CATABASIS PHARMACEUTICALS INC
Form 10-K
March 15, 2016

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2015

or

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

For the transition period from to
Commission File Number: 001-37467

Catabasis Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**26-3687168**
(State or other jurisdiction of incorporation or organization)	(IRS Employer Identification No.)
One Kendall Square Bldg. 1400E, Suite B14202 Cambridge, Massachusetts	**02139**
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code **(617) 349-1971**

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	**NASDAQ Global Market**

Securities registered pursuant to Section 12(g) of the Act: **None**

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act. o Yes ý No

Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. o Yes ý No

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

Indicate by check mark whether the registrant has submitted electronically and posted on its corporate Web site, if any, every Interactive Data File required to be submitted and posted pursuant to Rule 405 of Regulation S-T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit and post such files). Yes ý No o

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10-K or any amendment to this Form 10-K. ý

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

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

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

EXPLANATORY NOTE: Under the Jumpstart Our Business Startups Act, the registrant qualifies as an "emerging growth company." We therefore incorporate the scaled disclosures required of an emerging growth company in this Annual Report on Form 10-K.

Aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant, based on the last sale price for such stock on June 30, 2015: $69,104,534.

As of March 7, 2016, there were 15,336,333 shares of the registrant's Common Stock, par value $0.001 per share, outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive proxy statement relating to its 2016 Annual Meeting of Stockholders are incorporated by reference into Part III of this Annual Report on Form 10-K where indicated. The registrant intends to file such proxy statement with the U.S. Securities and Exchange Commission within 120 days after the end of the fiscal year to which this Annual Report on Form 10-K relates.

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SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS AND INDUSTRY DATA

This Annual Report on Form 10-K contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, contained in this Annual Report on Form 10-K, 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 therapeutics based on our SMART linker drug discovery platform;
- our plans to initiate Part B of our MoveDMD[SM] clinical trial of CAT-1004 for the treatment of Duchenne muscular dystrophy in the first half of 2016;
- ongoing and planned clinical trials for CAT-1004, CAT-2054 and other product candidates, whether conducted by us or by any future collaborators, including the timing of initiation of these trials and of the anticipated results;
- our plans to enter into collaborations for the development and commercialization of product candidates;
- the potential benefits of any future collaboration;
- our ability to receive research and development funding and achieve anticipated milestones under our collaborations;
- the timing of and our ability to obtain and maintain regulatory approvals for our product candidates;
- the rate and degree of market acceptance and clinical utility of any products for which we receive marketing approval;
- our commercialization, marketing and manufacturing capabilities and strategy;
- our intellectual property position and strategy;
- our ability to identify additional products or product candidates with significant commercial potential;
- our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- developments relating to our competitors and our industry; and
- the impact of government laws and regulations.

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

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You should read this Annual Report on Form 10-K 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 statement, whether as a result of new information, future events or otherwise, except as required by law.

REFERENCES TO CATABASIS

Except as otherwise indicated herein or as the context otherwise requires, references in this Annual Report on Form 10-K to "Catabasis," "the company," "we," "us," and "our" refer to Catabasis Pharmaceuticals, Inc.

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PART I

Item 1. 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 drug discovery platform. Our SMART linker drug discovery platform enables us to engineer product candidates that can simultaneously modulate multiple targets in a disease. Our proprietary product candidates impact pathways that are central to diseases where efficacy may be optimized by a multiple target approach. Our primary focus is on treatments for rare diseases. We are also developing other product candidates for the treatment of serious lipid disorders. We have applied our SMART linker drug discovery platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates.

CAT-1004 is an oral small molecule that we believe has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy, or DMD, regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. 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 inhibit NF-κB, or nuclear factor kappa-light-chain-enhancer of activated B cells, a protein that is activated in DMD and drives inflammation, fibrosis and muscle degeneration, and suppresses muscle regeneration. In animal models of DMD, CAT-1004 inhibited NF-κB activity, reduced muscle degeneration and improved muscle regeneration and function. Beneficial effects were observed in skeletal, diaphragm and cardiac muscle. In Phase 1 clinical trials in adults, CAT-1004 inhibited NF-κB and was well tolerated with no observed safety concerns. The United States Food and Drug Administration, or FDA, has granted orphan drug, fast track and rare pediatric disease designations to CAT-1004 for the treatment of DMD. The European Commission, or EC, also has granted orphan medicinal product designation to CAT-1004 for the treatment of DMD.

We are currently conducting the MoveDMD Phase 1/2 trial of CAT-1004 in boys with DMD between ages four and seven. We reported positive top-line results from Part A of the MoveDMD trial in January 2016. Top-line results indicated that all three doses of CAT-1004 studied were generally well tolerated with no safety signals observed. Top-line pharmacokinetic results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-κB was observed. Subject to regulatory approval of our proposed protocol, we expect to initiate Part B of the MoveDMD trial in the first half of 2016 and to report top-line Part B data in late 2016, contingent on patient enrollment. If the results from our MoveDMD 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. If the results from the Phase 3 clinical trial are positive, we intend to seek marketing approval for CAT-1004. We hold rights to CAT-1004 throughout the world.

Our CAT-2000 series is our other clinical-stage program. We applied our SMART linker drug discovery platform to engineer the CAT-2000 series product candidates to inhibit the Sterol Regulatory Element Binding Protein, or SREBP, pathway. We used different SMART linkers to produce two CAT-2000 series product candidates, CAT-2054 and CAT-2003. These product candidates possess different pharmacokinetic and biodistribution characteristics. CAT-2003, our first generation product candidate, is an orally administered molecule that inhibits the SREBP pathway predominately in the intestine. CAT-2054, our second generation product candidate, is an orally administered molecule that inhibits the SREBP pathway predominately in the liver. We are developing CAT-2054 for serious lipid disorders such as hypercholesterolemia.

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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; and Niemann-Pick C1-like 1, or NPC1L1. In a clinical trial of CAT-2003, we observed statistically significant reductions in triglycerides and low-density lipoprotein cholesterol, or 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. By inhibiting SREBP, a master regulator of lipid metabolism in the body, CAT-2054 has the potential to significantly reduce LDL-C; it may also have beneficial effects on other metabolic parameters such as triglycerides, glucose and liver fat. This profile may differentiate CAT-2054 from currently approved therapies for hypercholesterolemia and others in development. We are developing CAT-2054 to be used in addition to statins in patients who cannot achieve their LDL-C goals with statins alone. In August 2015, we announced positive top-line Phase 1 clinical trial data for CAT-2054. Based on these data, we initiated a Phase 2a trial in patients with hypercholesterolemia in December 2015, which is ongoing. We anticipate that we will report top-line data from the Phase 2a trial in the third quarter of 2016. Additionally, we are currently conducting studies and have generated positive data in preclinical models that support the therapeutic potential of the CAT-2000 series in Nonalcoholic Steatohepatitis, or NASH. We hold rights to CAT-2054 throughout the world, and we intend to seek a partner for the program prior to initiating Phase 3 clinical trials.

CAT-4001 is a SMART linker conjugate of monomethyl fumarate and DHA. CAT-4001 is a small molecule that activates Nrf2 and inhibits NF-κB that we are developing as a potential treatment for neurodegenerative diseases such as Friedreich's ataxia and amyotrophic lateral sclerosis, or ALS. 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. The Nrf2 and NF-κB pathways have been implicated in Friedreich's ataxia and ALS. We plan to conduct investigational new drug application, or IND, enabling studies in 2016 for CAT-4001. We hold rights to CAT-4001 throughout the world.

As of December 31, 2015, we owned four issued U.S. patents relating to composition of matter and method of use claims directed to CAT-1004, two issued U.S. patents relating to composition of matter and method of use claims directed to the CAT-2000 series, and one issued U.S. patent relating to composition of matter and method of use claims direct to CAT-4001. These patents are expected to expire between 2029 and 2031, without taking into account potential patent term extensions. In addition, our patent portfolio includes over 20 issued foreign patents, over 25 pending U.S. patent applications and over 100 pending foreign patent applications.

Our Scientific Approach

Our SMART linker drug discovery platform enables us to engineer product candidates that can simultaneously modulate multiple biological targets in a disease. Our proprietary product candidates impact pathways that are central to diseases where efficacy may be optimized by a multiple target approach.

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

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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 in a disease. While other technologies exist to conjugate or combine two bioactives, we believe that our SMART linker drug discovery platform provides substantial improvements over previous approaches to bioactive conjugation.

SMART Linker Drug Discovery Platform

We have leveraged our SMART linker drug discovery platform to engineer molecules that can simultaneously modulate multiple biological targets in a disease. Our drug discovery platform includes a broad array of linkers that we use to engineer molecular series. The linkers used in our drug discovery 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. Certain of our 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 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.

To create a conjugate using our SMART linker drug discovery 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 molecule to be carried to biological tissues and, following entry into cells, be cleaved by enzymes resident in the cells to release the bioactives.

We have SMART linker conjugates that 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 can 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 may be 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 diseases of interest within the same cell. In addition, release of the bioactives 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 are developing a pipeline of preclinical assets using our SMART linker drug discovery platform to potentially treat rare diseases including ALS, Friedreich's ataxia, cystic fibrosis and others.

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We believe that our SMART linker drug discovery platform has the potential to:

- enhance activity on diseases 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 within cells.

Our Product Candidates

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

CAT-1004

We believe that CAT-1004 has the potential to be the first disease-modifying oral therapy for the treatment of DMD that both inhibits muscle degeneration and promotes muscle regeneration, regardless of the underlying mutation. CAT-1004 is an orally administered SMART linker conjugate of salicylate and DHA, which we designed to enhance the activity of salicylate and DHA to inhibit 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 same cell. Emerging data suggest that NF-κB drives the loss of skeletal muscle mass in multiple diseases, including muscular dystrophies, atrophy and inflammatory myopathies. Scientific data also suggests that NF-κB is involved in the progression of a number of other rare diseases, and we are currently evaluating certain of these diseases as potential indications for CAT-1004. In December 2014, we submitted an IND to the FDA for CAT-1004 for DMD.

We are currently conducting the MoveDMD trial, a Phase 1/2 clinical trial of CAT-1004 for the treatment of DMD, in two parts. Part A of the MoveDMD trial enrolled ambulatory boys between ages four and seven with a genetically confirmed diagnosis of DMD across a range of

dystrophin mutations.

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The enrolled boys were steroid naive or had not used steroids for at least six months prior to the trial. Part A of the MoveDMD trial was conducted at three sites in the United States and assessed the safety, tolerability and pharmacokinetics of CAT-1004 in patients at three dosing levels following seven days of dosing. We reported top-line results in January 2016 indicating that all three doses of CAT-1004 studied were generally well tolerated with no safety signals observed. The majority of adverse events were mild, and the most common adverse events were gastrointestinal, primarily diarrhea. There were no serious adverse events and no drug discontinuations. Top-line pharmacokinetic results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-κB was observed. Part B of the MoveDMD trial will be a randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of CAT-1004 in DMD over a 12-week period. Subject to regulatory approval of our proposed protocol, we expect to initiate Part B of the MoveDMD trial in the first half of 2016, and to report top-line Part B data in late 2016, contingent on patient enrollment. If the results from our MoveDMD 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. If the results from the Phase 3 clinical trial are positive, we intend to seek marketing approval for CAT-1004.

The FDA has granted CAT-1004 orphan drug, fast track and rare pediatric disease designations for the treatment of DMD. The EC has granted orphan medicinal product designation to CAT-1004 for the treatment of DMD.

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 of DMD patients eventually results in death, generally in their 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,

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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 and increases levels of activated NF-κB 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.

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. Three of the most advanced product candidates, Sarepta Therapeutics' eteplirsen, PTC Therapeutics' ataluren, and BioMarin Pharmaceutical's drisapersen, 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 degeneration and enhance muscle regeneration.

CAT-1004 for the Treatment of Duchenne Muscular Dystrophy

Based on the mechanism of action by which CAT-1004 suppresses 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 an effective therapy 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 utrophin. We intend to commercialize CAT-1004 in North America ourselves and commercialize CAT-1004 outside of North America either ourselves or with a collaborator.

In Phase 1 clinical trials in adults and in Part A of our MoveDMD clinical trial in boys affected by DMD, CAT-1004 was observed to be well tolerated with no safety signals. We expect to initiate Part B

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of the MoveDMD trial in the first half of 2016, subject to regulatory approval of our proposed protocol.

CAT-1004 Clinical Development

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

Our CAT-1004 MoveDMD Phase 1/2 trial was designed to enroll ambulatory boys between ages four and seven with a genetically confirmed diagnosis of DMD who are steroid naive or had not used steroids for at least six months prior to the trial. Boys enrolled in the trial are not limited to any specific dystrophin mutations. The MoveDMD trial was designed to be conducted in two sequential parts, Part A, which is completed, and Part B, which we expect to initiate in the first half of 2016, subject to regulatory approval of our proposed protocol.

In Part A of the MoveDMD trial, which was conducted at three sites in the United States, we assessed the safety, tolerability and pharmacokinetics of CAT-1004 in 17 patients across three dosing levels following seven days of dosing. We also compared CAT-1004 exposure levels to exposure levels achieved in previous CAT-1004 clinical trials where inhibition of NF-κB was observed. In January 2016, we reported that all three doses of CAT-1004 tested were generally well tolerated with no safety signals observed. The majority of adverse events were mild, and the most common adverse events were gastrointestinal, primarily diarrhea. There were no serious adverse events and no drug discontinuations. Pharmacokinetic results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-κB was observed.

Part B of the MoveDMD trial is expected to 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 are expected to 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, 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. We expect that the MoveDMD trial will be conducted at ImagingDMD's clinical sites in the United States.

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 is a non-invasive imaging technique that allows investigators to view muscle structure and composition and measure disease status in children with DMD. MRI is sensitive to the changes in muscle structure and composition induced by disease processes such as inflammation, water accumulation, muscle damage and fat infiltration that occur in DMD. 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. Changes in the inflammatory MRI signal may be seen in less than 12 weeks, while changes in fat infiltration measures may take longer. Changes in these MRI measures have been correlated with longer-term changes in clinically meaningful measures of functional activity. Changes in MRI can show the effects of an investigational therapy on disease progression in DMD in an objective and quantifiable manner.

Both inflammation and fat 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 four-stair climb tests. In addition, assessments of muscle strength and a parent-proxy measure of functional ability will be included.

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Subject to regulatory approval of our proposed protocol, we expect to initiate Part B of the MoveDMD trial in the first half of 2016, and to report top-line Part B data in late 2016, contingent on patient enrollment. If the results from our MoveDMD 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. If the results from the Phase 3 clinical trial are positive, we intend to seek marketing approval for CAT-1004.

Parent Project Muscular Dystrophy and the Muscular Dystrophy Association are collaborating with us on the MoveDMD trial, including providing funding to support participant travel.

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

Trial	Description	Duration	Subjects Total	Subjects 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, with the most commonly reported adverse events 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 adverse events in the fasted state were mild in severity. Of the 35 subjects in the fed state, six reported diarrhea, six reported headache and four reported abdominal pain. The most common adverse events in the fed state were diarrhea, headache and abdominal pain, and all of the adverse events in the fed state were mild in severity. Subjects in the fed state receiving single doses of CAT-1004 of 4000 mg or more reported gastrointestinal adverse events more frequently than subjects receiving lower doses. No treatment-related severe adverse events were reported. There were no observed trends in laboratory,

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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 three- to eight-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 adverse events reported in more than one subject were each reported by two subjects. These adverse events 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 adverse events were mild in severity. No treatment-related severe adverse events 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 adverse events 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

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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 the omega-3 fatty acid eicosapentaenoic acid, or 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.

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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.

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.

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. 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.

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, Fast Track and Rare Pediatric Disease Designations

The FDA has granted CAT-1004 orphan drug, fast track and rare pediatric disease designations for the treatment of DMD. 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. 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. The FDA fast track process is designed to expedite the development and review of drugs to treat serious or life-threatening conditions and demonstrate the potential to address unmet medical needs. Companies that receive fast track designation are allowed to submit New Drug Applications, or NDAs, on a rolling basis, expediting the FDA review process, and benefiting from more frequent communication with the FDA to discuss all aspects of clinical development. In addition, drugs that receive fast track designation are eligible for accelerated approval and priority review if certain criteria are met. The FDA's rare pediatric disease designation gives us the potential to receive a priority review voucher if CAT-1004 is approved. However, the rare pediatric disease program is set to expire in September 2016 under a provision that sunsets the law after the FDA approves the third pediatric review voucher, which occurred in March 2015. There is pending legislation that would extend the program through December 2018.

The EC has granted orphan medicinal product designation to CAT-1004 for the treatment of DMD. Similar to the FDA orphan drug designation, the EC may designate a product as an orphan medicinal product if it is intended for the treatment of a life-threatening or chronically debilitating

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condition affecting not more than five in ten thousand persons. In Europe, marketing authorization for an orphan medicinal product generally leads to up to a ten-year period of market exclusivity if the product candidate is granted marketing authorization in the European Union.

CAT-2000 Series

Our other clinical-stage program is our CAT-2000 series. We applied our SMART linker drug discovery platform to engineer these product candidates as SMART linker conjugates of EPA and nicotinic acid in order to inhibit the SREBP pathway. 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 inhibits 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 inhibit the SREBP pathway predominately in the liver. We are developing CAT-2054 for the treatment of serious lipid disorders, such as hypercholesterolemia. We are currently conducting preclinical studies in collaboration with academic institutions and have also observed positive data in preclinical models that support the therapeutic potential of the CAT-2000 series in NASH.

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, ATP citrate lyase and NPC1L1. 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. Modulators of SREBP activity could have therapeutic benefit in treating these SREBP-mediated diseases.

We designed the CAT-2000 molecules to inhibit the maturation of SREBP and reduce the expression of key proteins involved in LDL-C, triglyceride and glucose metabolism. 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 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, or FASN, and acetyl-CoA carboxylase 2, or ACC-2, enzymes that play a central role in the synthesis of fatty acids and the regulation of 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, and thereby improve insulin resistance 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

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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. Accordingly, we believe that the CAT-2000 series may potentially be effective in the treatment of liver diseases associated with this specific mutation of PNPLA3, such as NASH, and their progression to hepatocellular carcinoma.

CAT-2054

We are developing CAT-2054 for the treatment of patients with hypercholesterolemia, or elevated LDL-C, for whom existing treatments are insufficient. As described above, 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 triglycerides and 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 has the potential for beneficial effects on levels of LDL-C, triglycerides, glucose and liver fat. This profile may differentiate CAT-2054 from currently approved therapies for hypercholesterolemia and others in development. We are developing CAT-2054 to be used in addition to statins in patients who cannot reach their LDL-C goals with statins alone.

We submitted an IND to the FDA for CAT-2054 in November 2014. In August 2015, we announced positive top-line data for CAT-2054 from a Phase 1 clinical trial. In this double-blind, randomized clinical trial, CAT-2054 was well tolerated with no serious adverse events observed in either single or multiple ascending dose arms. In the multiple ascending dose arm of the trial, decreases in median LDL-C levels of up to 20% were observed in healthy volunteers after 14 days of dosing and seven days of follow-up. CAT-2054 was also found to be well tolerated in combination with atorvastatin, the statin drug most commonly used in the treatment of hypercholesterolemia, and there was no evidence for impact of CAT-2054 on the pharmacokinetics of atorvastatin. Based on these data, we initiated a Phase 2a trial in patients with hypercholesterolemia in December 2015, which is ongoing. We anticipate that we will report top-line data from this trial in the third quarter of 2016. We are currently conducting studies, and have also observed positive data in preclinical models, that support the therapeutic potential of the CAT-2000 series in NASH. We hold rights to CAT-2054 throughout the world, and we intend to seek a partner for the program prior to initiating Phase 3 clinical trials.

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®, or rosuvastatin, the largest remaining branded

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prescription statin, generated worldwide sales of $5.0 billion for the 12-month period ended December 2015.

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 $3.8 billion for the 12-month period ended December 2015.

Monoclonal antibodies against PCSK9. Alirocumab, or Praluent , marketed by Sanofi and Regeneron and evolocumab, or Repatha , marketed by Amgen were approved by the FDA and European Medicines Agency, or EMA, in 2015 for the treatment of hypercholesterolemia in the United States and European Union. These drugs are injectable products that target PCSK9, increasing the clearance of LDL-C. Industry analysts project that these agents will achieve combined global sales of $4.4 billion in 2020, based on the ability of these agents to lower LDL-C by more than 50%. Other PCSK9 inhibitors in clinical development include Pfizer's bococizumab, and Alnylam/The Medicine Company's investigational RNAi therapeutic, ALN-PCSsc.

Inhibitors of ATP citrate lyase. In addition to the marketed therapies, Esperion Therapeutics is developing an agent that targets the synthesis of LDL-C through inhibition of ATP citrate lyase. ATP citrate lyase inhibitors target cholesterol synthesis in the liver but at an earlier step of the pathway than statins.

Despite the availability of these classes of drugs that lower LDL-C, many patients are unable to achieve their LDL-C goals using currently 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. However, we believe that Medivation, Inc., which is testing MDV-4463 in a Phase 1 clinical trial, is the only other company with a SREBP inhibitor in 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.

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CAT-2054 for the Treatment of Hypercholesterolemia

CAT-2054 is designed to inhibit SREBP in the liver and to reduce LDL-C levels in patients with hypercholesterolemia. 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, ATP citrate lyase, and NPC1L1. 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 who are intolerant to statins. CAT-2054 has the potential to be used before injectable PCSK9 monoclonal antibodies.

We intend to seek a partner for the CAT-2054 program prior to initiating Phase 3 clinical trials.

CAT-2054 Clinical Development

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

We conducted a randomized, double-blind, placebo-controlled Phase 1 trial in 118 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 also included multiple doses of CAT-2054 with atorvastatin to assess safety and pharmacokinetics of both compounds in combination in preparation for Phase 2 clinical trials. In August 2015, we reported positive top-line data from this trial. CAT-2054 was well tolerated with no serious adverse events observed in either the single or multiple ascending dose arms of the trial. In the multiple ascending dose arm of the trial, decreases in median LDL-C levels of up to 20% were observed in healthy volunteers after 14 days of dosing and seven days of follow-up. Importantly, CAT-2054 was also found to be well tolerated in combination with atorvastatin, the statin drug most commonly used in the treatment of hypercholesterolemia, and there was no evidence for impact of CAT-2054 on the pharmacokinetics of atorvastatin.

In the single ascending dose portion of the Phase 1 clinical trial, 40 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 adverse events were reported. No safety signals were observed in laboratory, vital sign or electrocardiogram results following CAT-2054 administration. The observed adverse events occurring under fed and fasted conditions at doses up to 500 mg were similar for CAT-2054 and placebo. The most common adverse events observed in fed and fasting conditions were nausea and diarrhea and all reported adverse events were mild. Of the 40 subjects, 10 subjects received placebo, two of whom reported diarrhea and one of whom reported nausea. Thirty subjects received CAT-2054, of whom six reported nausea, five reported diarrhea and three 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. Because decreases in free fatty acid levels are generally associated with nicotinic acid, we also measured free fatty acid levels after administration of CAT-2054, and observed no differences in free fatty acid levels relative to placebo. This is consistent with intracellular cleavage of CAT-2054 and intracellular delivery of the component bioactives.

In the 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. The plasma exposure of CAT-2054 was greater than the plasma

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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 molecule.

In the multiple ascending dose portion of the Phase 1 trial, 70 healthy volunteers received CAT-2054 in soft gelatin capsules or placebo at total daily doses ranging from 100 to 750 mg given orally once or twice per day for 14 days. CAT-2054 was also given concurrently with atorvastatin in one cohort. 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. CAT-2054 was well tolerated with no serious adverse events reported. No safety signals were observed in laboratory, vital signs or electrocardiogram results following CAT-2054 administration, and all subjects completed dosing. At the highest doses, the most common adverse events were gastrointestinal, all of which were mild. CAT-2054 was also well tolerated with no safety signals in subjects receiving atorvastatin. There was no evidence of clinically significant changes in atorvastatin pharmacokinetics when co-administered with CAT-2054.

We also measured lipid biomarkers in the healthy volunteers enrolled in the Phase 1 trial. 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%, which were statistically significant compared to baseline for all dose levels, were observed after 14 days of dosing and seven days of follow-up. 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. We also studied a coated capsule formulation of CAT-2054 in eight of the healthy volunteers in this trial. However, we do not plan further development of the coated capsule formulation; the results discussed above refer only to the uncoated formulation.

Phase 2a Clinical Trial

We initiated a randomized, double-blind, placebo-controlled Phase 2a trial of CAT-2054 in patients with hypercholesterolemia at multiple sites in the United States in December 2015. The CAT-2054 Phase 2a trial is a four-week randomized, double-blind, placebo-controlled trial. We plan to enroll approximately 150 patients who, after a run-in period of at least four weeks of receiving 40 mg of atorvastatin per day, will receive either one of four doses of CAT-2054 or placebo, in each case in addition to continuation of the atorvastatin regimen. The four CAT-2054 cohorts will receive the following doses of CAT-2054: 250 mg once daily, 250 mg twice daily, 400 mg once daily and 400 mg twice daily. Patients will be treated for four weeks, with 25 to 30 patients in each arm. The primary efficacy endpoint for this trial will be percent reduction in 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 such as triglycerides and glucose and glycosylated hemoglobin, or HbA1c, which is a measure of glucose levels over time. We anticipate that we will report top-line data from this trial in the third 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. At the end of the treatment period, we observed a statistically significant reduction of 31% in LDL-C levels

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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 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.

In an *in vitro* study, we observed that treatment of a human liver cell line with CAT-2054 reduced the secretion of PCSK9 protein. 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.

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

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. 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.

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. CAT-2054 abrogated the statin-induced increase in PCSK9 secretion.

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 epoxidase.

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 multifactorial chylomicronemia, or MFC, and refractory severe hypertriglyceridemia, or rSHTG, diseases of severe triglyceride elevations with niche patient populations. We submitted an IND to the FDA for CAT-2003 in September 2012. We have completed three Phase 2a trials in patient

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populations with elevated triglyceride levels 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 are conducting exploratory evaluation of CAT-2003 in other serious diseases that involve alterations in the SREBP pathway, such as NASH and hepatocellular carcinoma, either to support our development efforts for CAT-2054 or to develop CAT-2003 as a product candidate.

Effect of CAT-2003 in Hyperlipidemias

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. Given the superior distribution of CAT-2054 to the liver, we have chosen to prioritize the development of CAT-2054 over CAT-2003 for the treatment of hyperlipidemias.

CAT-4001

CAT-4001 is a SMART linker conjugate of monomethyl fumarate and DHA that we designed to combine the potentially beneficial activities of monomethyl fumarate and DHA on the Nrf2 and NF-κB pathways. CAT-4001 is a small molecule designed to activate the Nrf2 pathway and inhibit the NF-κB pathway. 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. 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 control the body's response to cellular stress and oxidative damage. The Nrf2 and NF-κB pathways have been implicated in Friedreich's ataxia and ALS.

We have shown that CAT-4001 modulates the Nrf2 and NF-κB pathways in both cellular assays and animal models. In these studies, we have also observed that the activity produced by CAT-4001 was greater than that produced by the individual bioactives, monomethyl fumarate and DHA, either alone or in combination at approximately equivalent amounts to those contained in the CAT-4001 conjugate. 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 Biogen's Tecfidera (dimethyl fumarate), 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.

Based on its mechanism of action, we believe that CAT-4001 has the potential to be a disease modifying agent in certain neurodegenerative diseases. In 2016, we plan to continue preclinical evaluation of CAT-4001 in animal models of Friedreich's ataxia as well as ALS, and to conduct IND-enabling activities for CAT-4001. If we are successful in these activities, we intend to advance CAT-4001 into a Phase 1 clinical trial in 2017.

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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.

Friedreich's ataxia occurs in both males and females and is estimated to affect 1 in 50,000 individuals. Based on this prevalence rate, we believe there are up to 6,000 patients with Friedreich's ataxia in the US and up to 15,000 Friedreich's ataxia patients in the European Union.

The Friedreich's Ataxia Research Alliance announced in January 2016 that we were the recipient of the Kyle Bryant Translational Research Award. The Kyle Bryant Translational Research Award specifically focuses on pre-clinical and clinical investigations that target treatments for Friedreich's ataxia.

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 occur. 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 intend to commercialize CAT-1004 in North America ourselves and commercialize CAT-1004 outside of North America either ourselves or with a collaborator. We intend to seek a partner for the CAT-2054 program prior to initiating Phase 3 clinical trials. In addition, we intend to expand the drug development applications of our SMART linker drug discovery 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 fine chemicals manufacturers. The other bioactive and linker raw materials that we use are also readily available from established pharmaceutical intermediate manufacturers. The components

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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 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 ataluren (Translarna) in the European Union for DMD patients with nonsense mutations and reported in January 2016 the completion of a rolling NDA submission to the FDA for marketing approval in the United States. In February 2016, PTC received a Refuse to File letter for ataluren from the FDA. BioMarin Pharmaceutical and Sarepta Therapeutics each have product candidates in clinical development based on a different scientific approach, which is referred to as exon-skipping. BioMarin received a complete response letter from the FDA in January 2016 for drisapersen (Kyndrisa). BioMarin has yet to announce next steps for the program. Sarepta is conducting Phase 3 clinical trials of its lead product candidate eteplirsen and has submitted an NDA to the FDA, with a PDUFA date of May 26, 2016. 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 submit filings to the FDA and EMA in 2016 to support regulatory approval of idebenone for the treatment of DMD in the United States and Europe. Eli Lilly conducted 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 delay the loss of ambulatory function in patients with DMD. Eli Lilly reported negative results from this trial in February 2016. A number of companies have products in earlier stages of clinical development for

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DMD, including Akashi Therapeutics, Bristol Myers Squibb, Capricor Therapeutics, Cardero Therapeutics, 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:

Anti-PCSK9 monoclonal antibodies and RNAi therapeutics. The PCSK9 monoclonal antibodies alirocumab (Praluent) marketed by Sanofi and Regeneron and evolocumab (Repatha) marketed by Amgen were approved by the FDA and the EMA in 2015 for the treatment of hypercholesterolemia in the United States and the European Union. PCSK9 inhibitors represent the first major new class of LDL-C reducing agents approved for the treatment of hyperlipidemia since statins. These agents are highly efficacious and well tolerated but are injectable and priced at a premium to current branded oral agents in this category. Industry analysts project that Praluent and Repatha will achieve combined global sales of $4.4 billion in 2020, based on the ability of these agents to lower LDL-C by more than 50%.

Cholesterol ester transfer protein (CETP) inhibitors. CETP inhibitors are intended to reduce the risk of atherosclerosis by both raising high-density lipoprotein cholesterol and reducing LDL-C. Multiple CETP inhibitor programs have been terminated due to safety (Pfizer's torcetrapib) or lack of efficacy (Roche's dalcetrapib and Eli Lilly's evecetrapib). Merck's anacetrapib (MK-0859) is being studied in a Phase 3 outcomes trial expected to be completed in 2017. Amgen/Dezima's CETP inhibitor TA-8995 has completed a Phase 2b clinical trial.

Other mechanisms. Esperion Therapeutics is developing bempedoic acid, or ETC-1002, an inhibitor of ATP citrate lyase that is currently in Phase 3 clinical trials for the treatment of hypercholesterolemia. Madrigal Pharmaceuticals is developing MGL-3196, an inhibitor of thyroid hormone receptors that has completed Phase 1 clinical trials in healthy volunteers.

Other SREBP inhibitors. In October 2015, Medivation, Inc. announced initiation of a Phase 1 clinical trial in normal healthy volunteers with its oral SREBP inhibitor, MDV-4463.

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 drug discovery 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

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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 protected or remain protectable 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 December 31, 2015, our patent estate included 14 issued U.S. patents, over 20 issued foreign patents, over 25 pending U.S. patent applications, and over 100 pending foreign patent applications.

With regard to CAT-1004, we have four issued U.S. patents with composition of matter and method of use claims directed to CAT-1004 and its use. The issued U.S. patents 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 nine different countries including Australia, China, Japan, 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 eight different countries including Australia, Mexico, China, Japan 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 covering 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 and one allowed U.S. patent application 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. In addition, we have patents that have been granted in five different countries including Japan, New Zealand and Taiwan, which are expected to expire in 2031, 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 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

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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;

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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.

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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 $2.3 million, and the sponsor of an approved NDA is also subject to annual product and establishment user fees, currently exceeding $114,000 per product and $585,000 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

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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

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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. There is limited experience with accelerated approvals by the FDA based on intermediate clinical endpoints. However, the FDA 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.

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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.

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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..."

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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

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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

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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.

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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 EC 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.

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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

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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

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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.

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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 December 31, 2015, we had 37 employees, 23 of whom were primarily engaged in research and development activities. A total of 16 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.

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,

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our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

Available Information

Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and any amendments to these reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, are available free of charge on our website located at www.catabasis.com as soon as reasonably practicable after they are filed with or furnished to the Securities and Exchange Commission (the "SEC"). These reports are also available at the SEC's Internet website at www.sec.gov. The public may also read and copy any materials filed with the SEC at the SEC's Public Reference Room at 100 F Street, N.E., Washington D.C. 20549. Information on the operation of the Public Reference Room may be obtained by calling the SEC at 1-800-SEC-0330.

A copy of our Corporate Governance Guidelines, Code of Business Conduct and Ethics and the charters of the Audit Committee, Compensation Committee and Nominating and Corporate Governance Committee are posted on our website, www.catabasis.com, under "Corporate Governance" and are available in print to any person who requests copies by contacting us by calling (617) 349-1971 or by writing to Catabasis Pharmaceuticals, Inc., One Kendall Square, Bldg. 1400E, Suite B14202, Cambridge, Massachusetts 02139.

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Item 1A. Risk Factors

We operate in a dynamic and rapidly changing business environment that involves risks and substantial uncertainty. The following discussion addresses risks and uncertainties that could cause, or contribute to causing, actual results to differ from expectations in material ways. In evaluating our business, investors should pay particular attention to the risks and uncertainties described below and in other sections of this Annual Report on Form 10-K and in our subsequent filings with the Securities and Exchange Commission, or SEC. These risks and uncertainties, or other events that we do not currently anticipate or that we currently deem immaterial also may affect our results of operations, cash flows and financial condition. The trading price of our common stock could also decline due to any of these risks, and you could lose all or part of your investment.

Risks Related to Our Financial Position and Need for Additional Capital

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

We have incurred significant annual net operating losses in every year since our inception. We expect to continue to incur significant and increasing operating losses for at least the next several years. Our net losses were $32.6 million, $21.9 million and $18.1 million for the years ended December 31, 2015, 2014 and 2013, respectively. As of December 31, 2015, we had an accumulated deficit of $108.0 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 our initial public offering of common stock, private placements of our preferred stock and debt financing, and have devoted substantially all of our financial resources and efforts to research and development, including preclinical studies and our clinical development programs. Our net losses may fluctuate significantly from quarter to quarter and year to year. Net losses and negative cash flows have had, and will continue to have, an adverse effect on our stockholders' equity (deficit) and working capital.

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

- continue to develop and conduct clinical trials with respect to our product candidates CAT-1004 and CAT-2054, including an ongoing Phase 1/2 clinical trial of CAT-1004 for the treatment of Duchenne muscular dystrophy, or DMD, for which we initiated patient enrollment in June 2015, and a Phase 2a clinical trial of CAT-2054 for the treatment of hypercholesterolemia for which we initiated patient dosing in December 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;

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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, we have incurred and will continue to incur significant additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on attractive terms, we may be forced to delay, reduce or eliminate our research and development programs or any future commercialization efforts.

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

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

We believe that our existing cash and cash equivalents as of December 31, 2015 will enable us to fund our operating expenses, debt service and capital expenditure requirements through the first quarter of 2017. Our estimate as to how long we expect our cash and cash equivalents to be able to fund our operations is based on assumptions that may prove to be wrong, and we could use our available capital resources sooner than we currently expect. Further, changing circumstances, some of which may be beyond our control, could cause us to consume capital significantly faster than we currently anticipate, and we may need to seek additional funds sooner than planned. Our future funding requirements, both short-term and long-term, will depend on many factors, including:

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

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

the costs of operating as a public company.

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

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

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

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

As of December 31, 2015, we had $8.9 million of outstanding borrowings under our credit facility with MidCap, Flexpoint and Square 1. We are required to repay principal and interest on these borrowings in monthly installments through October 2018. Subject to the restrictions in this existing credit facility, we could in the future incur additional indebtedness beyond our borrowings from MidCap, Flexpoint and Square 1.

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

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

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

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

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

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

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

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

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

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

We are dependent on the success of our product 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 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;

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- 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, myostatin and inflammatory mediators.

Many of these factors are beyond our control, including the outcome of clinical development, the regulatory submission process, potential threats to our intellectual property rights and the manufacturing, marketing and sales efforts of any future collaborator. If we are unable to develop, receive marketing approval for and successfully commercialize at least one of CAT-1004 or CAT-2054, on our own or with any future collaborator, or experience delays as a result of any of these or other factors, our business could be substantially harmed.

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

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

- compounds created through our SMART linker drug discovery platform may not demonstrate improved efficacy, safety or tolerability;
- potential product candidates may, on further study, be shown to have harmful side effects or other characteristics that indicate that they are unlikely to receive marketing approval and achieve market acceptance;

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

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

Our research programs to identify new product candidates will require substantial technical, financial and human resources, and we may be unsuccessful in our efforts to identify new product

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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.

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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 have not issued definitive guidance as to how to measure and demonstrate efficacy. We anticipate that the primary endpoint in our MoveDMD 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, as well as other strength and functional measures, including the North Star ambulatory assessment questionnaire and the pediatric outcome data collection instrument. 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 twelve week period of the trial.

The regulatory approval process for product candidates that target rare diseases, including DMD, Friedreich's Ataxia, and ALS, is uncertain.

Due to the lack of precedent, broad discretion of regulatory authorities, and a multitude of unique factors that impact the regulatory approval process, the likelihood of the approval of any of our product candidates that target rare diseases is uncertain, and we may not be able to anticipate, prepare for or satisfy requests or requirements from regulatory authorities, including completing and submitting planned INDs and NDAs for our product candidates, in a timely manner, or at all. For example, DMD is a rare disease for which there is currently no FDA approved therapeutic. Further, the FDA may determine, after evaluation of our data and analyses, that such data and analyses do not support an NDA submission, filing or approval. Due to this lack of predictability, we may not have the resources necessary to meet regulatory requirements and successfully complete a potentially protracted, expensive and wide-ranging approval process for commercialization of product candidates for rare diseases.

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

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

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

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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. For example, having completed Part A of our MoveDMD Phase 1/2 clinical trial, we currently intend to start Part B of the trial in the first half of 2016. However, the protocol for Part B of the trial is subject to prior FDA and Institutional Review Board approval, which may interfere with our anticipated timing. Further, 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 drug discovery platform may be particularly susceptible to failure to the extent they are based on compounds that others have previously studied or tested, but did not progress in development due to safety, tolerability or efficacy concerns or otherwise. Our failure to successfully complete clinical trials of our product candidates and to demonstrate the efficacy and safety necessary to obtain regulatory approval to market any of our product candidates would significantly harm our business.

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

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

Clinical testing is expensive, difficult to design and implement, can take many years to complete and is inherently uncertain as to outcome. We have not previously submitted an NDA to the FDA or similar drug approval filings to comparable foreign regulatory authorities for any of our product candidates. Any inability to complete preclinical and clinical development successfully could result in additional costs to us, or any future collaborators, and impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties. Moreover, if (1) we, or any

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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;

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- 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

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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 Part B of our Phase 1/2 clinical trial of CAT-1004 for which we expect to initiate patient enrollment in the first half of 2016, we intend 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

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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

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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.

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

We may collaborate with third parties for commercialization of any products that require a large sales, marketing and product distribution infrastructure. We intend to commercialize CAT-2054 and potentially other product candidates through collaboration, licensing and distribution arrangements with third parties. As a result of entering into arrangements with third parties to perform sales, marketing and distribution services, our product revenues or the profitability of these product revenues may be lower, perhaps substantially lower, than if we were to directly market and sell products in those markets. Furthermore, we may be unsuccessful in entering into the necessary arrangements with third parties or may be unable to do so on terms that are favorable to us. In addition, we may have little or no control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively.

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

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

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

We are initially developing CAT-2054 for the treatment of hypercholesterolemia. There are many widely available products, including statins, cholesterol absorption inhibitors and PCSK9 monoclonal antibodies, approved for the treatment of patients with hypercholesterolemia. The market and development pipeline for cholesterol regulating therapies is especially large and competitive. If

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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 cholesterol ester transfer protein inhibitors, including those being developed by Amgen and Merck; and other alternative therapies being developed by a range of competitors, including Esperion. We are also potentially developing CAT-2054 for the treatment of Nonalcoholic Steatohepatitis, or NASH. If CAT-2054 is approved for the treatment of NASH, it similarly will face strong competition from other therapeutic approaches, including those currently in development by a number of competitors already in clinical trials, among them, Intercept Pharmaceuticals, Inc., Genfit SA, Galmed Pharmaceuticals, Ltd. and Gilead Sciences, Inc.

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

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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

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profitably. These payors may not view our products, if any, as cost-effective, and coverage and reimbursement may not be available to our customers, or those of any future collaborators, or may not be sufficient to allow our products, if any, to be marketed on a competitive basis. Cost-control initiatives could cause us, or any future collaborators, to decrease the price we, or they, might establish for products, which could result in lower than anticipated product revenues. If the prices for our products, if any, decrease or if governmental and other third-party payors do not provide coverage or adequate reimbursement, our prospects for revenue and profitability will suffer.

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

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

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

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

decreased demand for our product candidates or products that we may develop;

injury to our reputation and significant negative media attention;

withdrawal of clinical trial participants;

significant costs to defend resulting litigation;

substantial monetary awards to trial participants or patients;

loss of revenue;

reduced resources of our management to pursue our business strategy; and

the inability to commercialize any products that we may develop.

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Although we maintain general liability insurance of $5.0 million in the aggregate and clinical trial liability insurance of $10.0 million in the aggregate, this insurance may not fully cover potential liabilities that we may incur. The cost of any product liability litigation or other proceeding, even if resolved in our favor, could be substantial. We will need to increase our insurance coverage if and when we begin selling any product candidate that receives marketing approval. In addition, insurance coverage is becoming increasingly expensive. If we are unable to obtain or maintain sufficient insurance coverage at an acceptable cost or to otherwise protect against potential product liability claims, it could prevent or inhibit the development and commercial production and sale of our product candidates, which 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.

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

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

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

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

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

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

- collaborators have significant discretion in determining the efforts and resources that they will apply to these collaborations;
- collaborators may not perform their obligations as expected;
- collaborators may not pursue development and commercialization of our product candidates or may elect not to continue or renew development or commercialization programs, based on clinical trial results, changes in the collaborators' strategic focus or available funding or external factors, such as an acquisition, that divert resources or create competing priorities;
- 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.

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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.

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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.

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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

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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 drug discovery 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

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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 drug discovery platform without infringing the intellectual property and other proprietary rights of third parties. Third parties have U.S. and non-U.S.

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issued patents and pending patent applications relating to compounds and methods of use for the treatment of DMD and hypercholesterolemia, the key indications for our priority programs. If any third-party patents or patent applications are found to cover our product candidates or their methods of use, we may not be free to manufacture or market our product candidates as planned without obtaining a license, which may not be available on commercially reasonable terms, or at all.

There is a substantial amount of intellectual property litigation in the biotechnology and pharmaceutical industries, and we may become party to, or threatened with, litigation or other adversarial proceedings regarding intellectual property rights with respect to our product candidates, including interference proceedings before the USPTO. 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

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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 noncompliance 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. Noncompliance 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 or where any future patent claims or other intellectual property rights may not be effective or sufficient to prevent them from competing with us.

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

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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, 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 members 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.

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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

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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 and orphan medicinal product designation from the European Commission (EC) 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 FDA has taken the position that, under certain circumstances, another drug with the same active moiety can be approved for the same condition. Specifically, the FDA's regulations provide that it can approve another drug with the same active moiety 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

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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.

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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;

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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;

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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

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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.

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

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

A rare pediatric disease designation may not lead to the receipt of a Priority Review Voucher, even if CAT-1004 is approved, due to the potential expiration of the FDA's Rare Pediatric Disease program.

The FDA has awarded rare pediatric disease Priority Review Vouchers to sponsors of drug candidates to treat rare pediatric disease products, if the treatment sponsors apply for this designation and meet certain criteria. Under this program, upon the approval of a qualifying NDA or biologics license application, or BLA, for the treatment of a rare pediatric disease, the sponsor of such an application would be eligible for a rare pediatric disease Priority Review Voucher that can be used to obtain priority review for a subsequent NDA or BLA. The Priority Review Voucher may be sold or transferred an unlimited number of times. In September 2015, the FDA notified us that we obtained rare pediatric disease designation for CAT-1004 for the treatment of DMD. The FDA's rare pediatric disease designation gives us the potential to receive a Priority Review Voucher if CAT-1004 is approved. However, the rare pediatric disease Priority Review Voucher program is set to expire in September 2016 under a provision that sunsets the law after the FDA approves the third pediatric review voucher, which was in March 2015, so we may not receive a Priority Review Voucher even if CAT-1004 is approved. Legislation is pending that could extend the Priority Review Voucher program through

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December 2018. There is no guarantee that this legislation will pass or that we will be in a position to obtain the Priority Review Voucher prior to expiration of the program, even if the legislation passes.

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.

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Risks Related to Our Common Stock

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

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

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

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

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

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

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

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and may remain an emerging growth company for up to five years. For so long as we remain an emerging growth company, we are permitted and plan to rely on exemptions from certain disclosure requirements that are applicable to other public companies that are not emerging growth companies. These exemptions include not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, or SOX Section 404, not being required to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board regarding mandatory audit firm rotation or a supplement to the auditor's report providing additional information about the audit and the financial statements, reduced disclosure obligations regarding executive compensation and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. Investors may find our common stock less attractive as a result of our reliance 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.

We have incurred and will continue to 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 have incurred and will continue to incur significant legal, accounting and other expenses that we did not incur as a private company. The Sarbanes-Oxley Act of 2002, the Dodd-Frank Wall Street Reform and Consumer Protection Act, the listing requirements of The NASDAQ Global Market and other applicable securities rules and regulations impose various requirements on public companies, including establishment and maintenance of effective disclosure and financial controls and corporate governance practices. We expect that we will need to hire additional accounting, finance and other personnel in connection with our efforts to comply with the requirements of being a public company and our management and other personnel will need to devote a substantial amount of time towards maintaining compliance with these requirements. These requirements will increase our legal and financial compliance costs and will make some activities more time-consuming and costly. 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 SEC. 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

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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 may be sold into the market in the near future, which could cause the market price of our common stock to decline significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock. As of December 31, 2015, we have outstanding 15,313,297 shares of common stock. Moreover, holders of an aggregate of 8,724,556 shares of our common stock, along with the holders of warrants to purchase 24,556 shares of common stock, 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 have filed a registration statement registering all shares of common stock that we may issue under our equity compensation plans. As of December 31, 2015, we had outstanding options to purchase an aggregate of approximately 1,723,554 shares of our common stock, of which options to purchase approximately 795,501 shares were vested. These shares can be freely sold in the public market upon issuance and once vested, subject to volume limitations applicable to affiliates.

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

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

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

Our executive officers and directors, combined with our stockholders who own more than 5% of our outstanding common stock and their affiliates, in the aggregate, beneficially owned shares representing approximately 75.5% of our capital stock as of December 31, 2015. As a result, if these stockholders were to choose to act together, they would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, these persons, if they choose to act together, would control the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets. This concentration of ownership control may:

- delay, defer or prevent a change in control;

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entrench our management or the board of directors; or

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

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

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

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

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

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

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

Item 1B. Unresolved Staff Comments

None.

Item 2. Properties

Our offices are located in Cambridge, Massachusetts and consist of approximately 19,000 square feet of leased office and laboratory space. The lease expires in June 2017. We believe that our existing facilities are sufficient for our needs for the foreseeable future.

Item 3. Legal Proceedings

From time to time we may become subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, as of the date of this Annual Report on Form 10-K, we do not believe we are party to any claim or litigation, the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 4. Mine Safety Disclosures

Not applicable.

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PART II

Item 5. Market for Registrant's Common Equity, Related Stockholder Matters and Issuer Purchases of Equity Securities

Market Information

Our common stock, $0.001 par value per share, has been publicly traded on the NASDAQ Global Market under the symbol "CATB" since June 25, 2015. Prior to that time, there was no public market for our common stock. The following table shows the high and low intraday sale prices per share of common stock as reported on the NASDAQ Global Market for the periods indicated:

2015:	High	Low
Second Quarter	$ 14.00	$ 11.51
Third Quarter	$ 16.96	$ 7.31
Fourth Quarter	$ 10.83	$ 6.32

On March 7, 2016, the last reported sale price for our common stock on the NASDAQ Global Market was $8.14 per share.

Holders

As of March 7, 2016, there were approximately 39 holders of record of our common stock. This number of holders of record does not include beneficial owners of our common stock whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

Dividends

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.

Comparative Stock Performance Graph

The performance graph in this Item 5 is not deemed to be "soliciting material" or to be "filed" with the Securities and Exchange Commission for purposes of Section 18 of the Securities and Exchange Act of 1934, as amended, or otherwise subject to the liabilities under that Section, and shall not be deemed incorporated by reference into any filing of Catabasis Pharmaceuticals, Inc. under the Securities Act of 1933 or the Securities Exchange Act of 1934, except to the extent we specifically incorporate it by reference into such a filing.

The following graph shows a comparison from June 25, 2015, the date on which our common stock first began trading on the NASDAQ Global Market, of the cumulative total return on an assumed investment of $100.00 in cash on June 25, 2015, in our common stock as compared to the same investment in the NASDAQ Composite Index, the NASDAQ Biotechnology Index, and the BioShares Biotechnology Clinical Trials Index, all through December 31, 2015. These returns are based on historical results and are not intended to suggest future performance. Data assumes the reinvestment of dividends. The graph assumes our closing sales price on June 25, 2015 of $13.00 per share as the initial value of our common stock and not the initial offering price to the public of $12.00 per share.

The comparisons shown in the graph below are based upon historical data. We caution that the stock price performance shown in the graph below is not necessarily indicative of, nor is it intended to forecast, the potential future performance of our common stock.

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COMPARISON OF CUMULATIVE TOTAL RETURN*
Catabasis Pharmaceuticals, Inc., NASDAQ Composite Index,
NASDAQ Biotechnology Index, and BioShares Biotechnology Clinical Trials Index

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* $100 invested on June 25, 2015

Cumulative Total Return Comparison

	6/25/2015	6/30/2015	9/30/2015	12/31/2015
Catabasis Pharmaceuticals, Inc.	$ 100.00	$ 93.92	$ 62.23	$ 61.00
Nasdaq Composite Index	$ 100.00	$ 97.55	$ 90.38	$ 97.95
Nasdaq Biotechnology Index	$ 100.00	$ 98.24	$ 80.56	$ 90.00
BioShares Biotechnology Clinical Trials Index	$ 100.00	$ 99.50	$ 72.47	$ 80.20

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Securities Authorized for Issuance under Equity Compensation Plans

The following table contains information about our equity compensation plans as of December 31, 2015:

Plan category	Number of securities to be issued upon exercise of outstanding stock options, warrants and rights (a)	Weighted-average exercise price of outstanding options, warrants and rights (b)	Number of securities remaining available for future issuance under equity compensation plans (excluding securities reflected in column (a)) (c)
Equity compensation plans approved by security holders	1,723,554(1)	$ 6.66	833,902(2)
Equity compensation plans not approved by security holders			
Total	1,723,554	$ 6.66	833,902

(1) Consists of stock options outstanding as of December 31, 2015 under our Amended and Restated 2008 Equity Incentive Plan, as amended, and our 2015 Stock Incentive Plan.

(2) Consists of shares issuable under our 2015 Stock Incentive Pland and our 2015 Employee Stock Purchase Plan, but does not reflect increases that were effective as of January 1, 2016

Recent Sales of Unregistered Securities

Set forth below is information regarding stock sold and options granted by us during the year ended December 31, 2015 that were not registered under the Securities Act of 1933, as amended, or the Securities Act. Included is the consideration, if any, we received for such shares and options and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, or the SEC, under which exemption from registration was claimed. No underwriters were involved in any such issuances.

(a)

Issuance of shares of preferred stock

In March 2015, we issued and sold an aggregate of 13,062,965 shares of our series B preferred stock to twenty investors at a price per share of $0.9503, for an aggregate purchase price of $12,413,736. These shares of series B preferred stock were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(a)(2) under the Securities Act and Regulation D promulgated thereunder, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to, or for sale in connection with, any distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(b)

Stock option grants

On February 20, 2015, pursuant to the terms of our Amended and Restated 2008 Stock Incentive Plan, as amended, we granted to certain of our employees and two advisors options to purchase an aggregate of 137,662 shares of our common stock, at an exercise price of $9.51 per share.

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On March 26, 2015, pursuant to the terms of our Amended and Restated 2008 Stock Incentive Plan, as amended, we granted to certain of our employees and two advisors options to purchase an aggregate of 73,236 shares of our common stock, at an exercise price of $11.05 per share.

On April 30, 2015, pursuant to the terms of our Amended and Restated 2008 Stock Incentive Plan, as amended, we granted to certain of our employees options to purchase an aggregate of 94,162 shares of our common stock, at an exercise price of $11.05 per share.

On June 30, 2015, pursuant to the terms of our 2015 Stock Incentive Plan, we granted to certain of our directors options to purchase an aggregate of 55,470 shares of our common stock, at an exercise price of $12.21 per share.

On July 8, 2015, we granted options to purchase an aggregate of 161,867 shares of common stock, at an exercise price of $14.05 per share, to an executive officer and director and to a consultant pursuant to our 2015 Stock Incentive Plan.

The issuances of stock options described above were issued pursuant to written compensatory plans or arrangements with our employees, directors and consultants, in reliance on the exemption provided by Rule 701 promulgated under the Securities Act, or pursuant to Section 4(a)(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. All recipients either received adequate information about us or had access, through employment or other relationships, to such information.

(c)

Warrant grants

On March 31, 2015, we issued warrants to purchase an aggregate of 157,844 shares of series B preferred stock at a price of $0.9503 per share to Square 1 Bank, Flexpoint MCLS Holdings, LLC and MidCap Financial Trust. These warrants were issued to investors in reliance upon the exemption from the registration requirements of the Securities Act, as set forth in Section 4(2) under the Securities Act, relative to transactions by an issuer not involving any public offering, to the extent an exemption from such registration was required. The recipients of securities in the transactions described above represented that they were accredited investors and were acquiring the securities for their own account for investment purposes only and not with a view to the public resale or distribution thereof and that they could bear the risks of the investment and could hold the securities for an indefinite period of time and appropriate legends were affixed to the instruments representing such securities issued in such transactions.

(d)

Conversion of shares of preferred stock

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

Purchases of Equity Securities

We did not purchase any of our registered equity securities during the period covered by this Annual Report on Form 10-K.

Use of Proceeds from IPO

In June 2015, we completed our initial public offering, or our IPO, in which we issued and sold 5,750,000 shares of our common stock at a public offering price of $12.00 per share, including 750,000 shares of common stock sold pursuant to the underwriters' exercise of their option to purchase additional shares of common stock, for aggregate gross proceeds of $69.0 million. All of the shares

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issued and sold in the IPO were registered under the Securities Act pursuant to a Registration Statement on Form S-1 (File No. 333-204144), which was declared effective by the SEC on June 24, 2015.

The net offering proceeds to us, after deducting underwriting discounts of $4.8 million and offering expenses payable by us totaling $2.5 million, were approximately $61.7 million.

As of December 31, 2015, we had used approximately $16.6 million of the net offering proceeds primarily to fund the costs of the clinical development of CAT-1004 and CAT-2054, to fund research and development to advance other product candidates and for working capital and general corporate purposes. None of the offering proceeds were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning 10.0% or more of any class of our equity securities or to any other affiliates, other than payments in the ordinary course of business to officers for salaries and to non-employee directors as compensation for board or board committee service. There has been no material change in our planned use of the net proceeds from our IPO as described in our final prospectus filed with the SEC pursuant to Rule 424(b)(4) on June 25, 2015.

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Item 6. Selected Financial Data

The selected consolidated statements of operations data for each of the three years in the period ended December 31, 2015 and the selected consolidated balance sheet data at December 31, 2015 and 2014 have been derived from our audited consolidated financial statements included elsewhere in this Annual Report on Form 10-K. We derived the consolidated financial data for the year ended December 31, 2012 and as of December 31, 2012 from our audited consolidated financial statements that are not included elsewhere in this Annual Report on Form 10-K. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our interim period results are not necessarily indicative of results to be expected in any future period.

The information set forth below should be read in conjunction with the "Management's Discussion and Analysis of Financial Condition and Results of Operations" section of this Annual Report on Form 10-K and with our consolidated financial statements and notes thereto included elsewhere in this Annual Report on Form 10-K. The selected consolidated financial data in this section are not intended to replace the consolidated financial statements and are qualified in their entirety by the consolidated financial statements and related notes included elsewhere in this Annual Report on Form 10-K.

	Catabasis Pharmaceuticals, Inc. Year Ended December 31,			
Consolidated Statement of Operations Data	**2015**	**2014**	**2013**	**2012**
	(in thousands, except share and per share data)			
Operating expenses	$ 31,659	$ 21,681	$ 18,119	$ 15,673
Loss from operations	(31,659)	(21,681)	(18,119)	(15,673)
Other income (expense)	$ (971)	$ (203)	$ 1	$ 4
Net loss	(32,630)	(21,884)	(18,118)	(15,669)
Net loss per share:				
Basic and Diluted	$ (4.06)	$ (51.56)	$ (47.80)	$ (42.26)
Weighted-average common shares outstanding used in net loss per share:				
Basic and Diluted	8,041,948	424,477	379,025	370,772
Balance Sheet Data:				
Cash and cash equivalents	$ 62,780	$ 14,668	$ 30,474	$ 5,434
Working capital	55,773	10,788	27,651	3,728
Total assets	64,223	15,964	31,002	6,314
Notes payable, net of current portion and discount	5,742	4,439		
Preferred stock		80,146	80,146	38,724
Common stock and additional paid-in-capital	158,503	2,327	1,312	942
Total stockholders' equity (deficit)	50,493	(73,053)	(52,184)	(34,436)

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Item 7. 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 consolidated financial statements and the related notes and other financial information included elsewhere in this Annual Report on Form 10-K. Some of the information contained in this discussion and analysis or set forth elsewhere in this Annual Report on Form 10-K, 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 Annual Report on Form 10-K 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 drug discovery platform. Our SMART linker drug discovery platform enables us to engineer product candidates that can simultaneously modulate multiple targets in a disease. Our proprietary product candidates impact pathways that are central to diseases where efficacy may be optimized by a multiple target approach. Our primary focus is on treatments for rare diseases. We are also developing other product candidates for the treatment of serious lipid disorders. We have applied our SMART linker drug discovery platform to build an internal pipeline of product candidates for rare diseases and plan to pursue partnerships to develop additional product candidates.

CAT-1004 is an oral small molecule that we believe has the potential to be a disease-modifying therapy for all patients affected by Duchenne muscular dystrophy, or DMD, regardless of the underlying dystrophin mutation. DMD is an ultimately fatal genetic disorder involving progressive muscle degeneration. 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. The United States Food and Drug Administration, or FDA, has granted orphan drug, fast track and rare pediatric disease designations to CAT-1004 for the treatment of DMD. The European Commission, or EC, also has granted orphan medicinal product designation to CAT-1004 for the treatment of DMD.

We are currently conducting the MoveDMD Phase 1/2 trial of CAT-1004 in boys with DMD between ages four and seven. We reported positive top-line results from Part A of the MoveDMD trial in January 2016. Subject to regulatory approval of our proposed protocol, we expect to initiate Part B of the MoveDMD trial in the first half of 2016 and to report top-line Part B data in late 2016, contingent on patient enrollment. If the results from our MoveDMD 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. If the results from the Phase 3 clinical trial are positive, we intend to seek marketing approval for CAT-1004. We hold rights to CAT-1004 throughout the world.

Our CAT-2000 series is our other clinical-stage program. We applied our SMART linker drug discovery platform to engineer the CAT-2000 series product candidates to inhibit the Sterol Regulatory Element Binding Protein, or SREBP, pathway. We used different SMART linkers to produce two CAT-2000 series product candidates, CAT-2054 and CAT-2003. These product candidates possess different pharmacokinetic and biodistribution characteristics. CAT-2003, our first generation product candidate, is an orally administered molecule that inhibits the SREBP pathway predominately in the intestine. CAT-2054, our second generation product candidate, is an orally administered molecule that inhibits the SREBP pathway predominately in the liver. We are developing CAT-2054 for serious lipid disorders such as hypercholesterolemia.

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By inhibiting SREBP, a master regulator of lipid metabolism in the body, CAT-2054 has the potential to significantly reduce low-density lipoprotein cholesterol, or LDL-C; it may also have beneficial effects on other metabolic parameters such as triglycerides, glucose and liver fat. This profile may differentiate CAT-2054 from currently approved therapies for hypercholesterolemia and others in development. We are developing CAT-2054 to be used in addition to statins in patients who cannot achieve their LDL-C goals with statins alone. We initiated a Phase 2a trial in patients with hypercholesterolemia in December 2015, which is ongoing. We anticipate that we will report top-line data from the Phase 2a trial in the third quarter of 2016. Additionally, we are currently conducting studies and have generated positive data in preclinical models that support the therapeutic potential of the CAT-2000 series in Nonalcoholic Steatohepatitis, or NASH.

CAT-4001 is a SMART linker conjugate of monomethyl fumarate and DHA. CAT-4001 is a small molecule that activates Nrf2 and inhibits NF-κB, or nuclear factor kappa-light chain enhancer of activated B cells, that we are developing as a potential treatment for neurodegenerative diseases such as Friedreich's ataxia and amyotrophic lateral sclerosis, or ALS. 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. The Nrf2 and NF-κB pathways have been implicated in Friedreich's ataxia and ALS. We plan to conduct investigational new drug application, or IND, enabling studies in 2016 for CAT-4001. We hold rights to CAT-4001 throughout the world.

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 primarily financed our operations through private placements of our preferred stock, a secured debt financing, and our IPO. From our inception through December 31, 2015, we had raised an aggregate of $172.2 million, of which $92.9 million was from private placements of preferred stock, $69.0 million represented in gross proceeds from our IPO, $10.0 million was from a secured debt financing and $0.3 million was from common stock option exercises.

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

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

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

We have not generated any revenue to date. We have incurred significant annual net operating losses in every year since our inception and expect to incur a net operating loss in 2015 and continue to incur net operating losses for the foreseeable future. As of December 31, 2015, we had an accumulated deficit of $108.0 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

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and conduct clinical trials with respect to our CAT-1004 and CAT-2054 product candidates; initiate and continue research, preclinical and clinical development efforts for our other product candidates and potential product candidates; maintain, expand and protect our intellectual property portfolio; establish a commercial infrastructure to support the marketing and sale of certain of our product candidates; hire additional personnel, such as clinical, regulatory, quality control and scientific personnel; and operate as a public company.

Financial Overview

Revenue

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

Research and Development Expenses

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

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

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

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

- CAT-1004 is an orally administered SMART linker conjugate of salicylate and DHA, that we designed to inhibit NF-κB, a protein that is activated in DMD and drives inflammation, fibrosis and muscle degeneration, and suppresses muscle regeneration. We are currently conducting the MoveDMD Phase 1/2 trial of CAT-1004 in boys with DMD between ages four and seven. We reported positive top-line results from Part A of the MoveDMD trial in January 2016. Top-line results indicated that all three doses of CAT-1004 studied were generally well tolerated with no safety signals observed. Top-line pharmacokinetic results demonstrated CAT-1004 average plasma exposure levels consistent with those previously observed in adults at which inhibition of NF-κB was observed. Subject to regulatory approval of our proposed protocol, we expect to initiate Part B of the MoveDMD trial in the first half of 2016 and to report top-line Part B data in late 2016, contingent on patient enrollment. If the results from our MoveDMD 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. If the results from the Phase 3 clinical trial are positive, we intend to seek marketing approval for CAT-1004. The FDA has

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granted CAT-1004 orphan drug, fast track and rare pediatric disease designations for the treatment of DMD. The European Commission has granted CAT-1004 orphan medicinal product designation to CAT-1004 for the treatment of DMD. We hold rights to CAT-1004 throughout the world.

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 primarily in the liver. By inhibiting SREBP, a master regulator of lipid metabolism in the body, CAT-2054 has the potential to significantly reduce LDL-C; it may also have beneficial effects on other metabolic parameters such as triglycerides, glucose and liver fat. This profile may differentiate CAT-2054 from currently approved therapies for hypercholesterolemia and others in development. We are developing CAT-2054 to be used in addition to statins in patients who cannot achieve their LDL-C goals with statins alone. We initiated a Phase 2a trial in patients with hypercholesterolemia in December 2015, which is ongoing. We anticipate that we will report top-line data from the Phase 2a trial in the third quarter of 2016. Additionally, we are currently conducting studies and have generated positive data in preclinical models that support the therapeutic potential of the CAT-2000 series in NASH. We hold rights to CAT-2054 throughout the world, and we intend to seek a partner for the program prior to initiating Phase 3 clinical trials.

CAT-2003 is our first-generation product candidate in the CAT-2000 series, and 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 in which we observed positive effects of CAT-2003 on triglycerides, LDL-C and glucose. We also observed gastrointestinal side effects. 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 drug discovery and the potential to treat lipid and metabolic disorders by modulating the SREBP pathway.

CAT-4001 is a conjugate of monomethyl fumarate and DHA that we designed to combine the potentially beneficial activities of monomethyl fumarate and DHA on the Nrf2 and NF-kB pathways. We are developing CAT-4001 initially for the treatment of neurodegenerative diseases in which the Nrf2 and NF-κB pathways have been implicated, such as Friedreich's ataxia and ALS. We plan to conduct investigational new drug application, or IND, enabling studies in 2016 for CAT-4001. We hold rights to CAT-4001 throughout the world.

Other research and development programs include 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.

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

	Year Ended December 31,		
	2015	2014	2013
CAT-1004	$ 6,036	$ 879	$ 108
CAT-2054	5,365	3,208	652
CAT-2003	1,028	3,807	6,727
Other research and platform programs	2,395	1,276	680
Costs not directly allocated to programs:			
Employee expenses including cash compensation, benefits and stock-based compensation	5,879	4,575	3,992
Facilities	828	733	754
Consultants and professional expenses, including stock-based compensation	987	736	605
Other	512	472	476
Total costs not directly allocated to programs	8,206	6,516	5,827
Total research and development expenses	$ 23,030	$ 15,686	$ 13,994

Since inception, the total direct expenses to support the CAT-1004 program have been $14.5 million. Since we began separately tracking CAT-2054 in 2013, the direct expenses to support that program have totaled $9.2 million.

The successful development of our product candidates is highly uncertain. Accordingly, at this time, we cannot reasonably estimate the nature, timing and costs of the efforts that will be necessary to complete the remainder of the development of these product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from 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 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

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

Other (Expense) Income, Net

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

Critical Accounting Policies and Significant Estimates

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

While our significant accounting policies are described in more detail in the notes to our financial statements included elsewhere in this Annual Report on Form 10-K, 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.

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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 issue stock-based awards to employees and non-employees, generally in the form of stock options. We account for our stock-based awards in accordance with the Financial Accounting Standards Board, or FASB, Accounting Standards Codification, or ASC, Topic 718, *Compensation Stock Compensation*, or ASC 718. ASC 718 requires all stock-based payments to employees, including grants of employee stock options and modifications to existing stock options, to be recognized in the consolidated statements of operations and comprehensive loss based on their fair values. We account for stock-based awards to non-employees in accordance with FASB ASC Topic 505-50, *Equity-Based Payments to Non-Employees*, which requires the fair value of the award to be re-measured at fair value as the award vests.

Our stock-based awards are subject to either service or performance-based vesting conditions. Compensation expense related to awards to employees and directors with service-based vesting conditions is recognized on a straight-line basis based on the grant date fair value over the associated service period of the award, which is generally the vesting term. Compensation expense related to awards to non-employees with service-based vesting conditions is recognized on the then-current fair value at each financial reporting date prior to the measurement date over the associated service period of the award, which is generally the vesting term, using the accelerated attribution method. Compensation expense related to awards to employees with performance-based vesting conditions is recognized based on the grant date fair value over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable. Compensation expense related to awards to non-employees with performance-based vesting conditions is recognized based on the then-current fair value at each financial reporting date prior to the measurement date

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over the requisite service period using the accelerated attribution method to the extent achievement of the performance condition is probable.

Described below is the methodology we have utilized in measuring stock-based compensation expense. Following the consummation of our IPO, stock option values have been determined based on the quoted market price of our common stock.

We estimate the fair value of our stock-based awards to employees and non-employees using the Black-Scholes option pricing model, which requires the input of subjective assumptions, including (i) the expected volatility of our stock, (ii) the expected term of the award, (iii) the risk-free interest rate and (iv) expected dividends. Due to the lack of company-specific historical and implied volatility data, we base our estimate of expected volatility on the historical volatility of a group of similar companies that are publicly traded. For these analyses, we select companies with comparable characteristics to ours including enterprise value, risk profiles and position within the industry, and with historical share price information sufficient to meet the expected life of the stock-based awards. We compute the historical volatility data using the daily closing prices for the selected companies' shares during the equivalent period of the calculated expected term of our stock-based awards. We will continue to apply this process until a sufficient amount of historical information regarding the volatility of our own stock price becomes available. We estimate the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the average of the vesting term and the original contractual term of the option. The risk-free interest rates for periods within the expected life of the option were based on the U.S. Treasury yield curve in effect during the period the options were granted.

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use 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 our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised. Stock-based compensation expense recognized in the financial statements is based on awards that are ultimately expected to vest.

We have computed the fair value of employee, director, consultant and advisor stock options at date of grant using the following weighted-average assumptions:

	Year Ended December 31,		
	2015	**2014**	**2013**
Weighted-average expected volatility	73.6 - 86.8%	75.2 - 83.4%	75.0 - 81.5%
Expected term (in years)	6.17 - 10.00	6.25 - 10.00	6.25 - 10.00
Risk free interest rate	0.92 - 2.45%	1.71 - 3.01%	0.92 - 2.03%
Expected dividend yield	0%	0%	0%

Prior to our IPO, the estimated fair value of our common stock was determined contemporaneously by our board of directors based on valuation estimates provided by management and prepared in accordance with the framework of the American Institute of Certified Public Accountants' *Technical Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation*. Certain of these valuation estimates were prepared with the assistance of a third-party specialist. Our contemporaneous valuations of our common stock were based on a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector and the prices at which we sold shares of preferred stock, the superior rights and preferences of securities senior to our common stock at the time of each grant and the likelihood of achieving a liquidity event such as an IPO.

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

	Year Ended December 31,		
	2015	2014	2013
Research and development	$ 681	$ 434	$ 224
General and administrative	977	463	119
Total	$ 1,658	$ 897	$ 343

Results of Operations

Comparison of the Years Ended December 31, 2015 and 2014

The following table summarizes our results of operations for the years ended December 31, 2015 and 2014, together with the dollar change in those items (in thousands):

	Year Ended December 31,		Period-to-Period Change
	2015	2014	
Operating expenses:			
Research and development	$ 23,030	$ 15,686	$ 7,344
General and administrative	8,629	5,995	2,634
Total operating expenses	31,659	21,681	9,978
Loss from operations	(31,659)	(21,681)	(9,978)
Other expense, net	(971)	(203)	(768)
Net loss	$ (32,630)	$ (21,884)	$ (10,746)

Research and Development Expenses

Research and development expenses increased by $7.3 million to $23.0 million for the year ended December 31, 2015 from $15.7 million for the year ended December 31, 2014, an increase of 46%. The increase in research and development expenses was primarily attributable to a net increase of $5.7 million in direct program costs, reflecting an increase of $5.2 million in costs related to CAT-1004 primarily related to the MoveDMD Phase 1/2 clinical trial, and a net increase of $0.5 million in costs related to our other programs. In addition, the costs related to internal research and development increased by $1.6 million, $0.8 million of which was attributable to compensation increases for new hires, $0.4 million of which was attributable to obligations under a letter agreement with a former employee, pursuant to which we agreed to make severance payments, $0.3 million of which was attributable to increases in consulting and professional services, and $0.1 million of which was attributable to increased facilities expense.

General and Administrative Expenses

General and administrative expenses increased by $2.6 million to $8.6 million for the year ended December 31, 2015 from $6.0 million for the year ended December 31, 2014, an increase of 43%. The increase in general and administrative expenses was primarily attributable to increased employee costs of $1.4 million associated with salaries, benefits, and stock-based compensation expenses for new hires; increased consulting and professional fees and franchise taxes of $0.8 million, driven by the costs of becoming and operating as a public company; increased insurance expense of $0.3 million due to our public company directors and officers insurance policy; and increased facilities expense of $0.1 million.

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Other Expense, Net

Other expense, net increased by $0.8 million to $1.0 million for the year ended December 31, 2015 from $0.2 million for the year ended December 31, 2014. Other expense primarily consists of interest expense, which increased by $0.8 million for the year ended December 31, 2015 due to the interest expense on our credit facility, which we entered into in August 2014.

Comparison of the Years Ended December 31, 2014 and 2013

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

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

Research and Development Expense

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 $0.8 million for CAT-1004 manufacturing and preclinical development costs, and an increase of $0.6 million in our general research and platform programs, 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 Expense

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.

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Other Expense (Income), Net

Other expense (income), net 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

From our inception to December 31, 2015, we raised an aggregate of $172.2 million, of which $92.9 million consisted of gross proceeds from private placements of preferred stock, $69.0 million consisted of gross proceeds from our IPO, $10.0 million consisted of gross proceeds from a secured debt financing and $0.3 million resulted from common stock option exercises. As of December 31, 2015, we had $62.8 million in cash and cash equivalents.

Initial Public Offering

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

Credit Facility

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

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

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

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of payment of the debt. At December 31, 2015 and December 31, 2014, we concluded that the likelihood of the acceleration of the debt was remote, as a material adverse change had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments.

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

Preferred Stock Financing

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

Funding Requirements

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

We expect that our cash and cash equivalents, including the proceeds from our IPO, will enable us to fund our operating expenses and capital expenditure requirements through the first quarter of 2017. We have based this estimate on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development of 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.

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

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

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

Cash Flows

Comparison of the Year Ended December 31, 2015 and 2014

The following table provides information regarding our cash flows for the year ended December 31, 2015 and 2014 (in thousands):

	Year Ended December 31,	
	2015	**2014**
Net cash used in operating activities	$ (29,793)	$ (20,412)
Net cash used in investing activities	(421)	(228)
Net cash provided by financing activities	78,326	4,834
Net increase (decrease) in cash and cash equivalents	$ 48,112	$ (15,806)

Net Cash Used in Operating Activities

Net cash used in operating activities was $29.8 million for the year ended December 31, 2015 and consisted primarily of a net loss of $32.6 million adjusted for non-cash items, including stock-based compensation expense of $1.7 million, non-cash interest expense of $0.3 million and depreciation and amortization expense of $0.2 million, and a net decrease in operating assets of $0.6 million, which resulted primarily from an increase in accrued expenses of $0.9 million and an increase in accounts payable of $0.2 million, partially offset by an increase in prepaid expenses and other current assets of $0.5 million.

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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 $0.3 million, and a net decrease in operating assets of $0.3 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 Investing Activities

Net cash used in investing activities was $0.4 million during the year ended December 31, 2015 compared to $0.2 million during the year ended December 31, 2014, an increase of $0.2 million, which primarily resulted from leasehold improvements pursuant to expanding our leased office space.

Net Cash Provided by Financing Activities

Net cash provided by financing activities was $78.3 million during the year ended December 31, 2015 compared to $4.8 million during the year ended December 31, 2014. The cash provided by financing activities for the year ended December 31, 2015 primarily consisted of net proceeds received from our IPO of $61.7 million, net proceeds of $12.3 million from the issuance of 13,062,965 shares of our series B preferred stock in March 2015, net borrowings of $4.2 million from our Credit Facility, and $0.1 million from stock option exercises. The cash provided by financing activities for the year ended December 31, 2014 primarily consisted of net borrowings of $4.7 million from our Credit Facility, and $0.1 million in proceeds from the exercise of common stock options.

Comparison of the Year Ended December 31, 2014 and 2013

The following table provides information regarding our cash flows for the years ended December 31, 2014 and 2013 (in thousands):

	Year Ended December 31,	
	2014	**2013**
Net cash used in operating activities	$ (20,412)	$ (16,366)
Net cash used in investing activities	(228)	(43)
Net cash provided by financing activities	4,834	41,449
Net decrease (increase) in cash and cash equivalents	$ (15,806)	$ 25,040

Net Cash Used in Operating Activities

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 $0.3 million, and a net decrease in operating assets of $0.3 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.

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Net Cash Used in Investing Activities

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 $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.

Off-Balance Sheet Arrangements

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

Contractual Obligations

The following table summarizes our significant contractual obligations as of payment due date by period at December 31, 2015:

	Payments due by period				
(In thousands)	**Total**	**Less than 1 Year**	**1 - 3 Years**	**3 - 5 Years**	**More than 5 Years**
Term loan(1)	$ 10,502	$ 3,915	$ 6,587	$	$
Operating lease obligations(2)	1,404	937	467		
Total contractual cash obligations	$ 11,906	$ 4,852	$ 7,054	$	$

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

(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.

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.

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Item 7A. Quantitative and Qualitative Disclosures about Market Risk

The market risk inherent in our financial instruments and in our financial position represents the potential loss arising from adverse changes in interest rates. As of December 31, 2015, we had cash and cash equivalents of $62.8 million and, as of December 31, 2014, we had cash and cash equivalents of $14.7 million, in each case 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. 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, 2015 and December 31, 2014, we had no liabilities denominated in foreign currencies.

Item 8. Financial Statements and Supplementary Data

The consolidated financial statements together with the report of our independent registered public company accounting firm, required to be filed pursuant to this Item 8 are appended to this Annual Report on Form 10-K. An index of those consolidated financial statements is found in Item 15 of this Annual Report on Form 10-K.

Item 9. Changes in and Disagreements with Accountants on Accounting and Financial Disclosure

There has been no change of accountants nor any disagreements with accountants on any matter of accounting principles or practices or financial disclosure required to be reported under this Item.

Item 9A. Controls and Procedures

Management's Evaluation of our Disclosure Controls and Procedures

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

As of December 31, 2015, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures as of the end of the period covered by this Annual Report on Form 10-K. Our management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives, and management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures. Our principal executive officer and principal financial officer have concluded based upon the evaluation described above that, as of December 31, 2015, our disclosure controls and procedures were effective at the reasonable assurance level.

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

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Changes in Internal Control over Financial Reporting

During the three months ended December 31, 2015, there have been no changes in our internal control over financial reporting, as such term is defined in Rules 13a-15 (f) and 15(d)-15(f) promulgated under the Exchange Act, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information

Not Applicable.

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PART III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this Item is set forth under the captions "Election of Directors," "Directors," "Corporate Governance," "Executive Officers," "Corporate Governance Code of Ethics" and "Compensation Governance Audit Committee Financial Expert" in our definitive proxy statement for our 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of December 31, 2015, and is incorporated into this Annual Report on Form 10-K by reference.

We are also required under Item 405 of Regulation S-K to provide information concerning delinquent filers of reports under Section 16 of the Securities and Exchange Act of 1934, as amended. This information will be set forth under the caption "Section 16(a) Beneficial Ownership Reporting Compliance" in our definitive proxy statement for the 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission no later than 120 days after the end of our fiscal year, and is incorporated herein by reference.

Item 11. Executive Compensation

The information required by this Item is set forth under the captions "Executive Officers," "Executive Compensation Compensation Discussion and Analysis," "Corporate Governance Compensation Committee Interlocks and Insider Participation," "Compensation Committee Report" and "Director Compensation" in our definitive proxy statement for our 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of December 31, 2015, and is incorporated into this Annual Report on Form 10-K by reference.

Item 12. Security Ownership of Certain Beneficial Owners and Management and Related Stockholder Matters

Securities Authorized for Issuance under Equity Compensation Plans

See "Securities Authorized for Issuance under Equity Compensation Plans" in Item 5 of this Annual Report on Form 10-K.

The other information required by this Item is set forth under the caption "Security Ownership of Certain Beneficial Owners and Management" in our definitive proxy statement for our 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of December 31, 2015, and is incorporated into this Annual Report on Form 10-K by reference.

Item 13. Certain Relationships and Related Transactions, and Director Independence

The information required by this Item is set forth under the captions "Corporate Governance Board Independence" and "Director Compensation Transactions with Related Persons" in our definitive proxy statement for our 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of December 31, 2015, and is incorporated into this Annual Report on Form 10-K by reference.

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Item 14. Principal Accountant Fees and Services

The information required by this Item is set forth under the caption "Independent Registered Public Accounting Firm" in our definitive proxy statement for our 2016 Annual Meeting of Stockholders to be filed with the Securities and Exchange Commission within 120 days of December 31, 2015, and is incorporated into this Annual Report on Form 10-K by reference.

PART IV

Item 15. Exhibits and Financial Statement Schedules

(a)(1) Financial Statements

The financial statements listed below are filed as part of this Annual Report on Form 10-K and are incorporated herein by reference.

(a)(2) Financial Statement Schedules

All financial schedules have been omitted because the required information is either presented in the consolidated financial statements filed as part of this Annual Report on Form 10-K or the notes thereto or is not applicable or required.

(a)(3) Exhibits

The exhibits required for this Annual Report on Form 10-K by Item 601 of Regulation S-K and Item 15(b) of Form 10-K are listed in the Exhibit Index immediately preceding the exhibits and are incorporated herein by reference.

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REPORT OF INDEPENDENT REGISTERED PUBLIC ACCOUNTING FIRM

The Board of Directors and Stockholders of
Catabasis Pharmaceuticals, Inc.

We have audited the accompanying consolidated balance sheets of Catabasis Pharmaceuticals, Inc. (the "Company") as of December 31, 2015 and 2014, and the related consolidated statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2015. 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 consolidated financial position of Catabasis Pharmaceuticals, Inc. at December 31, 2015 and 2014, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2015, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Boston, Massachusetts
March 15, 2016

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Catabasis Pharmaceuticals, Inc.

Consolidated Balance Sheets

(in thousands, except share and per share data)

	As of December 31,	
	2015	**2014**
Assets		
Current assets:		
Cash and cash equivalents	$ 62,780	$ 14,668
Prepaid expenses and other current assets	804	354
Total current assets	63,584	15,022
Property and equipment, net	504	288
Restricted cash	113	113
Other non-current assets	22	541
Total assets	$ 64,223	$ 15,964
Liabilities, convertible preferred stock, and stockholders' equity (deficit)		
Current liabilities:		
Accounts payable	$ 1,328	$ 1,132
Accrued expenses	3,278	2,793
Current portion of notes payable, net of discount	3,205	309
Total current liabilities	7,811	4,234
Deferred rent, net of current portion	26	67
Notes payable, net of current portion and discount	5,742	4,439
Other liability	151	23
Warrant liability		108
Total liabilities	13,730	8,871
Commitments (Note 9)		
Convertible preferred stock:		
Series A convertible preferred stock, $0.001 par value per share; zero and 68,837,703 shares authorized, issued and outstanding at December 31, 2015 and 2014, respectively		47,898
Series B convertible preferred stock, $0.001 par value per share; zero and 37,830,473 shares authorized, and zero and 34,129,571 shares issued and outstanding at December 31, 2015 and December 31, 2014, respectively		32,248
Stockholders' equity (deficit):		
Preferred stock, $0.001 par value per share, 5,000,000 and zero shares authorized at December 31, 2015 and 2014, respectively, zero shares issued and outstanding		
Common stock, $0.001 par value, 150,000,000 and 132,000,000 shares authorized at December 31, 2015 and 2014, respectively; 15,313,297 and 493,200 shares issued and outstanding at December 31, 2015 and 2014, respectively	15	1
Additional paid-in capital	158,488	2,326
Accumulated deficit	(108,010)	(75,380)
Total stockholders' equity (deficit)	50,493	(73,053)

Total liabilities, convertible preferred stock and stockholders' equity (deficit)	$	64,223	$	15,964

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

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Catabasis Pharmaceuticals, Inc.

Consolidated Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Year Ended December 31,		
	2015	**2014**	**2013**
Operating expenses:			
Research and development	$ 23,030	$ 15,686	$ 13,994
General and administrative	8,629	5,995	4,125
Total operating expenses	31,659	21,681	18,119
Loss from operations	(31,659)	(21,681)	(18,119)
Other income (expense):			
Other income, net	7	3	1
Interest expense	(978)	(206)	
Total other (expense) income	(971)	(203)	1
Net loss and comprehensive loss	$ (32,630)	$ (21,884)	$ (18,118)
Net loss per share basic and diluted	$ (4.06)	$ (51.56)	$ (47.80)
Weighted-average common shares outstanding used in net loss per share basic and diluted	8,041,948	424,477	379,025

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

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Catabasis Pharmaceuticals, Inc.

Consolidated Statements of Convertible Preferred Stock and Stockholders' Equity (Deficit)

(in thousands, except share data)

	Series A Convertible Preferred Stock		Series B Convertible Preferred Stock		Common Stock				
	Shares	Amount	Shares	Amount	Number of Shares	Par Value	Additional Paid-in Capital	Accumulated Deficit	Total Stockholders' Equity (Deficit)
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 common stock from initial public offering, net of issuance costs of $7.3 million					5,750,000	5	61,739		61,744
Issuance of series B convertible preferred stock, net of issuance cost of $82			13,062,965	12,331					
Conversion of convertible preferred stock into common stock	(68,837,703)	(47,898)	(47,192,536)	(44,579)	9,029,549	9	92,468		92,477
Conversion of series B preferred stock warrants into warrants for the purchase of common stock							206		206
Proceeds from exercises of common stock options					40,548		91		91
Stock-based compensation expense							1,658		1,658
Net loss								(32,630)	(32,630)
Balance at December 31, 2015		$		$	15,313,297	15	$ 158,488	$ (108,010)	$ 50,493

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

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Catabasis Pharmaceuticals, Inc.

Consolidated Statements of Cash Flows

(in thousands)

	Year Ended December 31,		
	2015	**2014**	**2013**
Operating activities			
Net loss	$ (32,630)	$ (21,884)	$ (18,118)
Reconciliation of net loss to net cash used in operating activities:			
Depreciation and amortization	202	248	320
Stock-based compensation expense	1,658	897	343
Non-cash interest expense	293	74	
Changes in assets and liabilities:			
Prepaid expenses and other current assets	(450)	(208)	75
Other assets	2		
Accounts payable	196	481	(129)
Accrued expenses	954	8	1,156
Deferred rent	(18)	(28)	(13)
Net cash used in operating activities	(29,793)	(20,412)	(16,366)
Investing activities			
Purchases of available-for-sale securities		(4,976)	
Sales of available-for-sale securities		4,976	
Purchases of property and equipment	(421)	(228)	(43)
Net cash used in investing activities	(421)	(228)	(43)
Financing activities			
Proceeds from initial public offering, net of issuance costs	61,744		
Proceeds from issuance of preferred stock, net of issuance costs	12,331		41,422
Proceeds from exercise of common stock options	91	118	27
Proceeds from borrowings	5,000	5,000	
Payments on borrowung	(833)		
Debt issuance costs	(7)	(284)	
Net cash provided by financing activities	78,326	4,834	41,449
Net increase (decrease) in cash and cash equivalents	48,112	(15,806)	25,040
Cash and cash equivalents, beginning of period	14,668	30,474	5,434
Cash and cash equivalents, end of period	$ 62,780	$ 14,668	$ 30,474
Supplemental disclosure of cash flow information			
Cash paid for interest	$ 684	$ 100	$
Non-cash financing activities			
Warrants for the purchase of series B preferred stock issued in conjunction with credit facility	$ 107	$ 110	$
Initial public offering costs in accounts payable and accrued liabilities	$ (492)	$ 492	$

Reclassification of deferred IPO costs from non-current assets to additional paid-in capital	$ 1,787	$	$
Conversion of convertible preferred stock to common stock	$ 92,477	$	$
Reclassification of warrant liability to additional paid-in capital	$ 206	$	$

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements

1. Organization and Operations

The Company

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

Liquidity

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

As of December 31, 2015, the Company had an accumulated deficit of $108.0 million. The Company has been primarily involved with research and development activities and has incurred operating losses and negative cash flows from operations since inception. The Company is subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its drug candidates, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology and market acceptance of the Company's products. The Company anticipates that it will continue to incur significant operating losses for the next several years as it continues to develop its product candidates. At December 31, 2015, the Company has sufficient cash and cash equivalents to fund operations through at least January 1, 2017.

2. Summary of Significant Accounting Policies

Reverse stock split

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

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

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements include the accounts of the Company and its wholly-owned subsidiary, Catabasis Securities Corporation. All intercompany balances and transactions have been eliminated in consolidation. These consolidated financial statements have been prepared in accordance with United States generally accepted accounting principles ("U.S. GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented.

Use of Estimates

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

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

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

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 backed by U.S. government securities, are stated at fair value. Cash and cash equivalents consist of the following (in thousands):

	December 31,	
	2015	2014
Cash	$ 776	$ 1,162
Money market fund	62,004	13,506
	$ 62,780	$ 14,668

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. 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, 2015 or 2014.

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

participants would use in pricing the asset or liability. Financial assets and liabilities are classified in their entirety based on the lowest level of input that is significant to the fair value measurement.

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

Deferred Financing

Deferred financing costs include costs directly attributable to the Company's offerings of its equity securities and its debt financings. Costs attributable to equity offerings are charged against the proceeds of the offering once the offering is completed. Costs attributable to debt financings are deferred and amortized over the term of the debt using the effective interest rate method.

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 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 significant impairment charges from inception through December 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. As of December 31, 2014, the total warrant liability was $0.1 million. There were no liability-classified warrants outstanding at December 31, 2015 (Note 8).

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

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 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, 2015, 2014 and 2013, 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,		
	2015	2014	2013
Research and development	$ 681	$ 434	$ 224
General and administrative	977	463	119
Total	$ 1,658	$ 897	$ 343

No related tax benefits were recognized for the years ended December 31, 2015, 2014 and 2013.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

Grant Awards

In the years ended December 31, 2015, 2014, and 2013, the Company received $100,000, $0, and $35,000, 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

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

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

	Year Ended December 31,		
	2015	**2014**	**2013**
Convertible preferred stock		8,012,988	8,012,988
Stock options	1,723,554	1,226,140	846,885
Common stock warrants	59,405	34,839	34,839
Preferred stock warrants		12,283	
	1,782,959	9,286,250	8,894,712

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, 2015, 2014, and 2013, the Company did not have any significant uncertain tax positions.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

2. Summary of Significant Accounting Policies (Continued)

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 December 31, 2015, 2014, and 2013, comprehensive loss was equal to net loss.

Recent Accounting Pronouncements

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

In April 2015, the FASB issued Accounting Standards Update ("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 impact of adopting the statement would result in the reclassification of $32,000 and $39,000 of deferred financing cost from prepaid and other current assets and $21,000 and $49,000 of other non-current assets as a reduction to notes payable and notes payable, net of current portion at December 31, 2015 and 2014, respectively. The Company did not early adopt the ASU.

In August 2014, the FASB issued ASU 2014-15, Presentation of Financial Statements Going Concern ("ASU 2014-15"), which is effective for annual periods ending after December 15, 2016. Early adoption is permitted. ASU 2014-15 provides new guidance on (1) management's responsibility in evaluating whether or not there is substantial doubt about a company's ability to continue as a going concern within one year from the date the financial statements are issued each reporting period and (2) related financial statement disclosures. The Company has not yet adopted the guidance prescribed by ASU 2014-15 and believes the new guidance will impact the disclosures in the notes to the Company's consolidated financial statements.

In February 2016, the FASB issued ASU 2016-02, *Leases*. This standard amends the existing guidance to require lessees to present most leases on their balance sheets but recognize corresponding expenses on their statements of operations. It is effective for annual reporting periods beginning after December 15, 2018, but early adoption is permitted. The Company is currently evaluating the impact that this standard will have on its consolidated financial statements.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

3. Financial Instruments

The following tables present information about the Company's financial assets and liabilities that have been measured at fair value, and indicates the fair value hierarchy of the valuation inputs utilized to determine such fair value. The Company determines the fair value of the Preferred Stock warrants (Note 8) using Level 3 inputs. Below is a summary of assets and liabilities measured at fair value on a recurring basis (in thousands):

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

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

As of December 31, 2015 and December 31, 2014, the Company's cash equivalents consisted principally of money market funds, which approximated their fair value due to their short-term nature. In connection with the completion of the IPO, warrants exercisable for Preferred Stock were automatically converted into warrants exercisable for Common Stock, resulting in the reclassification of the related warrant liability to additional paid-in capital as the warrants to purchase shares of Common Stock are accounted for as equity instruments (Note 8).

4. Restricted Cash

At December 31, 2015 and 2014, 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 9). The Company is required to maintain this deposit for the duration of the lease agreement.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

5. Property and Equipment

Property and equipment and related accumulated depreciation were as follows (in thousands):

		December 31,	
	Estimated Useful Life (Years)	2015	2014
Lab equipment	3	$ 1,350	$ 1,269
Computer equipment	3	166	110
Furniture and fixtures	5	77	30
Leasehold improvements	Lesser of useful life or remaining lease term	261	57
		1,854	1,466
Less accumulated depreciation and amortization		(1,350)	(1,178)
Total property and equipment, net		$ 504	$ 288

Depreciation and amortization expense was $0.2 million, $0.2 million, and $0.3 million for the years ended December 31, 2015, 2014, and 2013, respectively.

6. Accrued Expenses

Accrued expenses consisted of the following (in thousands):

	December 31,	
	2015	2014
Accrued compensation	$ 1,181	$ 796
Accrued contracted research costs	1,261	1,109
Accrued professional fees	181	791
Accrued other	655	97
Total	$ 3,278	$ 2,793

7. Notes Payable

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

Interest-only payments were due monthly on amounts outstanding under the Credit Facility until September 1, 2015 and, thereafter, interest and principal payments are due in 36 equal monthly

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

7. Notes Payable (Continued)

installments from October 1, 2015 through September 1, 2018. Amounts due under the Credit Facility bear interest at an annual rate of 7.49%. In addition, a final payment equal to 3.48% of any amounts drawn under the Credit Facility is due upon the earlier of the maturity date, acceleration of the term loans or prepayment of all or part of the term loans. The final payment is being accrued as additional interest expense using the effective-interest method from the date of issuance through the maturity date, and is recorded within other long-term liabilities. In the event of prepayment, the Company is obligated to pay 1% to 3% of the amount of the outstanding principal depending upon the timing of the prepayment. The effective interest rate as of December 31, 2015 was 11.2%.

In conjunction with Term Loan A, the Company issued warrants (the "2014 Warrants") to purchase 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders. In conjunction with Term Loan B, the Company issued warrants (the "2015 Warrants") to purchase an additional 157,844 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders (see Note 8). The 2014 Warrants and 2015 Warrants were exercisable immediately and have seven-year lives. The 2014 Warrants and 2015 Warrants were initially valued at $0.1 million and $0.1 million, respectively, using the Black-Scholes option-pricing model. The Company recorded debt discounts of $0.1 million and $0.1 million upon issuance of the 2014 Warrants and 2015 Warrants, respectively, which are being accreted as interest expense using the effective-interest method over the remaining term of the loan.

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

Upon the occurrence and continuation of an event of default, the lenders have the right to exercise certain remedies against the Company and the collateral securing the loans under the Credit Facility, including cash. Events of default include, among other things, failure to pay amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, which includes a material adverse change in the business, operations or conditions (financial or otherwise) of the Company or a material impairment of the prospect of repayment of any portion of the obligations, the occurrence of any default under certain other indebtedness and a final judgment against the Company in an amount greater than $250,000. The occurrence of a material adverse change could result in acceleration of the payment of the debt. At December 31, 2015 and December 31, 2014, the Company concluded that the likelihood of the acceleration of the debt was remote, as a material adverse change had not occurred and was unlikely to occur and therefore the debt was classified in current and long-term liabilities based on scheduled principal payments. Following the occurrence and during the continuance of an event of default, borrowings under the Credit Facility shall bear interest at a rate per annum, which is five hundred basis points, or 5.00%, above the rate that is otherwise applicable.

The Company assessed all terms and features of the Credit Facility in order to identify any potential embedded features that would require bifurcation or any beneficial conversion features. As part of this analysis, the Company assessed the economic characteristics and risks of the Credit Facility, including put and call features. The Company determined that all features of the Credit Facility were clearly and closely associated with a debt host and did not require bifurcation as a derivative liability,

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

7. Notes Payable (Continued)

or the fair value of the feature was immaterial to the Company's financial statements. The Company reassesses the features on a quarterly basis to determine if they require separate accounting.

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.* The December 22, 2015 amendment primarily changed the agreement to allow the Company to establish a subsidiary and did not impact the accounting treatment.

Estimated future principal payments at December 31, 2015 are as follows (in thousands):

Year Ending December 31,	Amount
2016	$ 3,334
2017	3,333
2018	2,500
Total	$ 9,167
Less: discount for warrants and costs paid to lender	(220)
Less: current portion	(3,205)
Note payable, net of current portion and discount	$ 5,742

During the years ended December 31, 2015, 2014, and 2013, the Company recognized $1.0 million, $0.2 million, and $0.0 million, respectively, of interest expense related to the Credit Facility.

8. Warrants

On August 27, 2014 and March 31, 2015, the Company issued the 2014 Warrants and 2015 Warrants to purchase an aggregate 315,688 shares of series B convertible preferred stock at an exercise price of $0.9503 per share to the lenders in connection with the Credit Facility (Note 7). The 2014 Warrants and 2015 Warrants were exercisable upon issuance and have a seven-year life. The 2014 Warrants and 2015 Warrants were recorded as a liability and re-measured at each reporting date using the then-current assumptions. In connection with the completion of the IPO, the 2014 Warrants and the 2015 Warrants were automatically converted into warrants exercisable for 24,566 shares of Common Stock with an exercise price of $12.21 per share, resulting in the reclassification of the related warrant liability to additional paid-in capital as the warrants to purchase shares of Common Stock met the criteria to be accounted for as equity-classified instruments. The warrant liability was re-measured to fair value immediately prior to reclassification to additional paid-in capital. As of December 31, 2015, the Company had no outstanding warrant liability.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

8. Warrants (Continued)

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

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

The fair value of warrants exercisable for 315,688 shares of series B convertible preferred stock was estimated using the Black-Scholes option pricing model with the following weighted-average assumptions:

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

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

At various dates from 2008 through 2010, the Company issued warrants to purchase an aggregate of 34,839 shares of Common Stock at an exercise price of $1.67 per share to various individuals, including its founders and employees of the Company. The warrants have six-year terms.

9. 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 (the "2011 Lease Amendment") that expanded the leased premises beginning in the second quarter of 2012. The 2011 Lease Amendment also extended the term of the existing lease through June 30, 2017. The 2011 Lease Amendment includes a free rent period for the expansion premises and escalating rent payments. In July 2015, the Company signed another lease amendment (the "2015 Lease Amendment") that expanded the leased premises beginning in the third quarter of 2015. The 2015 Lease Amendment includes escalating rent payments and is effective through June 30, 2017. The Company is recognizing rent expense on a straight-line basis over the lease term. The lease agreement provides for a five-year extension upon the completion of the lease term.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

9. Commitments (Continued)

Future minimum payments required under the non-cancelable operating lease as of December 31, 2015 are summarized as follows (in thousands):

Period Ending December 31,	Amount
2016	$ 937
2017	467
Total minimum lease payments	$ 1,404

Rent expense for the years ended December 31, 2015, 2014, and 2013 was $0.8 million, $0.7 million, and $0.7 million, respectively.

10. Convertible Preferred Stock

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

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

Upon the closing of the Company's IPO on June 30, 2015, all outstanding shares of the Company's Preferred Stock were automatically converted into 9,029,549 shares of Common Stock. As of December 31, 2015, the Company has 5,000,000 shares of Preferred Stock authorized for issuance, $0.001 par value per share, with none issued or outstanding.

Preferred stock may be issued from time to time in one or more series, each series to have such terms as stated or expressed in the resolutions providing for the issue of such series adopted by the board of directors of the Company. Preferred Stock which may be redeemed, purchased or acquired by the Company may be reissued except as otherwise provided by law.

11. Common Stock

As of December 31, 2015, the Company has 150,000,000 shares of Common Stock authorized for issuance, $0.001 par value per share, with 15,313,297 shares issued and outstanding. 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 any outstanding 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.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

11. Common Stock (Continued)

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 December 31, 2015.

Liquidation

In the event of any voluntary or involuntary liquidation, dissolution or winding up of the Company, the holders of Common Stock are entitled to share ratably in the Company's assets available for distribution to stockholders, subject to any preferential or other rights of any then-outstanding Preferred Stock.

Reserved for Future Issuance

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

	As of December 31,	
	2015	2014
Conversion of Series A Preferred Stock		5,356,996
Conversion of Series B Preferred Stock		2,655,992
Warrants for the purchase of Preferred Stock		12,283
Warrants for the purchase of Common Stock	59,405	34,839
Options to purchase Common Stock	2,557,456	1,385,341
Employee Stock Purchase Plan	182,352	
Total	2,799,213	9,445,451

12. Stock Incentive Plans

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

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

12. Stock Incentive Plans (Continued)

equal to the lowest of 1,297,334 shares of Common Stock, 4% of the number of shares of Common Stock outstanding on the first day of the fiscal year and an amount determined by the Company's board of directors.

As of December 31, 2015, the Company had reserved 1,421,672 shares of Common Stock under the 2008 Plan, of which none remained available for future issuance. As of December 31, 2015, the Company had reserved 1,135,784 shares of Common Stock under the 2015 Plan, of which 833,902 shares remained available for future issuance. Under the 2015 Plan, stock options may not be granted with exercise prices at less than fair value on the date of the grant.

Terms of stock option agreements, including vesting requirements, are determined by the Company's board of directors, subject to the provisions of the applicable stock incentive plan. Options 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 December 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 for employees and nonemployees follows:

	Shares	Weighted-Average Exercise Price	Weighted Average Remaining Contractual Term (years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2014	1,226,140	$ 4.14	8.15	$ 6,579
Granted	606,942	11.42		
Exercised	(40,548)	2.24		
Cancelled or forfeited	(68,980)	6.49		
Outstanding at December 31, 2015	1,723,554	$ 6.66	7.92	$ 4,267
Exercisable at December 31, 2015	795,501	$ 3.47	6.77	$ 3,562
Vested or expected to vest at December 31, 2015	1,607,456	$ 6.40	7.84	$ 4,223

The total intrinsic value of options exercised for the years ended December 31, 2015, 2014 and 2013 was $0.3 million, $0.6 million and $0.1 million, respectively. The total fair value of employee options vested for the years ended December 31, 2015, 2014 and 2013 was $1.4 million, $0.5 million and $0.3 million, respectively. The weighted-average grant date fair value of options granted to employees and non-employees for the years ended December 31, 2015, 2014 and 2013 was $7.68, $4.94 and $1.66, respectively.

At December 31, 2015, the total unrecognized compensation expense related to unvested stock option awards, including estimated forfeitures, was $4.8 million. The Company expects to recognize that cost over a weighted-average period of approximately 2.9 years.

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

12. Stock Incentive Plans (Continued)

Stock-Based Compensation Expense

The fair value of stock options granted to employees and non-employees was estimated using the Black-Scholes option-pricing model based on the following assumptions:

	Year Ended December 31,		
	2015	**2014**	**2013**
Weighted-average expected volatility	73.6 - 86.8%	75.2 - 83.4%	75.0 - 81.5%
Expected term (in years)	6.17 - 10.00	6.25 - 10.00	6.25 - 10.00
Risk free interest rate	0.92 - 2.45%	1.71 - 3.01%	0.92 - 2.03%
Expected dividend yield	0%	0%	0%

Volatility

Due to the lack of company specific historical and implied volatility data of its Common Stock, the Company 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.

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.

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.

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

12. Stock Incentive Plans (Continued)

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.

Employee Stock Purchase Plan

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

13. Income Taxes

For the years ended December 31, 2015, 2014, and 2013, the Company did not record a provision for federal or state income taxes as it has incurred cumulative net operating losses since inception.

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, 2015, 2014 and 2013:

	Year Ended December 31,		
	2015	**2014**	**2013**
Federal income tax (benefit) at statutory rate	34.00%	34.00%	34.00%
Permanent differences	(0.86)	(1.29)	(0.50)
Federal research and development credits and adjustments	2.64	2.70	5.80
State income tax, net of federal benefit	5.77	6.03	5.88
Other	0.28	(0.15)	(0.01)
Change in valuation allowance	(41.82)	(41.29)	(45.17)
Effective income tax rate	%	%	%

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

13. Income Taxes (Continued)

The Company's deferred tax assets consisted of the following (in thousands):

	Year Ended December 31,	
	2015	2014
Deferred tax assets		
Net operating loss carryforwards	$ 35,042	$ 22,577
Tax credit carryforwards	3,762	2,748
Capitalized research and development	4,087	4,780
Capitalized legal expenses	1,464	1,223
Other differences	815	195
Total deferred tax assets	45,170	31,523
Valuation allowance	(45,170)	(31,523)
Net deferred tax assets	$	$

The Company recorded increases to the valuation allowance of $13.6 million, $9.0 million, and $8.2 million during the years ended December 31, 2015, 2014 and 2013, respectively, 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, 2015 and 2014.

As of December 31, 2015, the Company had approximately $89.8 million of federal and $88.4 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 2035, if not utilized. Approximately $0.4 and $0.4 million of the federal and state net operating loss carryforward will result in an increase to additional paid-in capital if and when these carryforwards are used to reduce income taxes payable. Included in the federal and state net operating losses are deductions attributable to excess tax benefits from the exercise of non-qualified stock options of $0.4 million. The Company also had approximately $3.0 million of federal and $1.2 million of state tax credit carryforwards available to reduce future tax liabilities as of December 31, 2015, which will expire at varying times through the year 2035.

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

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Catabasis Pharmaceuticals, Inc.

Notes to Consolidated Financial Statements (Continued)

13. Income Taxes (Continued)

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.

As of December 31, 2015 and 2014, the Company did not have any significant unrecognized tax benefits.

As of December 31, 2015, 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, 2015 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, 2015, 2014, or 2013.

15. Quarterly Financial Information (unaudited, in thousands, except share and per share data)

	Three Months Ended			
	March 31, 2015	June 30, 2015	September 30, 2015	December 31, 2015
Operating expenses	$ 6,360	$ 7,765	$ 8,201	$ 9,333
Net loss	(6,500)	(8,039)	(8,485)	(9,606)
Net loss per share:				
Basic and Diluted	$ (13.14)	$ (8.07)	$ (0.55)	$ (0.63)
Weighted-average common shares outstanding used in net loss per share:				
Basic and Diluted	494,590	996,592	15,297,794	15,298,810

	Three Months Ended			
	March 31, 2014	June 30, 2014	September 30, 2014	December 31, 2014
Operating expenses	$ 4,469	$ 5,364	$ 5,970	$ 5,878
Net loss	(4,469)	(5,363)	(6,025)	(6,027)
Net loss per share:				
Basic and Diluted	$ (11.29)	$ (13.42)	$ (13.55)	$ (14.20)
Weighted-average common shares outstanding used in net loss per share:				
Basic and Diluted	395,774	399,766	444,787	424,477

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SIGNATURES

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

Catabasis Pharmaceuticals, Inc.

Date: March 15, 2016 By: /s/ JILL C. MILNE

Jill C. Milne
President and Chief Executive Officer

Pursuant to the requirements of the Securities Exchange Act of 1934, this report has been signed by the following persons on behalf of the registrant in the capacities and on the dates indicated.

Signature	Title	Date
/s/ JILL C. MILNE Jill C. Milne	President and Chief Executive Officer and Director (Principal Executive Officer)	March 15, 2016
/s/ IAN C. SANDERSON Ian C. Sanderson	Chief Financial Officer and Treasurer (Principal Financial and Accounting Officer)	March 15, 2016
/s/ MICHAEL ROSS Michael Ross	Director	March 15, 2016
/s/ NICHOLAS GALAKATOS Nicholas Galakatos	Director	March 15, 2016
/s/ JEAN GEORGE Jean George	Director	March 15, 2016
/s/ RON LAUFER Ron Laufer	Director	March 15, 2016
/s/ KENNETH BATE Kenneth Bate	Director	March 15, 2016

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EXHIBIT INDEX

Exhibit Number	Description of Exhibit
3.1(1)+	Restated Certificate of Incorporation of the Registrant
3.2(1)+	Amended and Restated Bylaws of the Registrant
4.1(2)+	Specimen stock certificate evidencing the shares of common stock
10.1(1)+	Warrant to purchase shares of Series B Preferred Stock issued on August 27, 2014 by the Registrant to Square 1 Bank
10.2(1)+	Warrant to purchase shares of Series B Preferred Stock issued on August 27, 2014 by the Registrant to Midcap Financial SBIC, L.P.
10.3(1)+*	Amended and Restated 2008 Equity Incentive Plan, as amended
10.4(1)+*	Form of Incentive Stock Option Agreement under Amended and Restated 2008 Equity Incentive Plan
10.5(1)+*	Form of Nonstatutory Stock Option Agreement under Amended and Restated 2008 Equity Incentive Plan
10.6(3)+*	2015 Stock Incentive Plan
10.7(3)+*	Form of Incentive Stock Option Agreement under 2015 Stock Incentive Plan
10.8(3)+*	Form of Nonstatutory Stock Option Agreement under 2015 Stock Incentive Plan
10.9(1)+*	Amended and Restated Employment Agreement, dated as of April 7, 2010, by and between the Registrant and Jill C. Milne, as amended
10.10(1)+*	Amended and Restated Employment Agreement, dated as of April 7, 201, by and between the Registrant and Michael Jirousek
10.11(1)+*	Offer Letter, dated as of October 21, 2013, by and between the Registrant and Ian C. Sanderson
10.12(1)+*	Form of Indemnification Agreement by and between the Registrant and each of its executive officers and directors
10.13(1)+	Credit and Security Agreement, dated as of August 27, 2014, by and among the Registrant, Midcap Financial SBIC, L.P., Square 1 Bank and the other lenders identified therein, as amended on March 31, 2015
10.14(1)+	Indenture of Lease, dated as of December 17, 2010, by and between the Registrant and RB Kendall Fee, LLC, as amended
10.15(1)+	Form of Common Stock Warrant
10.16(1)+*	Offer Letter, dated as of January 22, 2015, by and between the Registrant and Rick Modi
10.17(1)+	Warrant to purchase shares of Series B Preferred Stock issued on March 31, 2015 to Square 1 Bank
10.18(1)+	Warrant to purchase shares of Series B Preferred Stock issued on March 31, 2015 to Midcap Financial Trust
10.19(1)+	Warrant to purchase shares of Series B Preferred Stock issued on March 31, 2015 to Flexpoint MCLS Holdings, LLC
10.20(1)+*	2015 Employee Stock Purchase Plan

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Exhibit Number	Description of Exhibit
10.21(1)+*	Letter Agreement, dated May 15, 2015, by and between the Registrant and Michael Jirousek
10.22(4)+	Second Amendment of Lease, dated as of July 16, 2015, by and between the Registrant and DWF IV One Kendall, LLC
10.23*	Offer Letter, dated as of September 30, 2015, by and between the Registrant and Deirdre Cunnane
10.24	Second Amendment to Credit and Security Agreement, dated as of December 22, 2015, by and among the Registrant, Midcap Financial SBIC, L.P., Square 1 Bank and the other lenders identified therein
10.25	Summary of Non-employee Director Compensation Program
21.1	Subsidiaries of the Registrant
23.1	Consent of Ernst & Young LLP, independent registered public accounting firm
31.1	Certification of Principal Executive Officer pursuant to Rule 13a-14(a)/Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended
31.2	Certification of Principal Financial Officer pursuant to Rule 13a-14(a)/Rule 15d-14(a) of the Securities Exchange Act of 1934, as amended
32.1	Certification of Chief Executive Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
32.2	Certification of Chief Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	XBRL Instance Document
101.SCH	XBRL Taxonomy Extension Schema Document
101.CAL	XBRL Taxonomy Calculation Linkbase
101.LAB	XBRL Taxonomy Labels Linkbase Document
101.PRE	XBRL Taxonomy Presentation Linkbase Document
101.DEF	Taxonomy Extension Definition Linkbase Document

+ Previously filed.

* Management contract or compensatory plan arrangement.

(1) Incorporated by reference to the Registration Statement on Form S-1 (File No. 333-204144) filed with the Securities and Exchange Commission on May 13, 2015.

(2) Incorporated by reference to the Registration Statement on Form S-1 (File No. 333-204144) filed with the Securities and Exchange Commission on June 11, 2015.

(3) Incorporated by reference to the Registration Statement on Form S-1 (File No. 333-204144) filed with the Securities and Exchange Commission on June 3, 2015.

(4) Incorporated by reference to the Registrant's Quarterly Report on Form 10-Q for the quarter ended September 30, 2015.