and currently prevailing general conditions in the equity securities markets, including current market valuations of publicly traded companies considered comparable to our company. We cannot assure you, however, that the price at which the shares of our common stock will sell in the public market after this offering will not be lower than the initial public offering price or that an active trading market in our shares of common stock will develop and continue after this offering.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol "CATB."

The following table shows the per share and total underwriting discounts and commissions that we are to pay to the underwriters in connection with this offering. These amounts are shown assuming both no exercise and full exercise of the underwriters' over-allotment option:

	Paid by Catabasis	
	No exercise	Full exercise
Per share	$ 0.84	$ 0.84
Total	$ 4,200,000	$ 4,830,000

We estimate that expenses payable by us in connection with this offering, exclusive of underwriting discounts and commissions payable by us, will be approximately $2.2 million. We have also agreed to reimburse the underwriters for expenses in an amount up to $25,000 relating to the clearance of this offering with the Financial Industry Regulatory Authority, Inc.

In connection with this offering, the underwriters may purchase and sell shares of our common stock in the open market. Purchases and sales in the open market may include short sales, purchases to cover short positions, which may include purchases pursuant to the underwriters' over-allotment option, and other transactions that would stabilize, maintain or otherwise affect the price of our common stock.

- Short sales involve secondary market sales by the underwriters of a greater number of shares of our common stock than they are required to purchase in this offering:
  - "Covered" short sales are sales of shares of our common stock in an amount up to the number of shares of our common stock represented by the underwriters' over-allotment option.
  - "Naked" short sales are sales of shares of our common stock in an amount in excess of the number of shares of our common stock represented by the underwriters' over-allotment option.
- The underwriters can close out a short position by purchasing additional shares of our common stock, either pursuant to the underwriters' over-allotment option or in the open market.
  - To close a naked short position, the underwriters must purchase shares of our common stock in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

- To close a covered short position, the underwriters must purchase shares of our common stock in the open market or exercise their over-allotment option. In determining the source of shares of our common stock to close the covered short position, the underwriters will consider, among other things, the price of shares of our common stock available for purchase in the open market as compared to the price at which they may purchase shares of our common stock through their over-allotment option.

- As an additional means of facilitating this offering, the underwriters may bid for, and purchase, shares of our common stock on NASDAQ, as long as such bids do not exceed a specified maximum, to stabilize the price of the shares of our common stock.

Purchases to cover short positions and stabilizing purchases, as well as other purchases by the underwriters for their own accounts, may have the effect of preventing or retarding a decline in the market price of the shares. They may also cause the price of the shares of our common stock to be higher than the price that would otherwise prevail in the open market in the absence of these transactions. The underwriters may conduct these transactions on NASDAQ, in the over-the-counter market or otherwise. The underwriters are not required to engage in any of these transactions and may discontinue them at any time.

We have agreed to indemnify the underwriters against certain liabilities, including liabilities under the Securities Act, or to contribute to payments the underwriters may be required to make because of any of those liabilities.

A prospectus in electronic format may be made available on websites maintained by one or more of the underwriters or their respective affiliates. The representatives may agree with us to allocate a number of shares of our common stock to underwriters for sale to their online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' or their respective affiliates' websites and any information contained in any other website maintained by any of the underwriters or their respective affiliates is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors in this offering.

Conflicts of Interest

The underwriters are full-service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, principal investment, hedging, financing and brokerage activities. The underwriters and their respective affiliates may, from time to time, engage in transactions with and perform services for us in the ordinary course of their business for which they may receive customary fees and reimbursement of expenses. In the ordinary course of their various business activities, the underwriters and their respective affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (which may include bank loans or credit default swaps) for their own account and for the accounts of their customers and may at any time hold long and short positions in such securities and instruments. Such investments and securities activities may involve securities and/or instruments of ours or our affiliates. The underwriters and their affiliates may also make investment recommendations and/or publish or express independent research views in respect of such securities or financial instruments and may hold, or recommend to clients that they acquire, long or short positions in such securities and instruments.

Notice to Prospective Investors in the European Economic Area

In relation to each member state of the European Economic Area that has implemented the Prospectus Directive (each, a relevant member state), with effect from and including the date on which

the Prospectus Directive is implemented in that relevant member state (the relevant implementation date), an offer of shares of our common stock described in this prospectus may not be made to the public in that relevant member state other than:

- to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- to fewer than 100 or, if the relevant member state has implemented the relevant provision of the 2010 PD Amending Directive, 150 natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the relevant Dealer or Dealers nominated by us for any such offer; or
- in any other circumstances falling within Article 3(2) of the Prospectus Directive,

provided that no such offer of shares of our common stock shall require us or any underwriter to publish a prospectus pursuant to Article 3 of the Prospectus Directive.

For purposes of this provision, the expression an "offer of securities to the public" in any relevant member state means the communication in any form and by any means of sufficient information on the terms of the offer and the shares of our common stock to be offered so as to enable an investor to decide to purchase or subscribe for any shares of our common stock, as the expression may be varied in that member state by any measure implementing the Prospectus Directive in that member state, the expression "Prospectus Directive" means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the relevant member state) and includes any relevant implementing measure in the relevant member state, and the expression "2010 PD Amending Directive" means Directive 2010/73/EU.

The sellers of the shares of our common stock have not authorized and do not authorize the making of any offer of shares of our common stock through any financial intermediary on their behalf, other than offers made by the underwriters with a view to the final placement of the shares of our common stock as contemplated in this prospectus. Accordingly, no purchaser of the shares of our common stock, other than the underwriters, is authorized to make any further offer of the shares of our common stock on behalf of the sellers or the underwriters.

Notice to Prospective Investors in the United Kingdom

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (i) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, or the Order, or (ii) high net worth entities, and other persons to whom it may lawfully be communicated, falling within Article 49(2)(a) to (d) of the Order (each such person being referred to as a relevant person).

This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a relevant person should not act or rely on this document or any of its contents.

Notice to Prospective Investors in Australia

No prospectus or other disclosure document (as defined in the Corporations Act 2001 (Cth) of Australia, or Corporations Act) in relation to our common stock has been or will be lodged with the Australian Securities & Investments Commission, or ASIC. This document has not been lodged with

ASIC and is only directed to certain categories of exempt persons. Accordingly, if you receive this document in Australia:

- you confirm and warrant that you are either:
  - a "sophisticated investor" under section 708(8)(a) or (b) of the Corporations Act;
  - a "sophisticated investor" under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant's certificate to us which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made;
  - a person associated with the company under section 708(12) of the Corporations Act; or
  - a "professional investor" within the meaning of section 708(11)(a) or (b) of the Corporations Act, and to the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor, associated person or professional investor under the Corporations Act any offer made to you under this document is void and incapable of acceptance; and
- you warrant and agree that you will not offer any of our common stock for resale in Australia within 12 months of that common stock being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

Notice to Prospective Investors in France

Neither this prospectus nor any other offering material relating to the shares of our common stock described in this prospectus has been submitted to the clearance procedures of the *Autorité des Marchés Financiers* or of the competent authority of another member state of the European Economic Area and notified to the *Autorité des Marchés Financiers*. The shares of our common stock have not been offered or sold and will not be offered or sold, directly or indirectly, to the public in France. Neither this prospectus nor any other offering material relating to the shares of our common stock has been or will be:

- released, issued, distributed or caused to be released, issued or distributed to the public in France; or
- used in connection with any offer for subscription or sale of the shares of our common stock to the public in France.

Such offers, sales and distributions will be made in France only:

- to qualified investors (*investisseurs qualifiés*) and/or to a restricted circle of investors (*cercle restreint d'investisseurs*), in each case investing for their own account, all as defined in, and in accordance with articles L.411-2, D.411-1, D.411-2, D.734-1, D.744-1, D.754-1 and D.764-1 of the French Code monétaire et financier;
- to investment services providers authorized to engage in portfolio management on behalf of third parties; or
- in a transaction that, in accordance with article L.411-2-II-1°-or-2°-or 3° of the French Code *monétaire et financier* and article 211-2 of the General Regulations (*Règlement Général*) of the *Autorité des Marchés Financiers*, does not constitute a public offer (*appel public à l'épargne*).

The shares of our common stock may be resold directly or indirectly, only in compliance with articles L.411-1, L.411-2, L.412-1 and L.621-8 through L.621-8-3 of the French Code *monétaire et financier*.

Notice to Prospective Investors in Chile

The shares of our common stock are not registered in the Securities Registry (Registro de Valores) or subject to the control of the Chilean Securities and Exchange Commission (Superintendencia de Valores y Seguros de Chile). This prospectus and other offering materials relating to the offer of the shares do not constitute a public offer of, or an invitation to subscribe for or purchase, the shares in the Republic of Chile, other than to individually identified purchasers pursuant to a private offering within the meaning of Article 4 of the Chilean Securities Market Act (Ley de Mercado de Valores) (an offer that is not "addressed to the public at large or to a certain sector or specific group of the public").

Notice to Prospective Investors in Hong Kong

The shares of our common stock may not be offered or sold in Hong Kong by means of any document other than (i) in circumstances which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong), or (ii) to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder, or (iii) in other circumstances which do not result in the document being a "prospectus" within the meaning of the Companies Ordinance (Cap. 32, Laws of Hong Kong) and no advertisement, invitation or document relating to the shares of our common stock may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public in Hong Kong (except if permitted to do so under the laws of Hong Kong) other than with respect to shares of our common stock which are or are intended to be disposed of only to persons outside Hong Kong or only to "professional investors" within the meaning of the Securities and Futures Ordinance (Cap. 571, Laws of Hong Kong) and any rules made thereunder.

Notice to Prospective Investors in the State of Israel

In the State of Israel this prospectus shall not be regarded as an offer to the public to purchase shares of common stock under the Israeli Securities Law, 5728 - 1968, which requires a prospectus to be published and authorized by the Israel Securities Authority, if it complies with certain provisions of Section 15 of the Israeli Securities Law, 5728 - 1968, including, inter alia, if: (i) the offer is made, distributed or directed to not more than 35 investors, subject to certain conditions (the "Addressed Investors"); or (ii) the offer is made, distributed or directed to certain qualified investors defined in the First Addendum of the Israeli Securities Law, 5728 - 1968, subject to certain conditions (the "Qualified Investors"). The Qualified Investors shall not be taken into account in the count of the Addressed Investors and may be offered to purchase securities in addition to the 35 Addressed Investors. The company has not and will not take any action that would require it to publish a prospectus in accordance with and subject to the Israeli Securities Law, 5728 - 1968. We have not and will not distribute this prospectus or make, distribute or direct an offer to subscribe for our common stock to any person within the State of Israel, other than to Qualified Investors and up to 35 Addressed Investors.

Qualified Investors may have to submit written evidence that they meet the definitions set out in of the First Addendum to the Israeli Securities Law, 5728 - 1968. In particular, we may request, as a condition to be offered common stock, that Qualified Investors will each represent, warrant and certify to us and/or to anyone acting on our behalf: (i) that it is an investor falling within one of the categories listed in the First Addendum to the Israeli Securities Law, 5728 - 1968; (ii) which of the categories listed in the First Addendum to the Israeli Securities Law, 5728 - 1968 regarding Qualified Investors is applicable to it; (iii) that it will abide by all provisions set forth in the Israeli Securities Law, 5728 - 1968 and the regulations promulgated thereunder in connection with the offer to be issued common stock; (iv) that the shares of common stock that it will be issued are, subject to exemptions

available under the Israeli Securities Law, 5728 - 1968: (a) for its own account; (b) for investment purposes only; and (c) not issued with a view to resale within the State of Israel, other than in accordance with the provisions of the Israeli Securities Law, 5728 - 1968; and (v) that it is willing to provide further evidence of its Qualified Investor status. Addressed Investors may have to submit written evidence in respect of their identity and may have to sign and submit a declaration containing, inter alia, the Addressed Investor's name, address and passport number or Israeli identification number.

Notice to Prospective Investors in Japan

The shares of our common stock offered in this prospectus have not been and will not be registered under the Financial Instruments and Exchange Law of Japan. The shares of our common stock have not been offered or sold and will not be offered or sold, directly or indirectly, in Japan or to or for the account of any resident of Japan (including any corporation or other entity organized under the laws of Japan), except (i) pursuant to an exemption from the registration requirements of the Financial Instruments and Exchange Law and (ii) in compliance with any other applicable requirements of Japanese law.

Notice to Prospective Investors in Singapore

This prospectus has not been registered as a prospectus with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or invitation for subscription or purchase, of the shares of our common stock may not be circulated or distributed, nor may the shares of our common stock be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to persons in Singapore other than (i) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (ii) to a relevant person pursuant to Section 275(1), or any person pursuant to Section 275(1A), and in accordance with the conditions specified in Section 275 of the SFA or (iii) otherwise pursuant to, and in accordance with the conditions of, any other applicable provision of the SFA, in each case subject to compliance with conditions set forth in the SFA.

Where the shares of our common stock are subscribed or purchased under Section 275 of the SFA by a relevant party which is:

- a corporation (which is not an accredited investor (as defined in Section 4A of the SFA)) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or

- a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary of the trust is an individual who is an accredited investor,

shares, debentures and units of shares of our common stock and debentures of that corporation or the beneficiaries' rights and interest (howsoever described) in that trust shall not be transferred within six months after that corporation or that trust has acquired the shares of our common stock pursuant to an offer made under Section 275 of the SFA except:

- to an institutional investor (for corporations, under Section 274 of the SFA) or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and units of shares of our common stock and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than $200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions specified in Section 275 of the SFA;

- where no consideration is or will be given for the transfer; or

- where the transfer is by operation of law.

LEGAL MATTERS

The validity of the shares of our common stock offered hereby is being passed upon for us by Wilmer Cutler Pickering Hale and Dorr LLP. Cooley LLP is acting as counsel for the underwriters in connection with this offering.

EXPERTS

Ernst & Young, LLP, independent registered public accounting firm, has audited our financial statements at December 31, 2013 and 2014, and for the years then ended, as set forth in their report. We have included our financial statements in the prospectus and elsewhere in the registration statement in reliance on Ernst & Young LLP's report, given on their authority as experts in accounting and auditing.

WHERE YOU CAN FIND MORE INFORMATION

We have filed with the SEC a registration statement on Form S-1 under the Securities Act with respect to the shares of our common stock we are offering to sell. This prospectus, which constitutes part of the registration statement, does not include all of the information contained in the registration statement and the exhibits, schedules and amendments to the registration statement. For further information with respect to us and our common stock, we refer you to the registration statement and to the exhibits and schedules to the registration statement. Statements contained in this prospectus about the contents of any contract, agreement or other document are not necessarily complete, and, in each instance, we refer you to the copy of the contract, agreement or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference to such contract, agreement or document.

You may read and copy the registration statement of which this prospectus is a part at the SEC's public reference room, which is located at 100 F Street, N.E., Room 1580, Washington, DC 20549. You can request copies of the registration statement by writing to the Securities and Exchange Commission and paying a fee for the copying cost. Please call the SEC at 1-800-SEC-0330 for more information about the operation of the SEC's public reference room. In addition, the SEC maintains an Internet website, which is located at *www.sec.gov*, that contains reports, proxy and information statements and other information regarding issuers that file electronically with the SEC. You may access the registration statement of which this prospectus is a part at the SEC's Internet website. Upon completion of this offering, we will be subject to the information reporting requirements of the Securities Exchange Act of 1934, and we will file reports, proxy statements and other information with the SEC.

INDEX TO FINANCIAL STATEMENTS

REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders of
Catabasis Pharmaceuticals, Inc.

We have audited the accompanying balance sheets of Catabasis Pharmaceuticals, Inc. (the "Company") as of December 31, 2013 and 2014, and the related statements of operations and comprehensive loss, convertible preferred stock and stockholders' (deficit) equity, and cash flows for the years then ended. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Catabasis Pharmaceuticals, Inc. at December 31, 2013 and 2014, and the results of its operations and its cash flows for the years then ended in conformity with U.S. generally accepted accounting principles.

The accompanying financial statements have been prepared assuming that the Company will continue as a going concern. As discussed in Note 1 to the financial statements, the Company has incurred operating losses and negative cash flows from operations since inception and will be required to obtain additional financing, alternative means of financial support or both prior to December 31, 2015 in order to continue to fund its operations. These conditions raise substantial doubt about the Company's ability to continue as a going concern. Management's plans in regard to these matters are also described in Note 1. The financial statements do not include any adjustments that may result from the outcome of this uncertainty.

/s/ Ernst & Young LLP

Boston, Massachusetts
March 5, 2015, except for Note 15, as to which the date is June 11, 2015

Catabasis Pharmaceuticals, Inc.

Balance Sheets

(in thousands, except share and per share data)

	December 31,		March 31,	Pro Forma March 31,
	2013	2014	2015	2015
			(unaudited)	
Assets				
Current assets:				
Cash and cash equivalents	$ 30,474	$ 14,668	$ 24,303	$ 24,303
Prepaid expenses and other current assets	107	354	551	551
Total current assets	30,581	15,022	24,854	24,854
Property and equipment, net	308	288	260	260
Restricted cash	113	113	113	113
Other assets	—	541	1,498	1,498
Total assets	$ 31,002	$ 15,964	$ 26,725	$ 26,725
Liabilities, convertible preferred stock and stockholders' (deficit) equity				
Current liabilities:				
Accounts payable	$ 651	$ 1,132	$ 2,021	$ 2,021
Accrued expenses	2,279	2,793	1,467	1,467
Current portion of notes payable, net of discount		309	1,505	1,505
Total current liabilities	2,930	4,234	4,993	4,993
Deferred rent, net of current portion	110	67	55	55
Notes payable, net of current portion and discount	—	4,439	8,151	8,151
Other liability	—	23	39	39
Warrant liability	—	108	211	—
Commitments (Note 7)				
Series A convertible preferred stock, $0.001 par value; 68,837,703 shares authorized, issued and outstanding at December 31, 2013 and 2014 and March 31, 2015 (unaudited); 68,837,703 shares authorized and no shares issued or outstanding at March 31, 2015 (pro forma) (unaudited); aggregate liquidation preference of $48,186 at December 31, 2014 and March 31, 2015 (unaudited))	47,898	47,898	47,898	—
Series B convertible preferred stock, $0.001 par value; 37,830,473 shares authorized at December 31, 2013 and 2014 and 56,026,590 shares authorized at March 31, 2015 (unaudited); 34,129,571 shares issued and outstanding at December 31, 2013 and 2014 and 47,192,536 shares issued and outstanding at March 31, 2015 (unaudited); no shares issued and outstanding at March 31, 2015 (pro forma) (unaudited); (aggregate liquidation preference of $32,433 at December 31, 2014 and $44,847 at March 31, 2015 (unaudited))	32,248	32,248	44,579	—
Stockholders' (deficit) equity:				
Common stock, $0.001 par value; 132,000,000 shares authorized at December 31, 2013 and 2014 and 155,000,000 shares authorized at March 31, 2015 (unaudited); 393,346, 493,200, 518,245 and 9,547,796 shares issued and outstanding at December 31, 2013, December 31, 2014, March 31, 2015 (unaudited), and March 31, 2015 (pro forma) (unaudited), respectively	—	1	1	10
Additional paid-in capital	1,312	2,326	2,678	95,357
Accumulated deficit	(53,496)	(75,380)	(81,880)	(81,880)
Total stockholders' (deficit) equity	(52,184)	(73,053)	(79,201)	13,487
Total liabilities, convertible preferred stock and stockholders' (deficit) equity	$ 31,002	$ 15,964	$ 26,725	$ 26,725

Catabasis Pharmaceuticals, Inc.

Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
			(unaudited)	
Operating expenses:				
Research and development	$ 13,994	$ 15,686	$ 3,096	$ 4,616
General and administrative	4,125	5,995	1,373	1,744
Total operating expenses	18,119	21,681	4,469	6,360
Loss from operations	(18,119)	(21,681)	(4,469)	(6,360)
Other income (expense):				
Other income (expense), net	1	3	—	9
Interest expense	—	(206)	—	(149)
Total other income (expense), net	1	(203)	—	(140)
Net loss and comprehensive loss	$ (18,118)	$ (21,884)	$ (4,469)	$ (6,500)
Net loss per share—basic and diluted	$ (47.80)	$ (51.56)	$ (11.29)	$ (13.14)
Weighted-average number of common shares used in net loss per share—basic and diluted	379,025	424,477	395,774	494,590
Pro forma net loss per share—basic and diluted (unaudited)		$ (2.59)		$ (0.75)
Weighted average number of common shares used in pro forma net loss per share—basic and diluted (unaudited)		8,437,464		8,665,359

Catabasis Pharmaceuticals, Inc.

Statements of Convertible Preferred Stock and Stockholders' (Deficit) Equity

(in thousands, except share data)

	Series A Convertible Preferred Stock		Series B Convertible Preferred Stock		Common Stock		Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' (Deficit) Equity
	Shares	Amount	Shares	Amount	Number of Shares	Par Value			
Balance at December 31, 2012	55,700,752	$ 38,724	—	$ —	374,381	$ —	$ 942	$ (35,378)	$ (34,436)
Issuance of series A convertible preferred stock, net of issuance costs of $22	13,136,951	9,174	—	—	—	—	—	—	—
Issuance of series B convertible preferred stock, net of issuance costs of $185	—	—	34,129,571	32,248	—	—	—	—	—
Proceeds from exercises of common stock options	—	—	—	—	18,965	—	27	—	27
Stock-based compensation expense	—	—	—	—	—	—	343	—	343
Net loss	—	—	—	—	—	—	—	(18,118)	(18,118)
Balance at December 31, 2013	68,837,703	47,898	34,129,571	32,248	393,346	—	1,312	(53,496)	(52,184)
Proceeds from exercises of common stock options	—	—	—	—	99,854	1	117	—	118
Stock-based compensation expense	—	—	—	—	—	—	897	—	897
Net loss	—	—	—	—	—	—	—	(21,884)	(21,884)
Balance at December 31, 2014	68,837,703	47,898	34,129,571	32,248	493,200	1	2,326	(75,380)	(73,053)
Issuance of series B convertible preferred stock, net of issuance cost of $82 (unaudited)	—	—	13,062,965	12,331	—	—	—	—	—
Proceeds from exercises of common stock options (unaudited)	—	—	—	—	25,045	—	51	—	51
Stock-based compensation expense (unaudited)	—	—	—	—	—	—	301	—	301
Net loss (unaudited)	—	—	—	—	—	—	—	(6,500)	(6,500)
Balance at March 31, 2015	68,837,703	47,898	47,192,536	44,579	518,245	1	2,678	(81,880)	(79,201)
Conversion of series A convertible preferred stock into common stock (unaudited)	(68,837,703)	(47,898)	—	—	5,356,996	5	47,893	—	47,898
Conversion of series B convertible preferred stock into common stock (unaudited)	—	—	(47,192,536)	(44,579)	3,672,555	4	44,575	—	44,579
Conversion of series B preferred stock warrants into warrants for the purchase of common stock (unaudited)	—	—	—	—	—	—	211	—	211
Pro forma balance at March 31, 2015 (unaudited)	—	$ —	—	$ —	9,547,796	$ 10	$ 95,357	$ (81,880)	$ 13,487

Catabasis Pharmaceuticals, Inc.

Statements of Cash Flows

(in thousands)

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
			(unaudited)	
Operating activities				
Net loss	$ (18,118)	$ (21,884)	$ (4,469)	$ (6,500)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation and amortization	320	248	63	52
Stock-based compensation expense	343	897	146	301
Non-cash interest expense	—	74	—	38
Changes in operating assets and liabilities:				
Prepaid expenses and other current assets	75	(208)	(58)	(194)
Accounts payable	(129)	481	462	(79)
Accrued expenses	1,156	8	(742)	(837)
Deferred rent	(13)	(28)	(5)	(9)
Net cash used in operating activities	(16,366)	(20,412)	(4,603)	(7,228)
Investing activities				
Purchases of available-for-sale securities	—	(4,976)	—	—
Sales of available-for-sale securities	—	4,976	—	—
Purchases of property and equipment	(43)	(228)	(14)	(25)
Net cash used in investing activities	(43)	(228)	(14)	(25)
Financing activities				
Proceeds from issuance of preferred stock, net of issuance costs	41,422	—	—	12,331
Proceeds from exercise of common stock options	27	118	19	51
Proceeds from borrowings	—	5,000	—	5,000
Deferred initial public offering costs	—	—	—	(487)
Debt issuance costs	—	(284)	—	(7)
Net cash provided by financing activities	41,449	4,834	19	16,888
Net increase (decrease) in cash and cash equivalents	25,040	(15,806)	(4,598)	9,635
Cash and cash equivalents at beginning of period	5,434	30,474	30,474	14,668
Cash and cash equivalents at end of period	$ 30,474	$ 14,668	$ 25,876	$ 24,303
Supplemental disclosures of cash flow information:				
Cash paid during the period for interest	$ —	$ 100	$ —	$ 94
Non-cash financing activities:				
Warrants for the purchase of series B preferred stock issued in conjunction with credit facility	$ —	$ 110	$ —	$ 107
Deferred initial public offering cost in accounts payable and accrued liabilities	$ —	$ 492	$ —	$ 475

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

1. Nature of Business

Catabasis Pharmaceuticals, Inc. (the "Company") was incorporated on June 26, 2008, as a Delaware corporation with operations based in Cambridge, Massachusetts. The Company is dedicated to the discovery and development of medicines to treat inflammatory and metabolic diseases.

Liquidity

The Company has incurred operating losses and negative cash flows from operations since inception. As of December 31, 2014 and March 31, 2015, the Company had an accumulated deficit of $75.4 million and $81.9 million, respectively. 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. To date, the Company has funded its operations primarily through private placements of its convertible preferred stock and the issuance of debt. On August 27, 2014, the Company entered into a loan and security agreement (the "Credit Facility"). The Credit Facility, as amended on March 31, 2015, provides for initial borrowings of $5.0 million under a term loan and additional borrowings of up to $20.0 million under certain conditions (Note 6), of which $5.0 million was drawn on March 31, 2015. An additional $5.0 million was available to be drawn until May 31, 2015, subject to completion of an additional equity financing with net cash proceeds of at least $8.0 million. The Company did not draw the $5 million that was available through May 31, 2015. An additional $10.0 million is 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. In March 2015, the Company issued and sold an aggregate of 13,062,965 shares of its series B preferred stock ("Series B Preferred Stock") for gross proceeds of $12.4 million.

The Company will be required to obtain additional funding, alternative means of financial support, or both, in order to continue to fund its operations. These factors raise substantial doubt about the Company's ability to continue as a going concern. The Company intends to pursue a private offering of equity securities and a public offering of its common stock (the "Common Stock") to fund future operations. However, if the Company is unable to complete a sufficient private or public offering in a timely manner, it would need to pursue other financing alternatives, including private financing of debt or equity or collaboration agreements. There can be no assurances, however, that the current operating plan will be achieved or that additional funding will be available on terms acceptable to the Company, or at all.

The accompanying financial statements have been prepared assuming the Company will continue as a going concern, which contemplates the realization of assets and liabilities and commitments in the normal course of business. The financial statements for the year ended December 31, 2014 and the three months ended March 31, 2015 do not include any adjustments to reflect the possible future effects on the recoverability and classification of assets or the amounts and classification of liabilities that may result from this uncertainty related to the Company's ability to continue as a going concern.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies

Basis of Presentation

The accompanying financial statements have been prepared in accordance with United States (U.S.) 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.

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 utilizes significant estimates and assumptions in determining the fair value of its Common Stock. The board of directors determined the estimated fair value of the Common Stock based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which the Company sold shares of 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 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.

Unaudited Interim Financial Statements

The unaudited interim financial statements as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 and the related interim information contained within the notes to the financial statements are unaudited. The unaudited interim financial statements have been prepared on the same basis as the annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary to present fairly the Company's financial position as of March 31, 2015 and its results of operations and cash flows for the three months ended March 31, 2014 and 2015. The results of operations and cash flows for the three

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

months ended March 31, 2015 are not necessarily indicative of the results to be expected for the year ending December 31, 2015 or for any other future annual or interim period.

Unaudited Pro Forma Financial Information

In March 2015, the Company's board of directors authorized the management of the Company to file a registration statement with the Securities and Exchange Commission ("SEC") for the Company to sell shares of its Common Stock to the public. Upon the closing of a qualified public offering (as defined in the Company's certificate of incorporation), all of the Company's outstanding convertible preferred stock will automatically convert into Common Stock. The unaudited pro forma balance sheet and statement of convertible preferred stock and stockholders' (deficit) equity as of March 31, 2015 assume (1) the conversion of all outstanding convertible preferred stock into shares of Common Stock, and (2) the reclassification of the Company's warrant liability to additional paid-in capital, as a result of warrants to purchase preferred stock becoming warrants to purchase Common Stock upon the completion of this proposed offering.

Off-Balance Sheet Risk and Concentrations of Credit Risk

The Company has no off-balance sheet risk, such as foreign exchange contracts, option contracts or other foreign hedging arrangements. Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents and restricted cash. The primary objectives for the Company's investment portfolio are the preservation of capital and the maintenance of liquidity. The Company's investment policy includes guidelines on the quality of the institutions and financial instruments and defines allowable investments that the Company believes minimizes the exposure to concentration of credit risk.

Cash and Cash Equivalents

The Company considers highly liquid investments with a maturity of three months or less when purchased to be cash equivalents. Cash equivalents, which consist primarily of money market funds, are stated at fair value. Cash and cash equivalents consist of the following (in thousands):

	December 31,		March 31,
	2013	2014	2015
Cash	$ 1,472	$ 1,162	$ 5,798
Money market fund	29,002	13,506	18,505
	$ 30,474	$ 14,668	$ 24,303

Available-for-Sale Investments

The Company classifies all short-term investments with a remaining maturity when purchased of greater than three months as available-for-sale. Available-for-sale securities are recorded at fair value, with the unrealized gains and losses reported in other comprehensive income (loss). The amortized cost of debt securities is adjusted for the amortization of premiums and accretion of discounts to maturity.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

Such amortization is included in other income (expense), net. Realized gains and losses, interest, dividends and declines in value judged to be other-than-temporary are included in other income (expense), net.

The cost of securities sold is based on the specific identification method for purposes of recording realized gains and losses. To determine whether an other-than-temporary impairment exists, the Company considers whether it has the ability and intent to hold the investment until a market price recovery, and whether evidence indicating the recoverability of the cost of the investment outweighs evidence to the contrary. There were no available-for-sale investments outstanding at December 31, 2014 or March 31, 2015.

Deferred Public Offering Costs

Deferred public offering costs, which primarily consist of direct, incremental legal and accounting fees relating to the Company's initial public offering ("IPO"), are capitalized within other assets. The deferred issuance costs will be offset against IPO proceeds upon the consummation of the offering. In the event the offering is terminated, deferred offering costs will be expensed. The Company incurred $0.5 million and $1.5 million in deferred IPO costs as of December 31, 2014 and March 31, 2015, respectively, that are classified in other assets on the balance sheet.

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 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 11) using Level 3 inputs.

The following table summarizes the assets and liabilities measured at fair value on a recurring basis at December 31, 2013 (in thousands):

	Balance at December 31, 2013	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 29,002	$ 29,002	$ —	$ —
Total	$ 29,002	$ 29,002	$ —	$ —

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

The following table summarizes the assets and liabilities measured at fair value on a recurring basis at December 31, 2014 (in thousands):

	Balance at December 31, 2014	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 13,506	$ 13,506	$ —	$ —
Total	$ 13,506	$ 13,506	$ —	$ —
Liabilities:				
Warrant liability	$ 108	$ —	$ —	$ 108
Total	$ 108	$ —	$ —	$ 108

The following table summarizes the assets and liabilities measured at fair value on a recurring basis at March 31, 2015 (in thousands):

	Balance at March 31, 2015	Level 1	Level 2	Level 3
Assets:				
Cash equivalents	$ 18,505	$ 18,505	$ —	$ —
Total	$ 18,505	$ 18,505	$ —	$ —
Liabilities:				
Warrant liability	$ 211	$ —	$ —	$ 211
Total	$ 211	$ —	$ —	$ 211

The carrying amounts reflected in the balance sheets for cash and cash equivalents, restricted cash, prepaid expenses and other current assets, other assets, accounts payable and accrued expenses approximate their fair values at December 31, 2013 and 2014 and March 31, 2015, due to their short-term nature.

There have been no changes to the valuation methods during the years ended December 31, 2013 and 2014 or the three months ended March 31, 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 years ended December 31, 2013 and 2014 or the three months ended March 31, 2015. At March 31, 2015, the carrying value of the Company's debt approximated fair value, which was determined using Level 3 inputs, including a quoted interest rate.

Property and Equipment

Property and equipment consist of laboratory equipment, computer equipment, leasehold improvements and furniture and fixtures. Property and equipment is stated at cost and depreciated using the straight-line method over the estimated useful lives of the respective assets. Costs of major

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

additions and betterments are capitalized; maintenance and repairs, which do not improve or extend the life of the respective assets, are charged to expense as incurred. Upon retirement or sale, the cost of the disposed asset and the related accumulated depreciation are removed from the accounts and the resulting gain or loss is recognized.

Impairment of Long-Lived Assets

The Company continually evaluates whether events or circumstances have occurred that indicate that the estimated remaining useful life of its long-lived assets may warrant revision or that the carrying value of these assets may be impaired. The Company has not recognized any impairment charges through March 31, 2015.

Warrant Liability

The Company accounts for warrant instruments that either conditionally or unconditionally obligate the Company to transfer assets regardless of the timing of the redemption feature or price, even though the underlying shares may be classified as permanent or temporary equity. These warrants are subject to revaluation at each balance sheet date, and any changes in fair value are recorded as a component of other income (expense), until the earlier of their exercise or expiration or the completion of a liquidation event, including the completion of an initial public offering, at which time the warrant liability may be reclassified to stockholders' (deficit) equity if the criteria for recording the warrant as an equity instrument are met. The warrant liability totaled $0, $0.1 million, and $0.2 million at December 31, 2013 and 2014 and March 31 2015, respectively (Note 11).

Research and Development Expenses

Research and development costs are expensed as incurred. Research and development costs include salaries and personnel-related costs, stock-based compensation, consulting fees, fees paid for contract research services, the costs of laboratory equipment and facilities and other external costs. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are deferred. The deferred amounts are expensed as the related goods are delivered or the services are performed.

Stock-Based Compensation

The Company accounts for its stock-based compensation awards in accordance with Accounting Standards Codification ("ASC") Topic 718, *Compensation—Stock Compensation* ("ASC 718"). ASC 718 requires all share-based payments to employees, including grants of employee stock options, to be recognized in the statements of operations based on their grant date fair values. For stock options granted to employees and to members of the board of directors for their services on the board of directors, the Company estimates the grant date fair value of each option award using the Black- Scholes option-pricing model. The use of the Black-Scholes option-pricing model requires management to make assumptions with respect to the expected term of the option, the expected volatility of the Common Stock consistent with the expected life of the option, risk-free interest rates and expected dividend yields of the Common Stock. For awards subject to service-based vesting conditions, the

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

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

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

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

During the years ended December 31, 2013 and 2014 and the three months ended March 31, 2014 and 2015, the Company recorded stock-based compensation expense for employee and non-employee stock options and restricted stock, which was allocated as follows in the statements of operations (in thousands):

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
Research and development expense	$ 224	$ 434	$ 81	$ 168
General and administrative expense	119	463	65	133
	$ 343	$ 897	$ 146	$ 301

No related tax benefits were recognized for the years ended December 31, 2013 and 2014 or for the three months ended March 31, 2014 and 2015.

Grant Awards

In the years ended December 31, 2013 and 2014 and the three months ended March 31 2014 and 2015, the Company received $35,000, $0, $0 and $50,000, respectively, in grants from the Muscular Dystrophy Association and Parent Project for Muscular Dystrophy. The awards were recorded as a reduction to research and development expenses as the related expenses were incurred in the Company's statements of operations and comprehensive loss.

Net Loss Per Share and Unaudited Pro Forma 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.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

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:

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
Convertible preferred stock	8,012,988	8,012,988	8,012,988	9,029,551
Stock options	846,885	1,226,140	1,264,229	1,388,218
Common stock warrants	34,839	34,839	34,839	34,839
Preferred stock warrants	—	12,283	—	24,566
	8,894,712	9,286,250	9,312,056	10,477,174

Unaudited pro forma net loss per share is computed using the weighted-average number of common shares outstanding after giving effect to the conversion of all convertible preferred stock into shares of the Common Stock as if such conversion had occurred at the beginning of the period presented, or the date of original issuance, if later.

Income Taxes

The Company provides deferred tax assets and liabilities for the expected future tax consequences of temporary differences between the Company's financial statement carrying amounts and the tax basis of assets and liabilities using enacted tax rates expected to be in effect in the years in which the differences are expected to reverse. A valuation allowance is provided to reduce the deferred tax assets to the amount that will more likely than not be realized.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC Topic 740, *Expenses—Income Taxes*. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2013 and 2014 and March 31, 2015, the Company did not have any significant uncertain tax positions.

Segment Information

Operating segments are identified as components of an enterprise for which separate discrete financial information is available for evaluation by the chief operating decision maker, or decision making group, in making decisions on how to allocate resources and assess performance. The Company views its operations and manages its business in one operating segment. The Company operates in one geographic segment.

Comprehensive Loss

Comprehensive loss is defined as the change in equity of a business enterprise during a period from transactions and other events and circumstances from non-owner sources. For the years ended

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

2. Summary of Significant Accounting Policies (Continued)

December 31, 2013 and 2014 and for the three months ended March 31, 2014 and 2015, comprehensive loss was equal to net loss.

Recently Issued or Adopted 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 June 2014, the FASB issued Accounting Standards Update ("ASU") 2014-10, *Development Stage Entities (Topic 915)* ("ASU 2014-10"), which removes the definition of a development stage entity from the ASC, thereby removing the financial reporting distinction between development stage entities and other reporting entities. Accordingly, ASU 2014-10 eliminates the requirements for development stage entities to (1) present inception-to-date information in the statements of operations, cash flows and shareholder equity, (2) label financial statements as those of a development stage entity, (3) disclose a description of the development stage activities in which the entity is engaged and (4) disclose in the first year in which the entity is no longer a development stage entity that in prior years it had been in the development stage. ASU 2014-10 is effective for public business entities for annual periods beginning after December 15, 2014, and interim reporting periods beginning after December 15, 2015, with early adoption permitted. The Company early adopted the provisions of ASU 2014-10 in these financial statements.

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 consolidated financial statements.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

3. Property and Equipment

Property and equipment and related accumulated depreciation were as follows (in thousands):

	Estimated Useful Life (Years)	December 31, 2013	December 31, 2014	March 31, 2015
Lab equipment	3	$ 1,068	$ 1,269	$ 1,281
Computer equipment	3	108	110	122
Furniture and fixtures	5	30	30	30
Leasehold improvements	Lesser of useful life or remaining lease term	57	57	57
		1,263	1,466	1,490
Less accumulated depreciation and amortization		(955)	(1,178)	(1,230)
Total property and equipment, net		$ 308	$ 288	$ 260

Depreciation and amortization expense was $0.3 million, $0.2 million, $0.1 million and $0.1 million for the years ended December 31, 2013 and 2014 and for the three months ended March 31, 2014 and 2015, respectively.

4. Restricted Cash

At December 31, 2013 and 2014 and March 31, 2015, the Company had an outstanding letter of credit for $0.1 million as a security deposit for its operating lease agreement for office space (Note 7). The Company is required to maintain this deposit for the duration of the lease agreement.

5. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31, 2013	December 31, 2014	March 31, 2015
Accrued compensation	$ 785	$ 796	$ 433
Accrued contracted research costs	1,266	1,109	727
Accrued professional fees	135	791	51
Accrued other	93	97	256
Total	$ 2,279	$ 2,793	$ 1,467

6. 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

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

6. Notes Payable (Continued)

$5.0 million from the issuance of promissory notes under another term loan ("Term Loan B"). Of the additional $15.0 million available, $5.0 million ("Term Loan C") was available to be drawn, until May 31, 2015, subject to completion of an additional 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. An additional $10.0 million ("Term Loan D") is 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. All promissory notes issued 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. As of March 31, 2015, the Company had not drawn the Term Loan C and Term Loan D.

The Company is obligated to make monthly, interest-only payments on any term loans funded 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%. 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 as an other long-term liability in the accompanying balance sheets. The effective interest rate as of December 31, 2014 for the Term Loan A, including the final payment and non-cash interest, was 11.9%. The effective interest rate as of March 31, 2015 was 11.2%.

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.

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. 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. The Company recorded interest expense of $14,000 for the year ended December 31, 2014 and $11,000 for the three months ended March 31, 2015 related to accretion of such discount. The Company recorded warrant liabilities (Note 11) of $0.1 million for both the 2014 Warrants and 2015 Warrants that are classified as long-term liabilities in the accompanying balance sheets.

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

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

6. Notes Payable (Continued)

indebtedness or liens; paying dividends; making certain investments; and entering into certain other business transactions.

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 the Company 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 the business, operations or conditions (financial or otherwise) of the Company or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against the Company in an amount greater than $250,000. The occurrence of a material adverse change could result in acceleration of the payment of the debt. At December 31, 2014 and March 31, 2015, 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 5.00%, above the rate that is otherwise applicable.

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

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 December 31, 2014 are as follows (in thousands):

Year ending December 31:	
2014	$ —
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 discount	$ 4,439

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

6. Notes Payable (Continued)

Estimated future principal payments due under Term Loans A and B are as follows as of March 31, 2015 (in thousands):

Year ending December 31:	
2015	$ 833
2016	3,333
2017	3,333
2018	2,501
Total	$ 10,000
Less: discount for warrants and costs paid to lender	(344)
Less: current portion	(1,505)
Note payable, net of current portion and discount	$ 8,151

During the year ended December 31, 2014, the Company recognized $0.2 million of interest expense related to the Credit Facility. During the three months ended March 31, 2015, the Company recognized $0.1 million of interest expense related to the Credit Facility. The Company had no debt outstanding as of December 31, 2013.

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.

The future minimum lease payments under the non-cancelable operating lease as of December 31, 2014 are as follows (in thousands):

Year ending December 31:	
2015	$ 756
2016	760
2017	378
Total	$ 1,894

Rent expense for the years ended December 31, 2013 and 2014 and for the three months ended March 31, 2014 and 2015 was $0.7 million, $0.7 million, $0.2 million and $0.2 million, respectively.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

8. Convertible Preferred Stock

On April 17, 2013, the Company's board of directors authorized the Company to increase the authorized number of shares under the Series A convertible preferred stock ("Series A Preferred Stock") financing to 68,837,703. The Company subsequently issued 636,952 shares of Series A Preferred Stock at $0.70 per share, and received net proceeds of $0.4 million.

On October 31, 2013, the Company completed a Series B Preferred Stock financing and issued 34,129,571 shares of Series B Preferred Stock at $0.9503 per share, for net proceeds of $32.2 million.

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

The Company assessed the Series A and Series B Preferred Stock for any beneficial conversion features or embedded derivatives, including the conversion option, that would require bifurcation from the Series A and Series B Preferred Stock and receive separate accounting treatment. Based on the Company's determination that the Preferred Stock is an "equity host," the Company determined that all features of the Preferred Stock are clearly and closely related to the equity host, and do not require bifurcation as a derivative liability. On the date of issuance, the fair value of Common Stock into which the Series A and Series B Preferred Stock was convertible was less than the effective conversion price of the Series A and Series B Preferred Stock, and as such, there was no intrinsic value of the conversion option at the commitment date.

The Series A Preferred Stock and Series B Preferred Stock (collectively, the "Preferred Stock") have the following rights and preferences:

Voting

Holders of Preferred Stock are entitled to a number of votes equal to the number of shares of Common Stock into which the Preferred Stock is convertible on the date of record. Holders of the Preferred Stock vote together with the holders of Common Stock as a single class, except for the election of the Company's board of directors. For such election, holders of Series A Preferred Stock vote exclusively as a separate class to elect three directors and holders of Series B Preferred Stock vote exclusively as a separate class to elect one director. The remainder of the directors of the Company are elected by the holders of Common Stock and the Preferred Stock voting as a single class. The approval of 60% of the then outstanding shares of Preferred Stock is required for certain events that may impact the rights and preferences of the Preferred Stock, including the liquidation, winding up or dissolution of the business, an increase or decrease in the number of shares of Preferred Stock or Common Stock authorized for issuance, amendment of the certificate of incorporation in a manner that adversely effects the rights, preferences or privileges of the Preferred Stock, the purchase or redemption of or payment or declaration of dividends on shares of Preferred Stock or Common Stock, authorization of debt securities exceeding $1.0 million, or the adoption or amendment of any equity-based compensation plans to increase the number of shares issuable thereunder.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

8. Convertible Preferred Stock (Continued)

Dividends

Dividends accrue on Preferred Stock from the date of issuance at a rate of 8% per annum per share when and if declared by the Company's board of directors. Dividends are not cumulative. The Company has not declared any dividends to date. The Company shall not pay a dividend to other classes of securities until the holders of Preferred Stock have received a dividend of 8% per annum, per share from the date of issuance.

Liquidation Preference

In the event of any liquidation, dissolution, or winding up of the affairs of the Company, the holders of the then-outstanding Preferred Stock shall receive on a pari passu basis, before any payment shall be made to the holders of Common Stock, the greater of (1) $0.70 per share for Series A Preferred Stock plus all declared, but unpaid, dividends, and (2) $0.9503 per share for Series B Preferred Stock plus all declared, but unpaid, dividends, or (3) such amount per share of Preferred Stock payable as if converted into Common Stock. After the payment of any preferential amount to holders of Preferred Stock, any remaining assets of the Company shall be distributed ratably among the holders of Common Stock. If the assets or surplus funds to be distributed to the holders of the Preferred Stock are insufficient to permit the payment to such holders of their full preferential amount, the assets and surplus funds legally available for distribution shall be distributed ratably among the holders of the Preferred Stock in proportion to the full preferential amount that each holder is otherwise entitled to receive. As the Preferred Stock may become redeemable upon an event that is outside of the control of the Company, the Preferred Stock has been classified outside of permanent equity. Since the Preferred Stock is not initially redeemable and it is not probable that it will become redeemable, the carrying amount of the Preferred Stock has not been adjusted.

Conversion

Each share of Preferred Stock is convertible at the option of the holder into a number of fully paid shares of Common Stock as determined by dividing $0.70 by the conversion price in effect at the time for the Series A Preferred Stock and by dividing $0.9503 by the conversion price in effect at the time for the Series B Preferred Stock. The initial conversion prices of Series A and Series B Preferred Stock were $0.70 per share and $0.9503 per share, respectively, and are subject to adjustment in accordance with anti-dilution provisions contained in the Company's certificate of incorporation. Conversion is automatic immediately upon the closing of a firm commitment underwritten public offering in which the public offering price equals or exceeds $24.42 per share and the gross proceeds are not less than $50,000,000, or upon the written consent of the holders of at least 60% of the then-outstanding shares of Preferred Stock. Effective June 11, 2015, upon the closing of a firm commitment underwritten public offering in which the aggregate proceeds raised in the offering equal or exceed $50 million, the Company's Preferred Stock shall be automatically converted into common stock at the applicable conversion price (Note 15).

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

9. Common Stock

The voting, dividend and liquidation rights of holders of Common Stock are subject to and qualified by the rights, powers and preferences of the holders of Preferred Stock. The Company's Common Stock has the following characteristics:

Voting

The holders of Common Stock are entitled to one vote for each share of Common Stock held at all meetings of stockholders and written actions in lieu of meetings.

Dividends

The holders of Common Stock are entitled to receive dividends, if and when declared by the board of directors. Cash dividends may not be declared or paid to holders of Common Stock until paid on each series of outstanding Preferred Stock in accordance with their respective terms. No dividends have been declared or paid from the Company's inception through March 31, 2015.

Liquidation

After payment to the holders of Preferred Stock of their liquidation preferences, the holders of Common Stock are entitled to share ratably in the Company's assets available for distribution to stockholders, in the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company or upon the occurrence of a deemed liquidation event, as defined in the Company's certificate of incorporation.

10. Reserved for Future Issuance

The Company had reserved for future issuance the following number of shares of Common Stock as of December 31, 2013 and 2014 and March 31, 2015:

	December 31,		March 31,
	2013	2014	2015
Conversion of Series A Preferred Stock	5,356,996	5,356,996	5,356,996
Conversion of Series B Preferred Stock	2,655,992	2,655,992	3,672,555
Warrants for the purchase of Preferred Stock	—	12,283	24,566
Warrants for the purchase of Common Stock	34,839	34,839	34,839
Options to purchase Common Stock	1,484,497	1,385,341	1,503,972
	9,532,324	9,445,451	10,592,928

11. 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 Preferred Stock at an exercise price of $0.9503 per share to the lenders in connection with the Credit Facility (Note 6). The 2014 Warrants and 2015 Warrants were exercisable immediately on issuance and have a seven-year life. The fair value

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

11. Warrants (Continued)

of the 2014 Warrants and 2015 Warrants are re-measured at each reporting date using then-current assumptions. The 2014 Warrants and 2015 Warrants were valued using the Black-Scholes option-pricing model. Significant changes to the key assumptions used in the valuation could result in significantly different fair values of the 2014 Warrants and 2015 Warrants. The following assumptions were used in valuing the 2014 Warrants:

	August 27, 2014	December 31, 2014	March 31, 2015
Risk-free interest rate	2.05%	1.95%	1.63%
Expected dividend yield	0.00%	0.00%	0.00%
Expected term (in years)	7.0	6.7	6.4
Expected volatility	80.52%	78.16%	78.04%

The following assumptions were used in valuing the 2015 Warrants:

	March 31, 2015
Risk-free interest rate	1.68%
Expected dividend yield	0.00%
Expected term (in years)	7.0
Expected volatility	77.61%

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 January 1, 2014	$ —
Issuance of warrants at fair value	110
Change in fair value, recorded as a component of other expense	(2)
Balance at December 31, 2014	$ 108
Issuance of warrants at fair value	107
Change in fair value, recorded as a component of other expense	(4)
Balance at March 31, 2015	$ 211

No portion of the 2014 Warrants or 2015 Warrants had been exercised as of March 31, 2015.

At various dates from 2008 through 2010, the Company issued warrants to purchase 34,839 shares of Common Stock at an exercise price of $1.67 to various individuals, including its founders and employees of the Company. The warrants have a six-year term.

12. Stock Incentive Plan

The Company maintains the 2008 Stock Incentive Plan (the "Plan") for employees, directors, consultants and advisors to the Company. The Plan provides for the grant of incentive and

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

12. Stock Incentive Plan (Continued)

non-qualified stock options and restricted stock grants as determined by the Company's board of directors. As of December 31, 2014 and March 31, 2015, the Company had reserved 1,699,478 shares and 1,843,154 shares of Common Stock, respectively, under the Plan, of which 116,455 shares remained available for future issuance as of March 31, 2015. Under the Plan, stock options may not be granted with exercise prices at less than fair value on the date of the grant.

Terms of stock option agreements, including vesting requirements, are determined by the Company's board of directors, subject to the provisions of the Plan. Options and restricted stock awards granted by the Company generally vest ratably over four years, with a one-year cliff, and options are exercisable from the date of grant for a period of ten years. Restricted stock issuances and early exercises of stock options are subject to a Company right of repurchase at the original issuance price, which right lapses over the vesting period of the stock. For options and restricted stock awards granted through March 31, 2015, the exercise price or purchase price, as applicable, equaled the estimated fair value of the Common Stock as determined by the Company's board of directors on the date of grant.

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

	Number of Stock Options	Weighted-Average Exercise Price	Weighted-Average Remaining Contractual Term (in years)	Aggregate Instrinsic Value (in thousands)
Outstanding at December 31, 2013	846,885	$ 1.97	8.05	$ 4,099
Granted	549,281	6.89		
Exercised	(99,856)	1.18		
Forfeited	(70,170)	3.65		
Outstanding at December 31, 2014	1,226,140	$ 4.14	8.15	6,579
Granted	210,898	10.04		
Exercised	(25,045)	2.05		
Forfeited	(23,775)	3.82		
Outstanding at March 31, 2015	1,388,218	$ 5.08	8.22	$ 8,285
Exercisable at December 31, 2014	533,239	$ 2.24	7.11	$ 3,877
Vested and expected to vest at December 31, 2014	1,129,139	$ 3.97	8.07	$ 6,249
Exercisable at March 31, 2015	643,674	$ 2.95	7.19	$ 5,211
Vested and expected to vest at March 31, 2015	1,277,216	$ 4.87	8.13	$ 7,898

The total intrinsic value of options exercised for the year ended December 31, 2013 and 2014 and for the three months ended March 31, 2014 and 2015 was $0.1 million, $0.6 million, $21,000, and $0.2 million, respectively. The total fair value of employee options vested for the year ended

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

12. Stock Incentive Plan (Continued)

December 31, 2013 and 2014 and for the three months ended March 31, 2014 and 2015 was $0.3 million, $0.5 million, $0.1 million and $0.6 million, respectively.

At December 31, 2014 and March 31, 2015, the total unrecognized compensation expense related to unvested stock option awards, including estimated forfeitures, was $2.3 million and $3.4 million, respectively. The Company expects to recognize that cost over a weighted-average period of approximately 3.0 years and 3.1 years, respectively.

Stock-Based Compensation Expense

The weighted-average grant date fair value of options granted to employees and directors for the years ended December 31, 2013 and 2014, and for the three months ended March 31, 2014 and 2015 was $1.66, $4.94, $4.87, and $6.93, respectively. The fair value of stock options granted to employees and non-employees was estimated using the Black-Scholes option-pricing model based on the assumptions noted in the following table:

	Year Ended December 31,		Three Months Ended March 31,	
	2013	2014	2014	2015
Risk-free interest rate	0.92 - 2.03%	1.71 - 3.01%	1.96 - 3.01%	1.54 - 2.11%
Expected dividend yield	—	—	—	—
Expected term (in years)	6.25 - 10.0	6.25 - 10.0	6.25 - 10.0	6.25 - 10.0
Expected volatility	75.0 - 81.5%	75.2 - 83.4%	75.2 - 82.8%	76.5 - 84.0%

Volatility

Since the Company is privately held as of the date of these financial statements, it does not have relevant historical data to support its expected volatility. As such, the Company has used a weighted- average of expected volatility based on the volatilities of a representative group of publicly traded biopharmaceutical companies. For purposes of identifying representative companies, the Company considered characteristics such as number of product candidates in early stages of product development, area of therapeutic focus, length of trading history, similar vesting provisions and a similar percentage of stock options that were in-the-money. The expected volatility was determined using a weighted-average of the historical volatilities of the representative group of companies for a period equal to the expected term of the option grant. The Company intends to continue to consistently apply this process using the same representative companies until a sufficient amount of historical information regarding the volatility of the Company's own share price becomes available or until circumstances change, such that the identified entities are no longer representative companies. In the latter case, more suitable, similar entities whose share prices are publicly available would be utilized in the calculation.

Risk-Free Rate

The risk-free rate was based on the yield curve of U.S. Treasury securities with periods commensurate with the expected term of the options being valued.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

12. Stock Incentive Plan (Continued)

Expected Term

The Company uses the "simplified method" to estimate the expected term of stock option grants. Under this approach, the weighted-average expected life is presumed to be the average of the contractual term (ten years) and the vesting term (generally four years) of the Company's stock options, taking into consideration multiple vesting tranches. The Company utilizes this method due to lack of historical exercise data and the plain-vanilla nature of the Company's share-based awards.

Dividends

The Company has never paid, and does not anticipate paying in the foreseeable future, any cash dividends and therefore uses an expected dividend yield of zero in the option-pricing model.

Forfeitures

The Company is also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from its estimates. The Company uses historical data to estimate pre-vesting option forfeitures and record stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from the Company's estimates, the difference is recorded as a cumulative adjustment in the period the estimates are revised.

13. Income Taxes

A reconciliation of the U.S. statutory income tax rate to the Company's effective tax rate is as follows for the years ended December 31, 2013 and 2014:

	Year Ended December 31,	
	2013	2014
Federal income tax (benefit) at statutory rate	34.00%	34.00%
Permanent differences	(0.50)	(1.29)
Federal research and development credits and adjustments	5.80	2.70
State income tax, net of federal benefit	5.88	6.03
Other	(0.01)	(0.15)
Change in valuation allowance	(45.17)	(41.29)
Effective income tax rate	0.00%	0.00%

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

13. Income Taxes (Continued)

The provision (benefit) for income taxes was as follows for the years ended December 31, 2013 and 2014 (in thousands):

	Year Ended December 31,	
	2013	2014
Current:		
Federal	$ —	$ —
State	—	—
Total current	—	—
Deferred:		
Federal	—	—
State	—	—
Total deferred	—	—
Total	$ —	$ —

The Company's deferred tax assets consisted of the following (in thousands):

	December 31,	
	2013	2014
Deferred tax assets		
Net operating loss carryforwards	$ 10,982	$ 22,577
Tax credit carryforwards	1,955	2,748
Capitalized research and development	8,521	4,780
Capitalized legal expenses	883	1,223
Other differences	145	195
Total deferred tax assets	22,486	31,523
Valuation allowance	(22,486)	(31,523)
Net deferred tax assets	$ —	$ —

The Company recorded an increase to the valuation allowance of $8.2 million during the year ended December 31, 2013 and $9.0 million during the year ended December 31, 2014, due primarily to an increase in the net operating loss carryforwards and tax credits.

In assessing the realizability of deferred tax assets, the Company considers whether it is more likely than not that some portion or all of the deferred tax assets will not be realized. The ultimate realization of deferred tax assets is dependent upon the generation of future taxable income during the periods in which the temporary differences representing net future deductible amounts become deductible. Due to the Company's history of losses, the deferred tax assets were fully offset by a valuation allowance at December 31, 2013 and 2014.

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

13. Income Taxes (Continued)

As of December 31, 2014, the Company had approximately $57.6 million of federal and $56.5 million of state net operating loss carryforwards to offset future taxable income, if any. Such net operating loss carryforwards expire at varying times through the year 2034, if not utilized. The Company also had approximately $2.1 million of federal and $1.0 million of state tax credit carryforwards available to reduce future tax liabilities as of December 31, 2014.

The Internal Revenue Code of 1986, as amended (the "Code"), provides for a limitation of the annual use of net operating losses and other tax attributes (such as research and development tax credit carryforwards) following certain ownership changes (as defined by the Code) that could limit the Company's ability to utilize these carryforwards. At this time, the Company has not completed a study to assess whether an ownership change under Section 382 of the Code has occurred, or whether there have been multiple ownership changes since the Company's formation, due to the costs and complexities associated with such a study. The Company may have experienced various ownership changes, as defined by the Code, as a result of past financing transactions. Accordingly, the Company's ability to utilize the aforementioned carryforwards may be limited. Additionally, U.S. tax laws limit the time during which these carryforwards may be applied against future taxes. Therefore, the Company may not be able to take full advantage of these carryforwards for federal or state income tax purposes.

The Company does not have any significant unrecognized tax benefits.

As of December 31, 2014, the Company had not accrued interest or penalties related to uncertain tax positions. The Company's tax returns for the years ended December 31, 2008 through December 31, 2014 are still subject to examination by major tax jurisdictions.

14. Defined Contribution Benefit Plan

The Company sponsors a 401(k) retirement plan, in which substantially all of its full-time employees are eligible to participate. Participants may contribute a percentage of their annual compensation to this plan, subject to statutory limitations. The Company did not provide any contributions to this plan during the years ended December 31, 2013 and 2014 or the three months ended March 31, 2014 and 2015.

15. Subsequent Events

The Company has completed an evaluation of all subsequent events through the date this Amendment No. 2 to the registration statement on Form S-1 was filed with the SEC, to ensure that this filing includes appropriate disclosure of events both recognized in the financial statements as of March 31, 2015, and events which occurred subsequently but were not recognized in the financial statements. In connection with preparing for its initial public offering, the Company's board of directors and stockholders approved amendments to the Company's certificate of incorporation. These amendments became effective on June 11, 2015. Pursuant to these amendments:

- upon the closing of a firm commitment underwritten public offering in which the aggregate proceeds raised in the offering equal or exceed $50 million, the Company's Preferred Stock shall be automatically converted into common stock at the applicable conversion price;

Catabasis Pharmaceuticals, Inc.

Notes to Financial Statements (Continued)

Information as of March 31, 2015 and for the three months ended March 31, 2014 and 2015 is unaudited

15. Subsequent Events (Continued)

- a 1-for-12.85 reverse stock split of the Company's common stock was effected; and
- the authorized number of shares of common stock was increased to 200,000,000.

All share and per share amounts in the financial statements and notes thereto have been retroactively adjusted for all periods presented to give effect to the reverse stock split, including reclassifying an amount equal to the reduction in par value of common stock to additional paid-in capital.

The Company's board of directors adopted and the Company's stockholders approved the 2015 stock incentive plan ("2015 Plan"), which will become effective immediately prior to the effectiveness of the Company's initial public offering. 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 Company's board of directors adopted and the Company's stockholders approved the 2015 employee stock purchase plan, which will become effective upon the closing of the Company's IPO.

5,000,000 Shares

Catabasis Pharmaceuticals, Inc.

Common Stock

PROSPECTUS
June 24, 2015

Citigroup

Cowen and Company

Oppenheimer & Co.

Wedbush PacGrow

Through and including July 19, 2015 (25 days after the commencement of this offering), all dealers that buy, sell or trade shares of our common stock, whether or not participating in this offering, may be required to deliver a prospectus. This delivery requirement is in addition to the dealers' obligation to deliver a prospectus when acting as underwriters and with respect to their unsold allotments or subscriptions.