

# ESPERION THERAPEUTICS, INC.

## FORM 10-K (Annual Report)

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**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

**FORM 10-K**

(Mark One)

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

For the fiscal year ended December 31, 2017

Or

**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: 001-35986

**Esperion Therapeutics, Inc.**

(Exact Name of Registrant as Specified in its Charter)

**Delaware**  
(State or Other Jurisdiction of  
Incorporation or Organization)

**3891 Ranchero Drive, Suite 150  
Ann Arbor, Michigan 48108**  
(Address of Principal Executive Offices)

**26-1870780**  
(I.R.S. Employer Identification No.)

**48108**  
(Zip Code)

**(734) 887-3903**  
(Registrant's Telephone Number, Including Area Code)

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

<u>Title of each class</u>	<u>Name of each exchange on which registered</u>
Common Stock, \$0.001 par value	NASDAQ Stock Market LLC

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. Yes  No

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

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  No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S-K (§229.405 of this chapter) 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.

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

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

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

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

The aggregate market value of the voting stock held by non-affiliates of the registrant on June 30, 2017, based upon the closing price of \$46.28 of the registrant's common stock as reported on the NASDAQ Global Market, was \$807.0 million. Shares of the registrant's common stock held by each officer and director and each person known to the registrant to own 10% or more of the outstanding voting power of the registrant have been excluded in that such persons may be deemed affiliates. This determination of affiliate status is not a determination for other purposes.

As of February 1, 2018, there were 26,499,269 shares of the registrant's common stock, \$0.001 par value per share, outstanding.

**DOCUMENTS INCORPORATED BY REFERENCE**

Part III of this Annual Report on Form 10-K incorporates by reference information from the definitive Proxy Statement for the registrant's 2018 Annual Meeting of Shareholders, which is expected to be filed with the Securities and Exchange Commission not later than 120 days after the Registrant's fiscal year ended December 31, 2017.

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## Forward-Looking Statements

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, objectives of management and expected market growth, are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," 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 ability to obtain regulatory approval for the bempedoic acid / ezetimibe combination pill and bempedoic acid, including statements related to specific clinical studies or clinical observations that will be required for such approval;
- our ability to achieve clinical or regulatory milestones with our existing cash resources;
- the design, timing or outcome of our Phase 3 clinical program for the bempedoic acid / ezetimibe combination pill and bempedoic acid;
- the design, timing or outcome of our cardiovascular outcomes trial, or CVOT, of bempedoic acid;
- the design, timing or outcome of our other clinical studies of the bempedoic acid / ezetimibe combination pill and bempedoic acid;
- our ability to recruit and enroll patients, particularly statin intolerant patients, in any ongoing or future clinical study;
- our ability to replicate positive results from a completed clinical study in a future clinical study;
- our ability to fund our development programs with existing capital or our ability to raise additional capital in the future;
- the potential benefits, effectiveness or safety of the bempedoic acid / ezetimibe combination pill and bempedoic acid, as compared to statins and other low density lipoprotein cholesterol, or LDL-C, lowering therapies, either those currently available or those in development;
- our ability to respond and adhere to changes in regulatory requirements, including any requirement to conduct additional, unplanned clinical studies in connection with our pursuit of the bempedoic acid / ezetimibe combination pill or bempedoic acid as an LDL-C lowering therapy;
- guidelines relating to LDL-C levels and cardiovascular risk that are generally accepted within the medical community, including recent changes and any future changes to such guidelines;
- reimbursement policies, including any future changes to such policies or related government legislation, and their impact on our ability to market, distribute and obtain payment for the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved;

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- the accuracy of our estimates of the size and growth potential of the LDL-C lowering market and the rate and degree of the bempedoic acid / ezetimibe combination pill or bempedoic acid's market acceptance, if approved;
- our ability to obtain and maintain intellectual property protection for the bempedoic acid / ezetimibe combination pill or bempedoic acid without infringing on the intellectual property rights of others;
- the loss of any of our key scientific or management personnel;
- our intention to seek to establish strategic relationships or partnerships; and
- our ability to compete with other companies that are, or may be, developing or selling products that may compete with the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved.

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, so 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 based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this Annual Report on Form 10-K, particularly in Item 1A. Risk Factors, that could cause actual future 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, joint ventures or investments we may make.

You should read this Annual Report on Form 10-K and the documents that we have filed as exhibits to the 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 statements whether as a result of new information, future events or otherwise, except as required by applicable law.

## PART I

*All brand names or trademarks appearing in this report are the property of their respective holders. Unless the context requires otherwise, references in this report to "Esperion" the "Company," "we," "us," and "our" refer to Esperion Therapeutics, Inc.*

### Item 1. Business

#### Overview

We are the Lipid Management Company, a late-stage pharmaceutical company focused on developing and commercializing complementary, convenient, cost-effective, once-daily, oral therapies for the treatment of patients with elevated LDL-C. Through scientific and clinical excellence, and a deep understanding of cholesterol biology, the experienced lipid management team at Esperion is committed to developing new LDL-C lowering therapies that will make a substantial impact on reducing global cardiovascular disease, or CVD; the leading cause of death around the world. Bempedoic acid and our lead product candidate, the bempedoic acid / ezetimibe combination pill, are targeted therapies that have been shown to significantly reduce elevated LDL-C levels in patients with hypercholesterolemia, including patients inadequately treated with current lipid-modifying therapies.

The clinical development program for the bempedoic acid / ezetimibe combination pill consists of a single pivotal Phase 3 clinical study (1002FDC-053) in patients with hypercholesterolemia and with atherosclerotic cardiovascular disease, or ASCVD, and/or heterozygous familial hypercholesterolemia, or HeFH, including high CVD risk primary prevention patients, whose LDL-C is not adequately controlled despite receiving maximally tolerated lipid-modifying background therapy. 1002FDC-053 initiated in November 2017 and we expect to report top-line results in August 2018.

The global pivotal Phase 3 clinical development program for bempedoic acid, consisting of four clinical studies, fully enrolled approximately 3,600 high CVD risk patients with hypercholesterolemia and ASCVD and/or HeFH, or who are high CVD risk primary prevention, on optimized background lipid-modifying therapy and with elevated levels of LDL-C. These patients are on two distinct types of background lipid-modifying therapy: 1) patients on their maximally tolerated statin therapy, and 2) patients who are only able to tolerate less than the lowest approved daily starting dose of a statin, and can be considered statin intolerant. In March 2018, we expect to report top-line results from the first of the Phase 3 studies, Study 4 (1002-048). In May 2018, we expect to report top-line results from the 52-week long-term safety study, Study 1 (1002-040), and top-line results from Study 3 (1002-046). In September 2018, top-line results are expected from Study 2 (1002-047).

We intend to use positive results from our Phase 3 bempedoic acid / ezetimibe combination pill and bempedoic acid programs with a total of 4,000 patients to support our global regulatory submissions for tandem LDL-C lowering indications in the U.S. by the first quarter of 2019 and in Europe by the second quarter of 2019.

We are also conducting a global cardiovascular outcomes trial, or CVOT,—known as **C**holesterol **L**owering via **B**empedoic Acid, an **A**CL-inhibiting **R**egimen (CLEAR) Outcomes, for bempedoic acid in patients with hypercholesterolemia and high CVD risk and who can be considered statin intolerant. We initiated the CLEAR Outcomes CVOT in December 2016, and intend to use positive results from this CVOT to support our submissions for a CV risk reduction indication in the U.S. and Europe by 2022.

In December 2017, we submitted an investigational new drug, or IND, application to the Food and Drug Administration, or FDA, for a reformulated tablet of bempedoic acid for a nonalcoholic steatohepatitis, or NASH, indication, which was accepted in January 2018.

We were founded in January 2008, by former executives of and investors in the original Esperion Therapeutics, Inc., a biopharmaceutical company which was primarily focused on the research and

development of therapies to regulate high-density lipoprotein, or HDL. The original Esperion was acquired by Pfizer Inc. in 2004. Bempedoic acid was first discovered at the original Esperion and we subsequently acquired the rights to the product from Pfizer in 2008. We own the exclusive worldwide rights to bempedoic acid.

### **Bempedoic Acid / Ezetimibe Combination Pill**

Through the complementary mechanisms of action of inhibition of cholesterol synthesis (bempedoic acid) and inhibition of cholesterol absorption (ezetimibe), the bempedoic acid / ezetimibe combination pill is our lead, non-statin, orally available, once-daily, LDL-C lowering therapy. Inhibition of ATP Citrate Lyase, or ACL, by bempedoic acid reduces cholesterol biosynthesis and lowers LDL-C by up-regulating the LDL receptor. Inhibition of Niemann-Pick C1-Like 1 by ezetimibe results in reduced absorption of cholesterol from the gastrointestinal tract, thereby reducing delivery of cholesterol to the liver, which in turn upregulates the LDL receptors. Previously completed Phase 2 data demonstrated that this safe and well tolerated combination results in a 48 percent lowering of LDL-C, a 26 percent reduction in high sensitivity C-reactive protein, or hsCRP, and may potentially be associated with a lower occurrence of muscle-related side effects. The bempedoic acid / ezetimibe combination pill is being developed for patients at high CVD risk with hypercholesterolemia.

### **Bempedoic Acid**

With a targeted mechanism of action, bempedoic acid is a first-in-class, complementary, orally available, once-daily ACL inhibitor that reduces cholesterol biosynthesis and lowers LDL-C by up-regulating the LDL receptor, and may potentially be associated with a lower occurrence of muscle-related side effects. Completed Phase 1 and 2 studies conducted in more than 1,300 patients and over 800 patients treated with bempedoic acid have produced clinically relevant LDL-C lowering results of up to 30 percent as monotherapy and an incremental 20+ percent when added to stable statin therapy. Bempedoic acid is being developed for patients at high CVD risk with hypercholesterolemia. We acquired the rights to bempedoic acid from Pfizer in 2008. We own the exclusive worldwide rights to bempedoic acid and we are not obligated to make any royalty or milestone payments to Pfizer.

#### ***Mechanism of Action***

In November 2016, we announced the publication of "Liver-specific ATP Citrate Lyase inhibition by bempedoic acid decreases LDL-C and attenuates atherosclerosis," by Pinkosky et al., in *Nature Communications*. The paper systematically outlines the experiments and analyses undertaken by us and our collaborators to fully understand the mechanism of action for how bempedoic acid reduces LDL-C, including its specificity for the liver. Bempedoic acid is a prodrug that once activated, inhibits ACL, an enzyme upstream of HMG-CoA reductase (the molecular target of statins) in the cholesterol synthesis pathway. Like statins, bempedoic acid decreases cholesterol synthesis in the liver, which results in decreased intracellular cholesterol, up-regulation of LDL receptor activity and increased LDL-C clearance from the blood. Although bempedoic acid and statins both inhibit cholesterol synthesis in the liver, an important differentiating feature is that, unlike statins, bempedoic acid is inactive in skeletal muscle. Specifically, bempedoic acid is a prodrug which requires activation by a specific enzyme, very long-chain acyl-CoA synthetase, or ACSVL1, to convert bempedoic acid to its CoA activated form. This enzyme is present in the liver but not in skeletal muscle. Therefore, bempedoic acid does not inhibit the cholesterol biosynthesis pathway in skeletal muscle, thus providing a mechanistic basis for reduced potential for muscle-related adverse effects. Bempedoic acid has been shown to provide incremental lowering of LDL-C when used in combination with both ezetimibe and statins at all doses.



### ***Cardiovascular Disease and Elevated LDL-C***

Cardiovascular disease, which results in heart attacks, strokes and other cardiovascular events, represents the number one cause of death and disability in western societies. The American Heart Association, or AHA, estimates that approximately 800,000 deaths in the United States were caused by cardiovascular disease in 2013.

Elevated LDL-C is well-accepted as a significant risk factor for cardiovascular disease and the CDC estimates that 78 million U.S. adults have elevated levels of LDL-C. A consequence of elevated LDL-C is atherosclerosis, which is a disease characterized by the deposition of excess cholesterol and other lipids in the walls of arteries as plaque. The development of atherosclerotic plaques often leads to cardiovascular disease. The risk relationship between elevated LDL-C and cardiovascular disease was first defined by the Framingham Heart Study, which commenced in 1948 to define factors that contributed to the development of cardiovascular disease. The study enrolled participants who did not have any form of cardiovascular disease and followed them over a long period of time. Elevated LDL-C was identified early on as key risk factor for the eventual development of cardiovascular disease.

The hypothesis that lowering elevated levels of LDL-C would translate into reduced risk of cardiovascular disease was first proven in 1984 with the publication of the Lipid Research Clinics Coronary Primary Prevention Trial. In this study, treatment with cholestyramine, a bile acid sequestrant, showed a 20% reduction in LDL-C and, importantly, a 19% reduction in risk of cardiovascular disease death or nonfatal myocardial infarction, or heart attack. This was the first major clinical study to demonstrate a direct relationship between lowering LDL-C levels and reduced risk of major cardiovascular events.

The first marketed statin, lovastatin, was approved for use in the United States in 1987 as a therapy to lower elevated LDL-C levels. That same year, the National Cholesterol Education Program issued its first guidelines for the diagnosis and treatment of patients with elevated LDL-C. Over the subsequent 22 years, seven more statins were approved for use to lower elevated LDL-C levels.

In 1994 the first clinical outcomes study with a statin was published. This study demonstrated a significant reduction in risk for total mortality and major cardiovascular events. A series of additional clinical outcomes studies with statins have each shown that lowering elevated LDL-C translated into reduced risk for major cardiovascular events. The relationship between the extent of LDL-C lowering and reduction in cardiovascular risk appeared to be linear, which has supported a hypothesis that lower LDL-C is better for cardiovascular risk. This hypothesis was tested and proven in the TNT (Treating to New Targets) study where an on-treatment LDL-C level of 77 mg/dL associated with 80 mg of atorvastatin treatment translated into a statistically significant 22% reduction in risk of major cardiovascular events as compared with the 101 mg/dL on-treatment LDL-C level associated with 10 mg of atorvastatin.

**Major completed clinical outcomes studies with statin therapies**

<b>Study name</b>	<b>4S</b>	<b>WOSCOPS</b>	<b>AFCAPS/TexCAPS</b>	<b>TNT</b>	<b>JUPITER</b>
<b>Study drug</b>	Simvastatin	Pravastatin	Lovastatin	Atorvastatin	Rosuvastatin
<b>No. of patients</b>	4,444	6,595	6,605	10,001	17,803
<b>Study design</b>	Placebo controlled, monotherapy	Placebo controlled, monotherapy	Placebo controlled, monotherapy	Low dose vs high dose atorvastatin	Placebo controlled, monotherapy
<b>Patient population</b>	Secondary prevention	Primary Prevention	Primary Prevention	Secondary Prevention	Primary Prevention
<b>Baseline LDL-C (mg/dL)</b>	188	192	156	98	108
<b>LDL-C reduction</b>	35%	26%	26%	21%	50%
<b>CV RRR</b>	35%	31%	37%	22%	44%

In November 2014, the results of the IMPROVE-IT (IMPROved Reduction of Outcomes: Vytorin Efficacy International Trial) study were presented at the Scientific Sessions of the AHA. 18,144 patients with acute coronary syndrome were enrolled in IMPROVE-IT and were randomized to receive either 40 mg of simvastatin or 10 mg of ezetimibe/40 mg of simvastatin, and were followed until > 5,250 events (cardiovascular death, heart attack, documented unstable angina requiring hospitalization, coronary revascularization or stroke) occurred. The addition of ezetimibe to simvastatin resulted in a 6.4% relative risk reduction (p=0.016) in the aggregate of the events described above. This was the first study to demonstrate incremental clinical benefit with a non-statin when added to a statin.

The direct relationship between lower LDL-C levels and reduced risk for major cardiovascular events has been consistently demonstrated in 18 clinical studies completed over the last 28 years involving more than 90,000 patients. As a result, physicians are highly focused on lowering LDL-C levels in their patients, and we believe there is a trend towards even more aggressive LDL-C lowering. For example, in the United States, increased attention has been placed on aggressive LDL-C management by organizations such as the National Cholesterol Education Program, or NCEP, the AHA and the American College of Cardiology, or ACC. Additionally, both the Canadian Cardiovascular Society and the Joint British Societies have supported even lower LDL-C treatment targets for high-risk patients. This has led to the combination of statins with other treatments, such as ezetimibe.

In July 2004, the NCEP issued an update to its Adult Treatment Panel III clinical practice guidelines on cholesterol management, advising physicians to consider new, more intensive treatment options for people at very high risk, high risk and moderately high risk for cardiovascular disease. The LDL-C goals in these updated clinical practice guidelines, which are presented below, contemplate initiating drug therapy at lower LDL-C thresholds, thus expanding the number of potential patients for LDL-C lowering therapy.

**NCEP ATP III Clinical Practice Guidelines**

<b>Patient Cardiovascular Disease Risk</b>	<b>LDL-C Goal</b>
Very High Risk	< 70 mg/dL
Cardiovascular Disease and Cardiovascular Disease Risk Equivalent	< 100 mg/dL
Multiple (2+) Risk Factors	< 130 mg/dL
0 - 1 Risk Factor	< 160 mg/dL

In November 2013, the American College of Cardiology, or ACC, and the AHA issued new guidelines for the treatment of elevated cholesterol. For the first time in more than 20 years, the guidelines did not include specific, numerical LDL-C treatment goals for patients with elevated LDL-C. However, the guidelines strongly recommend the use of more potent statins and intensive statin therapy

in patients with elevated LDL-C. The guidelines also significantly expanded the number of patients eligible for statin therapy, including patients with a history of cardiovascular disease including stroke, patients with both Type 1 and Type 2 diabetes, all patients with LDL-C  $\geq$  190 mg/dL and patients with a 10-year risk of > 7.5% of developing cardiovascular disease. Also for the first time, the guidelines acknowledge the existence of statin intolerance, and incorporate statin intolerance into the consideration of treatment choices and into the evaluation of statin safety.

Other organizations continue to utilize goals of treatment in their guidelines. The National Lipid Association, or NLA, guidelines established < 100 mg/dL as the LDL-C goal of treatment for patients at low, moderate and high risk. Patients considered to be at very high risk have a goal of < 70 mg/dL of LDL-C. The International Atherosclerosis Society has recommended optimal LDL-C levels of < 100 mg/dL for patients who have not had a cardiovascular event, and < 70 mg/dl for patients who have had a cardiovascular event. It is anticipated that the ACC and AHA will be issuing new guidelines on "the treatment of blood cholesterol to reduce atherosclerotic cardiovascular risk in adults" by the first quarter of 2019.

***Patients with HeFH and/or ASCVD who need additional lowering of LDL-C—Market Opportunity for the Bempedoic Acid / Ezetimibe Combination Pill and Bempedoic Acid***

We are pursuing development of the bempedoic acid / ezetimibe combination pill and bempedoic acid as an add-on to maximally tolerated statin therapy for patients with ASCVD and/or HeFH who require additional lowering of LDL-C. Included within the ASCVD and HeFH patient populations are patients who are only able to tolerate less than the lowest approved daily starting dose of a statin and can be considered statin intolerant. The severity of elevated LDL-C in these patients, their level of CVD risk and their therapeutic options all vary widely.

Patients with ASCVD and persistently elevated LDL-C despite maximally tolerated statin therapy represent a large population with important unmet medical needs. In a retrospective analysis of United States data, approximately one-third of high-risk patients treated with statin monotherapy for more than three months failed to achieve LDL-C target levels of < 100mg/dL, and more than three-quarters did not achieve the more stringent goal of < 70 mg/dL. It is estimated that approximately 8.6 million patients in the United States and approximately 8.4 million patients in Europe currently taking statins require additional LDL-C lowering.

Muscle pain or weakness is the most common side effect experienced by statin users and the most common cause for discontinuing therapy. Moreover, a significant proportion of patients remain on statin therapy despite experiencing muscle-related side effects, and would require additional LDL-C lowering therapies to help them achieve their LDL-C treatment goals. Accordingly, we believe that in the presence of a safe and effective complementary, non-statin, oral, once-daily, small molecule LDL-C lowering therapy, the statin intolerant market could grow substantially. Approximately 3.5 million patients in the United States and approximately 3.3 million patients in Europe are estimated to only be able to tolerate less than the lowest approved daily starting dose of their statin and are therefore considered to be statin intolerant.

**Currently Approved Therapies**

The following table illustrates common therapies used to treat elevated LDL-C:

<b>Class of Therapy</b>	<b>Labeled Indication</b>	<b>Average LDL-C Change from Baseline</b>	<b>Key Issues/Side Effects</b>
Statins	Reduction in LDL-C in patients with elevated LDL-C	Up to 63%	<ul style="list-style-type: none"> <li>Skeletal muscle effects, elevated liver function tests</li> <li>FDA recently warned that the use of statins is associated with increases in HbA1c and fasting serum glucose levels</li> </ul>
	Reduction in total mortality		
Bile acid sequestrants	Reduction in risk of major adverse cardiovascular events (MACE) in multiple populations that were tested	Up to 20%	<ul style="list-style-type: none"> <li>Limited LDL-C lowering</li> <li>Gastrointestinal disorders</li> <li>Elevation in triglycerides</li> </ul>
	Reduction in LDL-C in patients with elevated LDL-C (1)		
Cholesterol absorption inhibitors	Retard the rate of progression and increase the rate of regression of coronary atherosclerosis	Up to 18%	<ul style="list-style-type: none"> <li>Limited LDL-C lowering; IMPROVE-IT study not in US prescribing information</li> </ul>
	Reduction in LDL-C in patients with elevated LDL-C		
Niacin	Reduction in LDL-C and triglycerides; increases in HDL-C, reduction in Lipoprotein (a)	Up to 17%	<ul style="list-style-type: none"> <li>Flushing (i.e., warmth or redness) hepatic toxicity, skeletal muscle effects and gout</li> <li>Limited LDL-C lowering</li> </ul>
	Reduction in recurrent nonfatal myocardial infarction (MI) in patients with prior history of MI		
Fibrates	Reduction in triglycerides and LDL-C in patients with hypertriglyceridemia or mixed dyslipidemia	Up to 21%	<ul style="list-style-type: none"> <li>Gallstones, skeletal muscle effects and liver disorders</li> <li>Limited LDL-C lowering (may in some cases raise LDL-C); used primarily for triglyceride lowering</li> </ul>
	Reduction in risk of developing coronary heart disease (CHD) in patients with Type IIb Fredericksons hyperlipidemia and no prior history of CHD		
Proprotein convertase subtilisin kexin 9 (PCSK9) inhibitors	Alirocumab: Reduction in LDL-C as adjunct to maximally tolerated statin therapy in patients with HeFH and/or ASCVD	Up to 54% (monotherapy)	<ul style="list-style-type: none"> <li>High cost as biologic, injectable route of administration</li> <li>No effect on hsCRP</li> <li>Ongoing CVOT</li> </ul>
	Evolocumab: Reduction in risk of myocardial infarction, stroke, and coronary revascularization in adults with established cardiovascular disease; Reduction in LDL-C alone or in combination with other lipid-lowering therapies for adults with primary hyperlipidemia		

(1) Welchol®, a bile acid sequestrant, is also approved for improving glycemic control in adults with Type 2 diabetes.

## Other Approved Therapies for Specific Populations

A small subpopulation of patients with extremely elevated levels of LDL-C, estimated to be approximately 900 patients in the U.S., suffer from homozygous familial hypercholesterolemia, or HoFH. HoFH is a serious and rare genetic disease and patients with HoFH lack or have dysfunctional LDL-receptors and cannot remove LDL-particles and LDL-C from the blood. As a result, untreated HoFH patients typically have LDL-C levels in the range of 450 mg/dL to 1,000 mg/dL. Microsomal triglyceride transfer protein, or MTP inhibitors, a PCSK9 inhibitor and an apolipoprotein B, or ApoB, antisense oligonucleotide are approved therapies to lower elevated LDL-C levels in patients with a clinical or laboratory diagnosis of HoFH. Given the serious safety concerns with the MTP inhibitor and ApoB antisense oligonucleotide, specifically hepatotoxicity, the FDA has restricted their usage to this narrow subpopulation.

### Statin Therapy

Statins are the standard of care for patients with hypercholesterolemia today and are highly effective at lowering LDL-C. This class of drugs includes atorvastatin calcium, marketed as Lipitor®, the most prescribed LDL-C lowering drug in the world.

Statins are selective, competitive inhibitors of HMG-CoA reductase, a rate-limiting enzyme in the cholesterol biosynthesis pathway in liver cells. Statin inhibition of cholesterol synthesis increases the number of LDL receptors on the surface of liver cells. This increase in LDL receptors increases uptake of LDL particles into liver cells from the blood, thus lowering LDL-C levels. Statins are also thought to have a potential effect on cholesterol synthesis in skeletal muscle. This effect could be linked to the myalgia associated with statin use as seen in certain patients with statin intolerance.

The benefits of statin use in lowering LDL-C levels and improving cardiovascular outcomes are well documented. Despite the effectiveness of statins and their broad market acceptance, there is a significant subset of patients who are unable to tolerate statins due to muscle pain or weakness, memory loss or increased glucose levels, or who are otherwise unable to reach their LDL-C goal on statin therapy alone. In rare but extreme cases, statins can lead to muscle breakdown, kidney failure and death. In addition, the FDA has recently warned that statins can cause hyperglycemia, an increase in blood sugar levels and create an increased risk of worsening of glycemic control and of new onset diabetes. There are approximately 36 million U.S. adults with elevated LDL-C levels who are not on an LDL-C lowering therapy. For these reasons, we believe there is a need for new therapies to treat patients with elevated LDL-C.

### Approved Therapies

#### *PCSK9 Inhibitors*

Proprotein convertase subtilisin kexin type 9, or PCSK9, inhibitors, an enzyme involved in the degradation of LDL receptors, are injectable, monoclonal antibodies to lower LDL-C. In 2015 the FDA approved two PCSK9 inhibitors: alirocumab, which was developed by Sanofi and Regeneron Pharmaceuticals, and evolocumab, which was developed by Amgen, Inc. These therapies were originally approved as an adjunct to diet and maximally tolerated statin therapy for patients with HeFH and/or ASCVD that require additional lowering of LDL-C. Additionally, evolocumab was approved as an adjunct to diet and other LDL-C lowering therapies for patients with HoFH. In 2016, Pfizer discontinued development of its PCSK9 inhibitor, bococizumab, due to unanticipated attenuation of LDL-C lowering over time in its Phase 3 studies. In February 2017, Amgen announced top-line results for the FOURIER ( **F**urther Cardiovascular **OU**tcomes **R**esearch with PCSK9 **I**nhibition in Subjects with **E**levated **R**isk) CVOT where evolocumab significantly reduced the risk of cardiovascular events. Full results of FOURIER were presented at the Scientific Sessions of the American College of Cardiology in March 2017, and were published in the New England Journal of Medicine in March 2017. In

December 2017, based upon the results of the FOURIER study, the indications for the use of evolocumab were updated to include reduction in risk of myocardial infarction, stroke and coronary revascularization in adults with established cardiovascular disease, and for use alone or in combination with other lipid-lowering therapies to reduce LDL-C in adults with primary hyperlipidemia.

It is expected that the top-line and full results of the ODYSSEY Outcomes CVOT, a cardiovascular outcomes study being conducted by Sanofi and Regeneron to evaluate whether alirocumab reduces the risk of cardiovascular disease, will be announced in the first quarter of 2018.

As described in currently approved U.S. prescribing information, PCSK9 inhibitors have demonstrated reductions of LDL-C when added on to maximally tolerated statin therapy in patients with HeFH and/or ASCVD of up to 64%. When PCSK9 inhibitors were used in patients with hypercholesterolemia considered to be statin intolerant, LDL-C levels were reduced by 45-56%. On December 1, 2017, it was announced that, based on the results of FOURIER, the U.S. prescribing information for evolocumab now includes an indication for the reduction in risk of myocardial infarction, stroke and coronary revascularization in patients with established cardiovascular disease. In addition, evolocumab is indicated for use alone or in combination with other lipid-lowering agents for patient with primary hyperlipidemia, including familial and nonfamilial hypercholesterolemia. Notwithstanding the LDL-C lowering efficacy of PCSK9 inhibitors, we believe their adoption by patients, physicians, and payors could be adversely impacted by their higher cost and their injectable route of administration.

## **Additional Therapies in Development**

### ***PCSK9 Inhibitors***

The Medicines Company/Alnylam are developing inclisiran, which is currently in Phase 3 clinical studies of eighteen months in length. Unlike the PCSK9 antibodies from Sanofi/Regeneron and Amgen, inclisiran is a long-acting RNA interference therapeutic agent that inhibits the synthesis of PCSK9. Findings from clinical studies suggest that inclisiran may be dosed every 6 months, with a 3 month timeframe only between first and second dose. Like the PCSK9 antibodies, inclisiran is an injectable therapy.

## **Clinical Experience**

To date, bempedoic acid has been studied in approximately 800 patients across multiple hypercholesterolemia patient populations: patients with elevated LDL-C levels; patients with Type 2 diabetes and elevated LDL-C levels; patients with elevated LDL-C levels and a history of statin intolerance; patients with elevated LDL-C levels taking low, moderate and high doses of the most commonly prescribed statins; and patients with both elevated LDL-C and hypertension. The individual design and results of each of the completed Phase 2 clinical studies of bempedoic acid are summarized below.

**Completed Clinical Studies**

To date, we have completed the following Phase 2 clinical studies of bempedoic acid:

<u>Description</u>	<u>Title</u>	<u>Treatment Duration</u>	<u>Subjects</u>	
			<u>Total</u>	<u>Treated</u>
1002-038	<b>Phase 2 clinical efficacy and safety study of the bempedoic acid / ezetimibe combination plus atorvastatin in patients with hypercholesterolemia</b>  A randomized, double-blind, placebo-controlled study that evaluated 180 mg of bempedoic acid, 10 mg of ezetimibe, and 20 mg of atorvastatin in patients with hypercholesterolemia	6 weeks	63	43
1002-035	<b>Phase 2 PK/PD clinical study in patients treated with high-dose statin therapy</b>  A randomized, double-blind, multi-center, placebo-controlled, parallel group clinical study that evaluated 180 mg of bempedoic acid versus placebo in patients already on stable 80 mg atorvastatin therapy	4 weeks	68	45
1002-014	<b>Phase 2 exploratory clinical safety study in patients with both elevated LDL-C and hypertension</b>  A randomized, double-blind, multi-center, placebo-controlled, parallel group exploratory study that evaluated 180 mg of bempedoic acid versus placebo in patients with both elevated LDL-C and hypertension	6 weeks	143	71
1002-009	<b>Phase 2 clinical study in patients with elevated LDL-C already receiving statin therapy</b>  A randomized, double-blind, multi-center placebo-controlled clinical study that evaluated 180 mg and 120 mg of bempedoic acid versus placebo in patients already on stable statin therapy	12 weeks	134	88
1002-008	<b>Phase 2 clinical study of safety and efficacy in patients with elevated LDL-C, with or without a history of statin intolerance</b>  A randomized, double-blind, parallel-group, multicenter study to evaluate the efficacy and safety of bempedoic acid monotherapy, ezetimibe monotherapy, and the combination of bempedoic acid and ezetimibe in patients with elevated LDL-C, with or without statin intolerance	12 Weeks	349	249

Description	Title	Treatment Duration	Subjects	
			Total	Treated
1002-007	<b>Phase 2 clinical study of safety and pharmacokinetic interaction in patients with elevated LDL-C on a background of atorvastatin 10 mg</b>	8 Weeks	58	42
	Placebo-controlled, randomized, double-blind, drug interaction study to evaluate the safety, tolerability and effect on atorvastatin pharmacokinetics of bempedoic acid added to atorvastatin 10 mg/day in patients with elevated LDL-C			
1002-006	<b>Phase 2 proof-of-concept clinical study in patients with elevated LDL-C and a history of statin intolerance</b>	8 Weeks	56	37
	Placebo-controlled, randomized, double-blind, multicenter study to evaluate the efficacy and safety of bempedoic acid in patients with elevated LDL-C and a history of statin intolerance			
1002-005	<b>Phase 2 proof-of-concept clinical study in patients with elevated-LDL-C and Type 2 diabetes</b>	4 Weeks	60	30
	Placebo-controlled, randomized, double-blind, single site clinical study to evaluate the LDL-C lowering efficacy and safety of bempedoic acid in patients with Type 2 diabetes			
1002-003	<b>Phase 2 proof-of-concept clinical study in patients with elevated LDL-C</b>	12 Weeks	177	133
	Placebo-controlled, randomized, double-blind, parallel group, multicenter clinical study to evaluate the LDL-C lowering efficacy and safety of bempedoic acid in patients with elevated LDL-C and either normal or elevated triglycerides			

Overall, bempedoic acid has been well-tolerated and associated with no dose-limiting adverse events, or AEs, in approximately 800 patients who received bempedoic acid.

**Phase 2 Clinical Studies Completed in 2017**

*1002-038—Phase 2 efficacy and safety study of the bempedoic acid / ezetimibe combination plus atorvastatin in patients with hypercholesterolemia*

On August 8, 2017, we announced top-line results from the Phase 2 clinical study (1002-038), also known as the triplet oral therapy study. The six-week, Phase 2, randomized, double-blind, placebo-controlled study evaluated the efficacy and safety of bempedoic acid 180 mg, ezetimibe 10 mg and atorvastatin 20 mg (the "bempedoic acid / ezetimibe combination plus atorvastatin", or "Combo + Statin"), versus placebo, in patients with hypercholesterolemia. The primary objective of the study was to assess the LDL-C lowering efficacy of the bempedoic acid / ezetimibe combination plus atorvastatin versus placebo. Secondary objectives included assessing the percent of treated patients achieving a reduction in LDL-C levels of  $\geq 50\%$ , the percent of treated patients reaching LDL-C levels of  $< 70$  mg/d, assessment of the effect of the bempedoic acid / ezetimibe combination plus



atorvastatin therapy on additional lipid and cardiometabolic risk markers, including total cholesterol, apoB, non-high-density lipoprotein-cholesterol, or non-HDL-C, and hsCRP, and assessment of the safety and tolerability of the bempedoic acid / ezetimibe combination plus atorvastatin therapy, including muscle-related adverse events, or AEs. Prior to randomization, patients were washed out of all lipid-lowering therapies for six weeks. 43 patients received the bempedoic acid / ezetimibe combination plus atorvastatin and 20 patients received placebo. While analyses of the complete efficacy and safety results from 100-038 are ongoing, the top-line results are summarized as follows:

#### LDL-Cholesterol Percent Change from Baseline to Week 6 Endpoint

Treatment Group	Number of Patients	LDL-C Baseline Mean (SD) mg/dL	LDL-C Week 6 Endpoint Mean (SD) mg/dL	Percent Change from Baseline	
				LS Mean (SE)	P Value
Combo + Statin	41	154 (18)	56 (17)	-64% (1.7)	<0.001
Placebo	20	156 (14)	152 (27)	-3% (3.34)	—

LS = least squares; SD = standard deviation; SE = standard error; mITT population

#### hsCRP Nonparametric Analysis

Treatment Group	Number of Patients	Baseline Level (mg/L)	Percent Change from Baseline	
			Median Change	P Value
Combo + Statin	41	1.94	-48%	<0.001
Placebo	19	1.64	-3%	—

mITT population

- After six weeks of treatment with the bempedoic acid / ezetimibe combination plus atorvastatin, the primary endpoint of the study, LDL-C levels were lowered by 64% ( $p < 0.001$ ), with an average reduction of 3% for patients dosed with placebo. The maximal effect on LDL-C lowering was seen at 3 weeks into the study.
- 95% of patients treated with the bempedoic acid / ezetimibe combination plus atorvastatin achieved an LDL-C reduction of  $\geq 50\%$ . 90% of the treated patients with the bempedoic acid / ezetimibe combination plus atorvastatin achieved an LDL-C level of  $< 70$  mg/dL.
- hsCRP, a marker of the underlying inflammation associated with CVD, was reduced by 48% ( $p < 0.001 = 0.26$ ) for patients dosed with the bempedoic acid / ezetimibe combination plus atorvastatin after six weeks of therapy, versus a 3% reduction with placebo.
- Clinically significant reductions in total cholesterol, apoB and non-HDL-C were seen in the patients treated with the bempedoic acid / ezetimibe combination plus atorvastatin.
- Discontinuation rates for the bempedoic acid / ezetimibe combination plus atorvastatin were low and comparable to placebo. There were no increases (repeated and confirmed) in liver function tests or levels of creatine kinase, or CK, an enzyme associated with muscle damage. Elevations in liver function tests and CK have been observed with use of statins.

#### Overall Safety Observations

To date, in completed studies, approximately 800 patients have been treated with bempedoic acid for periods of up to 12 weeks at maximum repeated doses of 240 mg per day. Bempedoic acid has been

safe and well-tolerated with no dose-limiting side effects identified to date in our ongoing or completed clinical studies. No clinical safety trends have emerged to date.

Study	Phase	Patient Population	Study Design	Duration	Patients (Treated)	Doses	LDL Lowering Efficacy (placebo corrected)
1002-038	Phase 2	Elevated LDL-C	Placebo controlled	6 weeks	63 (43)	180 mg bempedoic acid, 10 mg ezetimibe, 20 mg atorvastatin	Up to 64%
1002-035	Phase 2	Elevated LDL-C	Placebo controlled, 80 mg atorvastatin	4 weeks	68 (45)	180 mg	Up to 22%
1002-014	Phase 2	Elevated LDL-C; hypertension	Placebo controlled	6 weeks	143 (71)	180mg	Up to 24%
1002-009	Phase 2	Elevated LDL-C; statin add-on	Placebo controlled,	12 weeks	134 (89)	120mg, 180mg	Up to 20%
1002-008	Phase 2	Elevated LDL-C; statin intolerant and tolerant	Monotherapy and in combination with ezetimibe	12 weeks	349 (250)	120 mg, 180 mg	Up to 30% Up to 48%
1002-007	Phase 2	Elevated LDL-C; statin add-on	Placebo controlled, 10 mg atorvastatin	8 weeks	58 (42)	60, 120, 180, 240 mg	Up to 22%
1002-006	Phase 2	Elevated LDL-C; statin intolerant	Placebo controlled	8 weeks	56 (37)	60, 120, 180, 240 mg	Up to 29%
1002-005	Phase 2	Elevated LDL-C; T2DM	Placebo controlled	4 weeks	60 (30)	80, 120 mg	Up to 39%
1002-004	Phase 1	Healthy subjects	Multiple ascending dose, PK	2 weeks	24 (18)	40, 180, 220 mg	Up to 36%
1002-003	Phase 2	Elevated LDL-C	Placebo controlled	12 weeks	177 (133)	40, 80, 120 mg	Up to 25%
1002-002	Phase 1	Healthy subjects	Multiple ascending dose, PK/PD	2/4 weeks	53 (39)	20, 60, 100, 120 mg	Up to 17%
1002-001	Phase 1	Healthy subjects	Single dose, PK	Single dose	18 (18)	2.5, 10, 45, 125, 250 mg	Not applicable

## Ongoing Clinical Studies

### *1002FDC-053—Phase 3 efficacy and safety study of the bempedoic acid / ezetimibe combination pill in patients with hypercholesterolemia*

On November 6, 2017, we announced the initiation of the pivotal Phase 3 clinical study to assess the efficacy and safety of the bempedoic acid / ezetimibe combination pill in patients with hypercholesterolemia and ASCVD and/or HeFH, including high CVD risk primary prevention patients, whose LDL-C is not adequately controlled despite receiving maximally tolerated lipid-modifying background therapy. The 12-week, pivotal Phase 3 randomized, double-blind, placebo-controlled, parallel-dose study consists of four treatment arms evaluating the efficacy and safety of a once-daily, oral, fixed dose combination pill of 180 mg of bempedoic acid and 10 mg of ezetimibe versus placebo, 180 mg of bempedoic acid alone and 10 mg of ezetimibe alone. The study is expected to enroll approximately 350 patients at up to 125 U.S. sites. The co-primary objectives of the study are to assess LDL-C lowering efficacy in patients treated with the bempedoic acid / ezetimibe combination pill versus placebo, 180 mg of bempedoic acid and 10 mg of ezetimibe alone. Secondary objectives include assessing the safety and tolerability of the bempedoic acid / ezetimibe combination pill versus placebo, 180 mg of bempedoic acid and 10 mg of ezetimibe alone and effects on other risk markers, including hsCRP, non-HDL-C, apoB and total cholesterol. We expect to report top-line results in August 2018.

### *Study 1—Global pivotal Phase 3 long-term safety and tolerability study in patients with hypercholesterolemia on maximally tolerated background lipid-modifying therapy*

Study 1 is a 52-week global pivotal Phase 3 randomized, double-blind, placebo-controlled study evaluating the long-term safety and tolerability of bempedoic acid 180 mg versus placebo in high CVD risk patients with hypercholesterolemia and with ASCVD and/or HeFH whose LDL-C is not adequately controlled with current lipid-modifying therapies, and who are taking maximally tolerated statin therapy. The study enrolled 2,230 patients at approximately 100 sites in the U.S., Canada and Europe. The primary objective is to assess safety and tolerability of patients treated with bempedoic acid for 52 weeks. Secondary objectives include assessing the LDL-C lowering efficacy of bempedoic

acid on top of maximally tolerated statin and other lipid-modifying therapies at 12, 24 and 52 weeks versus placebo. Effects on other risk markers, including non-high-density lipoprotein, or non-HDL-C, total cholesterol, apolipoprotein B, or apoB, and hsCRP, will also be evaluated. We expect to report top-line results in May 2018.

Additional safety data will be obtained from an open-label extension study which will enroll approximately 1,400 of the 2,230 patients enrolled in Study 1. Initiated in February 2017, this open-label extension study will evaluate the long-term safety of bempedoic acid 180 mg versus placebo in high CVD risk patients with hypercholesterolemia and with ASCVD and/or HeFH whose LDL-C is not adequately controlled with current lipid-modifying therapies, and who are taking maximally tolerated statin therapy. This open-label extension study will be conducted at approximately 100 sites included in the parent study in the U.S., Canada and Europe. The primary objective is to assess the long-term safety in patients treated with bempedoic acid for up to 1.5 years. Secondary objectives include evaluating the 52- and 78-week effects of bempedoic acid on lipid and cardiometabolic risk markers, including LDL-C, non-HDL-C, total cholesterol, apoB and hsCRP.

***Study 2—Global pivotal Phase 3 LDL-C lowering efficacy and safety study in patients with hypercholesterolemia not adequately controlled with current lipid-modifying therapy***

Study 2 is a 52-week global pivotal Phase 3 randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of bempedoic acid 180 mg versus placebo in high CVD risk patients with hypercholesterolemia with ASCVD and/or HeFH, whose LDL-C is not adequately controlled with current lipid-modifying therapies, and who are taking maximally tolerated statin therapy. This study enrolled 779 patients at approximately 125 sites in the U.S., Canada and Europe. The primary objective is to assess the 12-week LDL-C lowering efficacy of patients treated with bempedoic acid versus placebo. Secondary objectives include evaluating the 24-week LDL-C lowering efficacy, and 52-week safety and tolerability of bempedoic acid versus placebo. Effects on other risk markers, including non-HDL-C, total cholesterol, apoB, and hsCRP, will also be evaluated. We expect to report top-line results in September 2018.

***Study 3—Global pivotal Phase 3 LDL-C lowering efficacy and safety study in patients with hypercholesterolemia not adequately controlled with current lipid-modifying therapy, including patients considered statin intolerant***

Study 3 is a 24-week global pivotal Phase 3 randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of bempedoic acid 180 mg versus placebo in high CVD risk patients with ASCVD and/or HeFH, or who are high risk primary prevention, whose LDL-C is not adequately controlled with current lipid-modifying therapies, and who are only able to tolerate less than the lowest approved daily starting dose of a statin and can be considered statin intolerant. This study enrolled 345 patients at approximately 70 sites in the U.S. and Canada. The primary objective is to assess the 12-week LDL-C lowering efficacy of patients treated with bempedoic acid versus placebo. Secondary objectives include evaluating the 24-week LDL-C lowering efficacy, safety and tolerability of bempedoic acid versus placebo and effects on other risk markers, including non-HDL-C, total cholesterol, apoB and hsCRP. We expect to report top-line results in May 2018.

***Study 4—Global pivotal Phase 3 LDL-C lowering efficacy and safety study in patients with hypercholesterolemia not adequately controlled with current lipid-modifying therapy, including ezetimibe, and patients considered statin intolerant***

Study 4 is a 12-week global pivotal Phase 3 randomized, double-blind, placebo-controlled study evaluating the efficacy and safety of bempedoic acid 180 mg versus placebo as an add-on to ezetimibe 10 mg in high CVD risk patients with ASCVD and/or HeFH, whose LDL-C is not adequately controlled with current lipid-modifying therapies, including ezetimibe, and who are only able to tolerate

the lowest or less than the lowest approved daily starting dose of a statin and can be considered statin intolerant. This study enrolled 269 patients at approximately 75 sites in the U.S., Canada and Europe. The primary objective is to assess the 12-week LDL-C lowering efficacy of patients treated with bempedoic acid versus placebo when added to ezetimibe. Secondary objectives include evaluating safety and tolerability of bempedoic acid when added to ezetimibe, and effects on other risk markers, including non-HDL-C, total cholesterol, apoB and hsCRP. We expect to report top-line results in March 2018.

***Global Cardiovascular Outcomes Trial—CLEAR Outcomes***

CLEAR Outcomes is an event driven, global, randomized, double-blind, placebo-controlled study to assess the effects of bempedoic acid in patients with ASCVD and/or HeFH, or who are at high risk for CVD, with hypercholesterolemia and who are only able to tolerate less than the lowest approved daily starting dose of a statin and can be considered statin intolerant. The CLEAR Outcomes CVOT is expected to enroll approximately 12,600 patients with ASCVD or at high risk for CVD in up to 1,000 sites in approximately 30 countries. The study is expected to enroll over a 30 month period with a total estimated study duration of approximately 4.75 years. The expected average treatment duration will be 3.75 years with a minimum treatment duration of approximately 2.25 years. Patients enrolling in the study will be required to have a history of, or be at high risk for, CVD with LDL-C levels greater than 100 mg/dL despite background lipid-lowering therapy, resulting in an expected average baseline LDL-C level in all patients of approximately 135 mg/dL. The primary efficacy endpoint of the event-driven global study is the effect of bempedoic acid versus placebo on the risk of major adverse cardiovascular events (cardiovascular death, non-fatal myocardial infarction, non-fatal stroke, or coronary revascularization; also referred to as "four-component MACE"). We initiated CLEAR Outcomes in December 2016, and the study is intended to support our submissions for a CV risk reduction indication in the U.S. and Europe by 2022.

***1002-039—Phase 2 efficacy and safety study of bempedoic acid when added-on to an injectable proprotein convertase subtilisin/kexin type 9 inhibitor in patients with hypercholesterolemia***

On July 26, 2017, we announced the initiation of the Phase 2 clinical study to assess the efficacy and safety of bempedoic acid when added-on to an injectable PCSK9i therapy. The eight-week, Phase 2, randomized, double-blind, placebo-controlled study will evaluate the efficacy and safety of once-daily, oral bempedoic acid 180 mg and once-monthly injection of Repatha® (evolocumab) 420 mg versus placebo and once-monthly injection of Repatha® 420 mg. The study enrolled 59 patients with hypercholesterolemia at approximately 20 sites across the U.S. The primary objective of the study is to assess the incremental LDL-C lowering efficacy of bempedoic acid versus placebo in patients receiving PCSK9i therapy. Secondary objectives include assessing the safety and tolerability of bempedoic acid versus placebo in patients on PCSK9i therapy and effects on other risk markers, including non-HDL-C, total cholesterol, apoB and hsCRP. This non-registrational study will assess the incremental LDL-C lowering efficacy and continued safety and tolerability of a once-daily, oral bempedoic acid pill added-on to an injectable biologic therapy in patients with elevated LDL-C levels. We expect to report top-line results in March 2018.

**Research and Development Expenses**

Research and development expenses for the year ended December 31, 2017, were \$147.6 million.

## **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 partnership or co-promotion arrangements with an established pharmaceutical company. To develop the appropriate commercial infrastructure to launch the bempedoic acid / ezetimibe combination pill and bempedoic acid in the United States, if approved, as a treatment for patients with elevated LDL-C, we would need to invest significant financial and managerial resources. We engage in partnering discussions with third parties from time to time. If we elect to seek approval and launch commercial sales of the bempedoic acid / ezetimibe combination pill and bempedoic acid outside of the United States or for broader patient populations in the United States, including patients who are unable to reach their LDL-C goal with a statin therapy, we may either do so on our own or by establishing alliances with one or more pharmaceutical company collaborators, depending on, among other things, the applicable indications, the related development costs and our available resources.

## **Manufacturing and Supply**

Bempedoic acid is a small molecule drug that is synthesized from readily available raw materials using conventional chemical processes. We currently have no manufacturing facilities and limited personnel with manufacturing experience. We rely on contract manufacturers to produce both drug substances and drug products required for our clinical studies. All lots of drug substance and drug product used in clinical studies are manufactured under current good manufacturing practices. We plan to continue to rely upon contract manufacturers and, potentially, collaboration partners to manufacture commercial quantities of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved.

## **Licenses**

In April 2008, we entered into an asset transfer agreement with Pfizer pursuant to which we acquired all intellectual property owned by Pfizer relating exclusively to the bempedoic acid program. We also entered into a license agreement providing a worldwide, exclusive, fully paid-up license of certain residual background intellectual property not transferred pursuant to the asset transfer agreement, and we granted Pfizer a worldwide, exclusive, fully paid-up license to certain patent rights owned or controlled by us relating to development programs other than bempedoic acid. The license to us covers the development, manufacturing and commercialization of bempedoic acid. There are no restrictions or limitations and we may grant sublicenses under the license agreements. Pfizer is not entitled to any royalties, milestones or any similar development or commercialization payments under the terms of the agreements, and the licenses granted are irrevocable and may not be terminated for any cause, including intentional breaches or breaches caused by gross negligence.

## **Intellectual Property**

We strive to protect and enhance the proprietary technologies that we believe are important to our business, including seeking and maintaining patents intended to cover our products and compositions, their methods of use and any other inventions that are important to the development of our business. 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.

Our success will depend significantly on our ability to obtain and maintain patent and other proprietary protection for commercially important technology, inventions and know-how related to our business, defend and enforce our patents, preserve the confidentiality of our trade secrets and operate without infringing the valid and enforceable patents and proprietary rights of third parties. We also rely on know-how, continuing technological innovation and in-licensing opportunities to develop, strengthen

and maintain the proprietary position of the bempedoic acid / ezetimibe combination pill, bempedoic acid and our other development programs.

As of December 31, 2017, our patent estate, including patents we own, on a worldwide basis, included approximately 23 issued United States patents and 6 pending United States patent applications and 18 issued patents and 40 pending patent applications in other foreign jurisdictions. Of our worldwide patents and pending applications, only a subset relates to our small molecule program. Bempedoic acid is claimed in U.S. Patent No. 7,335,799 that is scheduled to expire in December 2025, which includes 711 days of patent term adjustment, and may be eligible for a patent term extension period of up to five years. U.S. Patent Nos. 9,000,041, 8,497,301 and 9,624,152 claim methods of using bempedoic acid. We also have a pending U.S. patent application directed to bempedoic acid. There are currently six issued patents and one pending application in countries outside the United States that relate to bempedoic acid.

A subset of this portfolio relates to our bempedoic acid / ezetimibe combination pill and bempedoic acid and one or more statins. We have 17 pending applications outside the U.S., and one inside the U.S., with claims directed to methods of treatment using the bempedoic acid / ezetimibe combination. Additionally, we own one pending U.S. application directed to the manufacturing of our bempedoic acid / ezetimibe combination pill. We also have 17 pending application outside the United States, and one inside the U.S., with claims directed to methods of treatment using a fixed dose combination of bempedoic acid and one or more statins.

In addition to the patents we own, we also hold an exclusive, worldwide, fully paid-up license on any residual background intellectual property not transferred from Pfizer in the asset transfer.

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 date of filing the non-provisional application. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. In addition, in certain instances, a patent term can be extended to recapture a portion of the term effectively lost as a result of the FDA regulatory review period. However, the restoration period cannot be longer than five years and the total patent term including the restoration period must not exceed 14 years following FDA approval. The duration of foreign patents varies in accordance with provisions of applicable local law, but typically is also twenty years from the earliest effective filing date. Our issued U.S. patents will expire on dates ranging from 2021 to 2030. However, the actual protection afforded by a patent varies on a claim by claim basis for each applicable product, from country to country and depends upon many factors, including the type of patent, the scope of its coverage, the availability of regulatory related extensions, the availability of legal remedies in a particular country and the validity and enforceability of the patent.

Furthermore, the patent positions of biotechnology and pharmaceutical products and processes like those we intend to develop and commercialize are generally uncertain and involve complex legal and factual questions. No consistent policy regarding the breadth of claims allowed in such patents has emerged to date in the U.S. The patent situation outside the U.S. is even more uncertain. Changes in either the patent laws or in interpretations of patent laws in the U.S. and other countries can diminish our ability to protect our inventions, and enforce our intellectual property rights and more generally, could affect the value of intellectual property. Accordingly, we cannot predict the breadth of claims that may be allowed or enforced in our patents or in third-party patents.

The biotechnology and pharmaceutical industries are characterized by extensive litigation regarding patents and other intellectual property rights. Our ability to maintain and solidify our proprietary position for our drugs and technology will depend on our success in obtaining effective claims and enforcing those claims once granted. We do not know whether any of the patent applications that we

may file or license from third parties will result in the issuance of any patents. The issued patents that we own or may receive in the future, may be challenged, invalidated or circumvented, and the rights granted under any issued patents may not provide us with proprietary protection or competitive advantages against competitors with similar technology. Furthermore, our competitors may be able to independently develop and commercialize similar drugs or duplicate our technology, business model or strategy without infringing our patents. Because of the extensive time required for clinical development and regulatory review of a drug we may develop, it is possible that, before any of our drugs can be commercialized, any related patent may expire or remain in force for only a short period following commercialization, thereby reducing any advantage of any such patent.

As a result of the America Invents Act of 2011, the United States transitioned to a first-inventor-to-file system in March 2013, under which, assuming the other requirements for patentability are met, the first inventor to file a patent application will be entitled to the patent. This will require us to minimize the time from invention to the filing of a patent application.

We may rely, in some circumstances, on trade secrets and unpatented know-how to protect our technology. However, trade secrets can be difficult to protect. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our consultants, scientific advisors and contractors and invention assignment agreements with our employees. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached and we may not have adequate remedies for any breach. In addition, our trade secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions. For more information, please see "Risk Factors—Risks Related to our Intellectual Property."

Our commercial success will also depend in part on not infringing the proprietary rights of third parties. It is uncertain whether the issuance of any third-party patent would require us to alter our development or commercial strategies, or our drugs or processes, obtain licenses or cease certain activities. Our breach of any license agreements or failure to obtain a license to proprietary rights that we may require to develop or commercialize our future drugs may have a material adverse impact on us. If third parties prepare and file patent applications in the U.S. that also claim technology to which we have rights, we may have to participate in interference proceedings in the U.S. Patent and Trademark Office, or USPTO, to determine priority of invention.

In addition, substantial scientific and commercial research has been conducted for many years in the areas in which we have focused our development efforts, which has resulted in third parties having a number of issued patents and pending patent applications. Patent applications in the U.S. and elsewhere are published only after eighteen months from the priority date. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made. Therefore, patent applications relating to drugs similar to bempedoic acid and any future drugs, discoveries or technologies we might develop may have already been filed by others without our knowledge.

## **Competition**

Our industry is highly competitive and subject to rapid and significant technological change. Our potential competitors include large pharmaceutical and biotechnology companies, specialty pharmaceutical and generic drug companies, academic institutions, government agencies and research institutions. Key competitive factors affecting the commercial success of our product candidates are

likely to be efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement.

The market for cholesterol regulating therapies is especially large and competitive. The product candidates we are currently developing, if approved, will face intense competition, either as monotherapies or as combination therapies.

Many of our existing or potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Mergers and acquisitions in the pharmaceutical and biotechnology industries may result in even more resources being concentrated among a small number of our competitors. Accordingly, our competitors may be more successful than we may be in obtaining FDA approval for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective, or more effectively marketed and sold, than any drug we may commercialize and may render our product candidates obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our product candidates. Our competitors may also obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours. We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. Finally, the development of new treatment methods for the diseases we are targeting could render our drugs non-competitive or obsolete. See "Risk Factors—Risks Related to our Business and the Clinical Development and Commercialization of Our Product Candidates—Our market is subject to intense competition. If we are unable to compete effectively, our opportunity to generate revenue from the sale of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, will be materially adversely affected."

## **Regulatory Matters**

### ***Government Regulation and Product Approval***

Government authorities in the United States at the federal, state and local level, and other countries, extensively regulate, among other things, the research, development, testing, manufacture, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, marketing, export and import of products such as those we are developing. Our product candidates, including the bempedoic acid / ezetimibe combination pill and bempedoic acid, must be approved by the FDA through the NDA process before they may legally be marketed in the United States.

### ***United States Drug Development Process***

In the United States, the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, or FDCA, and implementing regulations. The process of obtaining regulatory approvals and compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process, or after approval, may subject an applicant to administrative or judicial sanctions. These sanctions could include the FDA's refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. The process required by the FDA before a drug may be marketed in the United States generally involves the following:

- completion of nonclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices regulations;



- submission to the FDA of an IND, which must become effective before human clinical studies may begin;
- performance of adequate and well-controlled human clinical studies according to Good Clinical Practices, or GCP, to establish the safety and efficacy of the proposed drug for its intended use;
- submission to the FDA of an NDA for a new drug;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with cGMP; and
- FDA review and approval of the NDA.

The testing and approval process requires substantial time, effort and financial resources and we cannot be certain that any approvals for our product candidates will be granted on a timely basis, if at all.

Once a pharmaceutical product candidate is identified for development, it enters the nonclinical, also referred to as preclinical, testing stage. Nonclinical tests include laboratory evaluations of product chemistry, toxicity, formulation and stability, as well as animal studies. An IND sponsor must submit the results of the nonclinical tests, together with manufacturing information, analytical data and any available clinical data or literature, to the FDA as part of the IND. The sponsor must also include a protocol detailing, among other things, the objectives of the initial clinical study, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated if the initial clinical study lends itself to an efficacy evaluation. Some nonclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA places the clinical study on a clinical hold within that 30-day time period. In such a case, the IND sponsor and the FDA must resolve any outstanding concerns before the clinical study can begin. Clinical holds also may be imposed by the FDA at any time before or during clinical studies due to safety concerns or non-compliance, and may be imposed on all drug products within a certain class of drugs. The FDA also can impose partial clinical holds, for example prohibiting the initiation of clinical studies of a certain duration or for a certain dose.

All clinical studies must be conducted under the supervision of one or more qualified investigators in accordance with GCP regulations. These regulations include the requirement that all research subjects provide informed consent. Further, an institutional review board, or IRB, must review and approve the plan for any clinical study before it commences at any institution. An IRB considers, among other things, whether the risks to individuals participating in the clinical study are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the information regarding the clinical study and the consent form that must be provided to each clinical study subject or his or her legal representative and must monitor the clinical study until completed.

Each new clinical protocol and any amendments to the protocol must be submitted to the IND for FDA review, and to the IRBs for approval. Protocols detail, among other things, the objectives of the clinical study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety.

Human clinical studies are typically conducted in three sequential phases that may overlap or be combined:

- *Phase I.* The product is initially introduced into healthy human subjects and tested for safety, dosage tolerance, absorption, metabolism, distribution and excretion. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing may be conducted in patients.

- *Phase 2.* Involves clinical studies in 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 and schedule.
- *Phase 3.* Clinical studies are undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population at geographically dispersed clinical study sites. These clinical studies are intended to establish the overall risk/benefit ratio of the product and provide an adequate basis for product labeling.

Progress reports detailing the results of the clinical studies must be submitted at least annually to the FDA and safety reports must be submitted to the FDA and the investigators for serious and unexpected adverse events. Phase 1, Phase 2 and Phase 3 testing may not be completed successfully within any specified period, if at all. The FDA or the sponsor may suspend or terminate a clinical study at any time on various grounds, including a finding that the research subjects or patients are being exposed to an unacceptable health risk. Similarly, an IRB can suspend or terminate approval of a clinical study at its institution if the clinical study is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

Concurrent with clinical studies, companies usually complete additional animal studies and must also develop additional information about the chemistry and physical characteristics of the product and finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the manufacturer must develop methods for testing the identity, strength, quality and purity of the final product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the product candidate does not undergo unacceptable deterioration over its shelf life.

### ***U.S. Review and Approval Processes***

The results of product development, nonclinical studies and clinical studies, along with descriptions of the manufacturing process, analytical tests conducted on the drug, proposed labeling and other relevant information, are submitted to the FDA as part of an NDA for a new drug, requesting approval to market the product. The submission of an NDA is subject to the payment of a substantial user fee; a waiver of such fee may be obtained under certain limited circumstances. For example, the agency will waive the application fee for the first human drug application that a small business or its affiliate submits for review.

In addition, under the Pediatric Research Equity Act of 2003, or PREA, made into permanent law pursuant to Food and Drug Administration Safety and Innovation Act (FDASIA), an NDA or supplement to an NDA must contain data to assess the safety and effectiveness of the drug 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. The FDA may grant deferrals for submission of data or full or partial waivers.

The FDA reviews all NDAs submitted to ensure that they are sufficiently complete for substantive review before it accepts them for filing. The FDA may request additional information rather than accept an NDA for filing. In this event, the NDA must be re-submitted with the additional information. The re-submitted application also is 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 reviews an NDA to determine, among other things, whether a product is safe and effective for its intended use and whether its manufacturing is cGMP-compliant to assure and preserve the product's identity, strength, quality and purity. Before approving an NDA, the FDA will inspect the facility or facilities where the product is manufactured. 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. The FDA also can require, or an NDA applicant may voluntarily propose, a Risk Evaluation and Mitigation Strategy, or REMS, to ensure the benefits of a drug outweigh its risks. Elements of a REMS may include "dear doctor letters," a medication guide, and in some cases restrictions on distribution. These elements are negotiated as part of the NDA approval, and in some cases may delay the approval date. Once adopted, REMS are subject to periodic assessment and modification. The FDA may refer the NDA to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions. An advisory committee is a panel of experts who provide advice and recommendations when requested by the FDA on matters of importance that come before the agency. The FDA is not bound by the recommendation of an advisory committee.

The approval process is lengthy and difficult and the FDA may refuse to approve an NDA if the applicable regulatory criteria are not satisfied or may require additional clinical data or other data and information. Even if such data and information are submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical studies are not always conclusive and the FDA may interpret data differently than we interpret the same data. The FDA will issue a complete response letter if the agency decides not to approve the NDA in its present form. The complete response letter usually describes all of the specific deficiencies that the FDA identified in the NDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical studies. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the NDA, addressing all of the deficiencies identified in the letter, or withdraw the application or request an opportunity for a hearing.

If a product receives regulatory approval, the approval may be significantly limited to specific patient populations, therapeutic settings, risk categories of disease, and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product. Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. In addition, the FDA may require further Phase 3 and Phase 4 testing to be conducted, which involves clinical studies designed to further assess a drug's safety and effectiveness after NDA approval and may require testing and surveillance programs to monitor the safety of approved products that have been commercialized.

#### ***Patent Term Restoration and Marketing Exclusivity***

Depending upon the timing, duration and specifics of FDA approval of the use of our product candidates, some of our U.S. patents may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally 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 approval of that application. Only one patent applicable to an approved drug is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we intend to apply for restorations of patent term for some of our currently owned or licensed patents to add patent life beyond their current expiration dates, depending on the expected length of the clinical studies and other factors involved in the filing of the relevant NDA, however there can be no assurance that any such extension will be granted to us.

Market exclusivity provisions under the FDCA can also delay the submission or the approval of certain applications. The FDCA provides a five-year period of non-patent marketing exclusivity within the United States to the first applicant to gain approval of an NDA for a new chemical entity. A drug is a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety, which is the molecule or ion responsible for the action of the drug substance. During the exclusivity period, the FDA may not accept for review an abbreviated new drug application, or ANDA, or a 505(b)(2) NDA submitted by another company for another version of such drug where the applicant does not own or have a legal right of reference to all the data required for approval. However, an application may be submitted after four years if it contains a certification of patent invalidity or non-infringement. The FDCA also provides three years of marketing exclusivity for an NDA, 505(b)(2) NDA or supplement to an existing NDA if new clinical investigations, other than bioavailability studies, that were conducted or sponsored by the applicant are deemed by the FDA to be essential to the approval of the application, for example new indications, dosages or strengths of an existing drug. This three-year exclusivity covers only the conditions of use associated with the new clinical investigations and does not prohibit the FDA from approving ANDAs for drugs containing the original active agent. Five-year and three-year exclusivity will not delay the submission or approval of a full NDA. However, an applicant submitting a full NDA would be required to conduct or obtain a right of reference to all of the nonclinical studies and adequate and well-controlled clinical studies necessary to demonstrate safety and effectiveness.

Pediatric exclusivity is another type of exclusivity in the United States. Pediatric exclusivity, if granted, provides an additional six months to an existing exclusivity or statutory delay in approval resulting from a patent certification. This six-month exclusivity, which runs from the end of other exclusivity protection or patent delay, may be granted based on the voluntary completion of a pediatric clinical study in accordance with a FDA-issued "Written Request" for such a clinical study.

Certain foreign countries permit extension of patent term for a newly approved drug and/or grant a period of data exclusivity and/or market exclusivity. For example, depending upon the timing and duration of the marketing authorization process in certain European countries, a newly approved drug may be eligible for a supplementary protection certification, or SPC, which can extend the basic patent right for the drug for a period up to five years.

### ***Post-Approval Requirements***

Any drugs for which we receive FDA approval are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, complying with certain electronic records and signature requirements and complying with FDA promotion and advertising requirements. The FDA strictly regulates labeling, advertising, promotion and other types of information on 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. Further, manufacturers of drugs must continue to comply with cGMP requirements, which are extensive and require considerable time, resources and ongoing investment to ensure compliance. In addition, changes to the manufacturing process generally require prior FDA approval before being implemented and other types of changes to the approved product, such as adding new indications and additional labeling claims, are also subject to further FDA review and approval.

Drug manufacturers and other entities involved in the manufacturing and distribution of approved drugs are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. The cGMP requirements apply to all stages of the manufacturing process, including the production, processing, sterilization, packaging, labeling, storage and shipment of the drug. Manufacturers must establish validated systems to ensure that products meet specifications and

regulatory standards, and test each product batch or lot prior to its release. We rely, and expect to continue to rely, on third parties for the production of clinical quantities of our product candidates. Future FDA and state inspections may identify compliance issues at the facilities of our contract manufacturers that may disrupt production or distribution or may require substantial resources to correct.

The FDA may withdraw a product approval if compliance with regulatory requirements is not maintained or if problems occur after the product reaches the market. Later discovery of previously unknown problems with a product may result in restrictions on the product or even complete withdrawal of the product from the market. Further, the failure to maintain compliance with regulatory requirements may result in administrative or judicial actions, such as fines, warning letters, holds on clinical studies, product recalls or seizures, product detention or refusal to permit the import or export of products, refusal to approve pending applications or supplements, restrictions on marketing or manufacturing, injunctions or civil or criminal penalties.

From time to time, legislation is drafted, introduced and passed in Congress that could significantly change the statutory provisions governing the approval, manufacturing and marketing of products regulated by the FDA. In addition to new legislation, the FDA regulations and policies are often revised or reinterpreted by the agency in ways that may significantly affect our business and our product candidates. It is impossible to predict whether further legislative or FDA regulation or policy changes will be enacted or implemented and what the impact of such changes, if any, may be.

### ***Foreign Regulation***

In addition to regulations in the United States, we will be subject to a variety of foreign regulations governing clinical studies and commercial sales and distribution of our product candidates to the extent we choose to sell any products outside of the United States. Whether or not we obtain FDA approval for a product, we must obtain approval of a product by the comparable regulatory authorities of foreign countries before we can commence clinical studies or marketing of the product in those countries. The approval process varies from country to country and the time may be longer or shorter than that required for FDA approval. The requirements governing the conduct of clinical studies, product licensing, pricing and reimbursement vary greatly from country to country. As in the United States, post-approval regulatory requirements, such as those regarding product manufacture, marketing, or distribution would apply to any product that is approved outside the United States.

### **Employees**

As of December 31, 2017, we had 57 full-time employees. Three of our employees have Ph.D. degrees and two have M.D. degrees. 38 of our employees are engaged in research and development activities. None of our employees are represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

### **Facilities**

Our corporate headquarters are located in Ann Arbor, Michigan where we lease and occupy approximately 7,900 square feet of office space. We lease and occupy an additional 5,500 square feet of office space in Ann Arbor, Michigan to support our clinical development operations. We believe our current facilities will be sufficient to meet our needs until expiration.

### **Legal Proceedings**

On January 12, 2016, a purported stockholder of the Company filed a putative class action lawsuit in the United States District Court for the Eastern District of Michigan, against us and Tim Mayleben, captioned *Kevin L. Dougherty v. Esperion Therapeutics, Inc., et al.* (No. 16-cv-10089). The lawsuit alleges

that we and Mr. Mayleben violated Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and SEC Rule 10b-5 by allegedly failing to disclose in an August 17, 2015, public statement that the FDA would require a cardiovascular outcomes trial before approving our lead product candidate. The lawsuit seeks, among other things, compensatory damages in connection with an allegedly inflated stock price between August 18, 2015, and September 28, 2015, as well as attorneys' fees and costs. On May 20, 2016, an amended complaint was filed in the lawsuit and on July 5, 2016, we filed a motion to dismiss the amended complaint. On December 27, 2016, the court granted our motion to dismiss with prejudice and entered judgment in our favor. On January 24, 2017, the plaintiffs in this lawsuit filed a motion to alter or amend the judgment. In May 2017, the court denied the plaintiff's motion to alter or amend the judgement. On June 19, 2017, the plaintiffs filed a notice of appeal to the Sixth Circuit Court of Appeals and on September 14, 2017, they filed their opening brief in support of the appeal. The appeal was fully briefed on December 7, 2017.

On December 15, 2016, a purported stockholder of the Company filed a derivative lawsuit in the Court of Chancery of the State of Delaware against Tim Mayleben, Roger Newton, Mary McGowan, Nicole Vitullo, Dov Goldstein, Daniel Janney, Antonio Gotto Jr., Mark McGovern, Gilbert Omenn, Scott Braunstein, and Patrick Enright. The Company is named as a nominal defendant. The lawsuit alleges that the defendants breached their fiduciary duties to the Company when they made or approved improper statements on August 17, 2015, regarding our lead product candidate's path to FDA approval, and failed to ensure that reliable systems of internal controls were in place at the Company. The lawsuit seeks, among other things, any damages sustained by the Company as a result of the defendants' alleged breaches of fiduciary duties, including damages related to the above-referenced securities class action, an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures, restitution from the defendants, and attorneys' fees and costs. In light of, among other things, the early stage of the litigation, we are unable to predict the outcome of this matter and are unable to make a meaningful estimate of the amount or range of loss, if any, that could result from an unfavorable outcome.

In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

#### **Available Information**

Our website address is [www.esperion.com](http://www.esperion.com). Our Annual Reports on Form 10-K, Quarterly Reports on Form 10-Q, Current Reports on Form 8-K and amendments to those reports filed or furnished pursuant to Section 13(a) or 15(d) of the Securities Exchange Act of 1934 are available free of charge through the investor relations page of our internet website as soon as reasonably practicable after we electronically file such material with, or furnish it to, the Securities and Exchange Commission. Alternatively, these reports may be accessed at the SEC's website at [www.sec.gov](http://www.sec.gov).

**Item 1A. Risk Factors**

*Except for the historical information contained herein or incorporated by reference, this report and the information incorporated by reference contains forward-looking statements that involve risks and uncertainties. These statements include projections about our accounting and finances, plans and objectives for the future, future operating and economic performance and other statements regarding future performance. These statements are not guarantees of future performance or events. Our actual results could differ materially from those discussed in this report. Factors that could cause or contribute to these differences include, but are not limited to, those discussed in the following section, as well as those discussed in Part II, Item 7 entitled "Management's Discussion and Analysis of Financial Condition and Results of Operations" and elsewhere throughout this report and in any documents incorporated in this report by reference.*

*You should consider carefully the following risk factors, together with all of the other information included or incorporated in this report. If any of the following risks, either alone or taken together, or other risks not presently known to us or that we currently believe to not be significant, develop into actual events, then our business, financial condition, results of operations or prospects could be materially adversely affected. If that happens, the market price of our common stock could decline, and stockholders may lose all or part of their investment.*

**Risks Related to our Business and the Clinical Development and Commercialization of our Product Candidates**

***We depend almost entirely on the success of two product candidates, the bempedoic acid / ezetimibe combination pill and bempedoic acid, which are in Phase 3 clinical development. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, our product candidates.***

The bempedoic acid / ezetimibe combination pill and bempedoic acid are our only product candidates in clinical development, and our business depends almost entirely on their successful clinical development, regulatory approvals and commercialization. We currently have no drug products for sale and may never be able to develop marketable drug products. The bempedoic acid / ezetimibe combination pill and bempedoic acid may require substantial additional clinical development, testing, and regulatory approvals before we are permitted to commence their commercialization. The clinical studies of our product candidates are, and the manufacturing and marketing of our product candidates will be, subject to extensive and rigorous review and regulation by numerous government authorities in the U.S. and in other countries where we intend to test and, if approved, market any product candidate. Before obtaining regulatory approvals for the commercial sale of any product candidate, we must demonstrate through preclinical testing and clinical studies that the product candidate is safe and effective for use in each target indication. This process can take many years and require the expenditure of substantial resources beyond the proceeds we have raised, and may include post-marketing studies and surveillance, including a Risk Evaluation and Mitigation Strategy, or REMS program. Of the large number of drugs in development in the U.S., only a small percentage successfully complete the approval process at the FDA, EMA or any other foreign regulatory agency, and are commercialized. Accordingly, even if we are able to obtain the requisite financing to continue to fund our development and clinical programs, we cannot assure you that the bempedoic acid / ezetimibe combination pill and bempedoic acid or any other of our product candidates will be successfully developed or commercialized.

We are not permitted to market our product candidates in the U.S. or Europe until we receive approval of an NDA from the FDA, a MAA from the EMA, or in any other foreign countries until we receive the requisite approval from such countries. As a condition to submitting an NDA or MAA for the bempedoic acid / ezetimibe combination pill to treat patients with hypercholesterolemia, we initiated and intend to complete the pivotal Phase 3 clinical study (1002FDC-053) in addition to the

global pivotal Phase 3 LDL-C lowering program for bempedoic acid, to support an NDA submission for an LDL-C lowering indication. As a condition to submitting an NDA or MAA for bempedoic acid to treat patients with hypercholesterolemia, we have currently completed nine Phase 2 clinical studies and expect to complete the global pivotal Phase 3 LDL-C lowering efficacy and safety studies to support an NDA submission for an LDL-C lowering indication, and to complete the CLEAR Outcomes CVOT to support an NDA submission for a CVD risk reduction indication.

Additionally, we currently intend to submit NDAs in tandem for the bempedoic acid / ezetimibe combination pill and for bempedoic acid for LDL-C lowering indications by the first quarter of 2019 if we successfully complete our Phase 3 1002FDC-053 clinical study and Phase 3 LDL-C lowering program, based on the FDA's recent guidance that these programs are adequate to support approval of an LDL-C lowering indication. However, there is no guarantee that the FDA will view results from our Phase 3 1002FDC-053 clinical study or global pivotal Phase 3 LDL-C lowering program alone as sufficient to support approval of an LDL-C lowering indication for the bempedoic acid / ezetimibe combination pill or bempedoic acid. In the event that FDA determines LDL-C lowering is no longer a surrogate endpoint for initial approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid in the future, we would plan to submit our NDA for bempedoic acid with a proposed indication of CV risk reduction in statin intolerant patients on the basis of a completed and successful CLEAR Outcomes CVOT, which would include the results of the global pivotal Phase 3 LDL-C lowering program, by 2022. Obtaining approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA may delay, limit or deny approval of the bempedoic acid / ezetimibe combination pill and bempedoic acid for many reasons, including, among others:

- the FDA, EMA or any other regulatory authorities may change their approval policies or adopt new regulations, including with respect to whether LDL-C lowering is a surrogate endpoint for initial approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid;
- the FDA, EMA or any other regulatory authorities may change their approval policies for an LDL-C lowering indication for the bempedoic acid / ezetimibe combination pill or bempedoic acid if there is a shift in the future standard-of-care for statin intolerant patients with hypercholesterolemia;
- we may not be able to demonstrate that the bempedoic acid / ezetimibe combination pill and bempedoic acid are safe and effective in treating patients with hypercholesterolemia to the satisfaction of the FDA, EMA or any other regulatory agency;
- the results of our clinical studies may not meet the level of statistical or clinical significance required by the FDA or EMA for marketing approval;
- the magnitude of the treatment effect must also be clinically meaningful along with the drug's safety for a favorable benefit/risk assessment by the FDA, EMA or any other regulatory agency;
- the FDA, EMA or any other regulatory agency may change in the future the number, design, size, duration, patient enrollment criteria, exposure of patients, or conduct or implementation of our clinical studies;
- the FDA, EMA or any other regulatory agency may require that we conduct additional clinical studies;
- the FDA, EMA or any other regulatory agency may not approve the formulation, specifications or labeling of the bempedoic acid / ezetimibe combination pill or bempedoic acid;
- the clinical research organizations, or CROs, that we retain to conduct our clinical studies may take actions outside of our control that materially adversely impact our clinical studies;



- the FDA, EMA or any other regulatory agency may find the data from preclinical studies and clinical studies insufficient to demonstrate that the clinical and other benefits of the bempedoic acid / ezetimibe combination pill or bempedoic acid outweigh the safety risks;
- the FDA, EMA or any other regulatory agency may disagree with our interpretation of data from our preclinical studies and clinical studies;
- the FDA, EMA or any other regulatory agency may not accept data generated at our clinical study sites;
- if our NDAs, if and when submitted, are reviewed by an advisory committee, the FDA may have difficulties scheduling an advisory committee meeting in a timely manner or the advisory committee may recommend against approval of our applications or may recommend that the FDA require, as a condition of approval, additional preclinical studies or clinical studies, limitations in approved labeling or distribution and use restrictions;
- the FDA, EMA or any other regulatory agency may require the development of a REMS as a condition of approval or post-approval; or
- the FDA, EMA or any other regulatory agency may not approve the manufacturing processes or facilities of third-party manufacturers with which we contract.

Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain regulatory approval for and successfully market the bempedoic acid / ezetimibe combination pill and bempedoic acid. Moreover, because our business is almost entirely dependent upon these product candidates, any setback in our pursuit of its regulatory approval would have a material adverse effect on our business and prospects.

The development and approvals required for the approval of the bempedoic acid / ezetimibe combination pill are substantially identical to those for bempedoic acid, and the risks relating to the clinical development and approval of bempedoic acid apply equally to the bempedoic acid / ezetimibe combination pill. The FDA accepted our submission of an IND application for the bempedoic acid / ezetimibe combination pill in the second quarter of 2016 and we completed a bioavailability study. We announced the clinical development and regulatory plans for the bempedoic acid / ezetimibe combination pill in June 2017. Any failure in our development of bempedoic acid would materially and adversely affect our ability to develop, seek approval for and commercialize the bempedoic acid / ezetimibe combination pill for the planned indications. In addition, even if bempedoic acid succeeds in its clinical development and is approved for one or more indications, there can be no assurance that the bempedoic acid / ezetimibe combination pill would be developed successfully and approved for the same indications or at all, and vice versa.

***Failures or delays in the completion of our global pivotal Phase 3 efficacy and safety studies, our pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill or our CLEAR Outcomes CVOT for bempedoic acid could result in increased costs to us and could delay, prevent or limit our ability to generate revenue and continue our business.***

In January 2016, we commenced our global pivotal Phase 3 long-term safety and tolerability study (Study 1). We initiated our three remaining global pivotal Phase 3 LDL-C lowering efficacy studies and the CLEAR Outcomes CVOT in December 2016. We do not know whether our ongoing clinical studies will be completed on schedule, if at all. We initiated our pivotal Phase 3 1002FDC-053 clinical study for the bempedoic acid / ezetimibe combination pill in November 2017. We do not know whether this study will be completed on schedule. Successful completion of such clinical studies and, if required by the FDA due to a change in regulatory policy, our CLEAR Outcomes CVOT, are likely prerequisites to submitting an initial NDA to the FDA, MAA to the EMA or a similar application to any other foreign regulatory authorities from whom we seek to obtain approval and, consequently, the ultimate approval

and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid. The commencement and completion of clinical studies can be delayed or prevented for a number of reasons, including, among others:

- the FDA, EMA or any other regulatory authority may not agree to the study design or overall program;
- the FDA, EMA or any other regulatory authority may place a clinical study on hold;
- delays in reaching or failing to reach agreement on acceptable terms with prospective CROs and study sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and study sites;
- inadequate quantity or quality of a product candidate or other materials necessary to conduct clinical studies;
- difficulties or delays obtaining institutional review board, or IRB, approval to conduct a clinical study at a prospective site or sites;
- challenges in recruiting and enrolling patients to participate in clinical studies or in our CLEAR Outcomes CVOT, including the size and nature of the patient population, the proximity of patients to clinical sites, eligibility criteria for the clinical study, the nature of the clinical study protocol, the availability of approved effective treatments for the relevant disease and competition from other clinical study programs, including PCSK9 inhibitors, for similar indications;
- severe or unexpected drug-related side effects experienced by patients in a clinical study, including instances of muscle pain or weakness or other side effects;
- reports from preclinical or clinical testing of other cardiometabolic therapies that raise safety or efficacy concerns; and
- difficulties retaining patients who have enrolled in a clinical study but may be prone to withdraw due to rigors of the study, lack of efficacy, side effects, personal issues or loss of interest.

Clinical studies may also be delayed or terminated as a result of ambiguous or negative interim results. In addition, a clinical study may be suspended or terminated by us, the FDA, the EMA, the IRBs at the sites where the IRBs are overseeing a clinical study, a data safety monitoring committee, or DMC, overseeing the clinical study at issue or any other regulatory authorities due to a number of factors, including, among others:

- failure to conduct the clinical study in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical study operations or study sites by the FDA, EMA or any other regulatory authorities that reveals deficiencies or violations that require us to undertake corrective action, including the imposition of a clinical hold;
- unforeseen safety issues;
- changes in government regulations or administrative actions;
- problems with clinical supply materials; and
- lack of adequate funding to continue the clinical study.

***Positive results from completed Phase 1 and Phase 2 clinical studies of bempedoic acid are not necessarily predictive of the results of our ongoing global pivotal Phase 3 LDL-C lowering studies and CLEAR Outcomes CVOT of bempedoic acid or our pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill, nor do they guarantee approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid by the FDA, EMA or any other regulatory agency. If we cannot replicate the positive results from our completed Phase 1 and Phase 2 clinical studies of bempedoic acid in our ongoing and planned clinical studies and CVOT, we may be unable to successfully develop, obtain regulatory approval for and commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid.***

There is a high failure rate for drugs proceeding through clinical studies. Even if we are able to complete our ongoing global pivotal Phase 3 LDL-C studies, CLEAR Outcomes CVOT, pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill, and any potential additional Phase 3 clinical studies of bempedoic acid according to our current development timeline, the positive results from our completed Phase 1 and Phase 2 clinical studies of bempedoic acid, including those of our Phase 2 PK/PD (1002-035) study completed in October 2016, may not be replicated in our ongoing global pivotal Phase 3 LDL-C studies, CLEAR Outcomes CVOT, or pivotal Phase 3 1002FDC-053 clinical study results, nor do they guarantee approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid by the FDA, EMA or any other regulatory authorities in a timely manner or at all. Many companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical studies after achieving positive results in early stage development, and we cannot be certain that we will not face similar setbacks. These setbacks have been caused by, among other things, preclinical findings made while clinical studies were underway or safety or efficacy observations made in clinical studies, including previously unreported adverse events. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in preclinical studies and clinical studies nonetheless failed to obtain FDA and/or EMA approval. If we fail to obtain positive results in our ongoing global pivotal Phase 3 LDL-C studies, CLEAR Outcomes CVOT, pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill, and any potential additional Phase 3 clinical studies of bempedoic acid, the development timeline and regulatory approval and commercialization prospects for our leading product candidate, and, correspondingly, our business and financial prospects, would be materially adversely affected.

***We will need substantial additional capital in the future. If additional capital is not available, we will have to delay, reduce or cease operations.***

We reported top-line results from our Phase 2 (1002-008) clinical study in October 2014, our Phase 2 (1002-009) clinical study in March 2015, our Phase 2 (1002-014) exploratory clinical safety study in July 2015, and our Phase 2 PK/PD (1002-035) clinical study and Phase 1 PK (1002-037) study in October 2016. We held our End-of-Phase 2 meeting with the FDA in August 2015. In January 2016, we commenced our global pivotal Phase 3 long-term safety study (Study 1). We engaged in active dialogue in 2016 with the FDA and EMA to discuss our global pivotal Phase 3 clinical program for bempedoic acid and, based on that dialogue, announced our clinical development and regulatory plans for bempedoic acid in June 2016. We initiated our global pivotal Phase 3 LDL-C lowering efficacy studies and our CLEAR Outcomes CVOT in December 2016. In March 2017, we announced that the FDA confirmed that our global pivotal Phase 3 LDL-C lowering program is adequate to support approval of an LDL-C lowering indication for bempedoic acid, and reached an agreement with the FDA on the definition of statin intolerance. In June 2017, we announced that the FDA confirmed the regulatory pathway to approval for the bempedoic acid / ezetimibe combination pill. However, there is no guarantee that the FDA will view results from our Phase 3 1002FDC-053 clinical study or global

pivotal Phase 3 LDL-C lowering program alone as sufficient to support approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid. We currently intend to submit an NDA for an LDL-C lowering indication for the bempedoic acid / ezetimibe combination pill through the abbreviated 505(b)(2) pathway by the first quarter of 2019 if we successfully complete our Phase 3 1002FDC-053 clinical study and our global pivotal Phase 3 LDL-C lowering program. We currently intend to submit an NDA for bempedoic acid for an LDL-C lowering indication in patients with hypercholesterolemia by the first quarter of 2019 if we successfully complete our global pivotal Phase 3 LDL-C lowering program.

In the event that FDA determines LDL-C lowering is no longer a surrogate endpoint for initial approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid in the future, we would plan to submit our NDA for bempedoic acid (monotherapy) for a CV risk reduction indication on the basis of a completed and successful CVOT, which would include the results of the global pivotal Phase 3 LDL-C lowering program, by 2022. We expect that these clinical studies, plus any additional clinical studies that we undertake for the clinical development of the bempedoic acid / ezetimibe combination pill or bempedoic acid, will consume substantial additional financial resources. We expect that our existing cash and cash equivalents only will be sufficient to fund through the expected approvals of the bempedoic acid / ezetimibe combination pill and bempedoic acid in the first quarter of 2020. We will need to raise additional capital to continue to fund the further development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid and our operations. Our future capital requirements may be substantial and will depend on many factors including:

- the scope, size, rate of progress, results and costs of completing our CLEAR Outcomes CVOT of bempedoic acid;
- the scope, size, rate of progress, results and costs of completing our global pivotal Phase 3 LDL-C lowering program of bempedoic acid, which currently includes multiple global pivotal Phase 3 clinical efficacy and safety studies;
- the scope, size, rate of progress, results and costs of clinical development of the bempedoic acid / ezetimibe combination pill for the same indications as bempedoic acid, including that of our pivotal Phase 3 clinical study;
- the cost, timing and outcome of our efforts to obtain marketing approval for the bempedoic acid / ezetimibe combination pill and bempedoic acid, including to fund the preparation and submission of two NDAs with the FDA and two MAAs with the EMA for the bempedoic acid / ezetimibe combination pill and bempedoic acid and to satisfy related FDA and EMA requirements;
- the number and characteristics of any additional product candidates we develop or acquire;
- the costs associated with commercializing the bempedoic acid / ezetimibe combination pill and bempedoic acid or any future product candidates if we receive marketing approval, including the cost and timing of developing sales and marketing capabilities or entering into strategic collaborations to market and sell the bempedoic acid / ezetimibe combination pill and bempedoic acid or any future product candidates;
- the cost of manufacturing the bempedoic acid / ezetimibe combination pill and bempedoic acid or any future product candidates and any products we successfully commercialize; and
- the costs associated with general corporate activities, such as the cost of filing, prosecuting and enforcing patent claims.

Changing circumstances may cause us to consume capital significantly faster than we currently anticipate. Because the outcome of any clinical study is highly uncertain, we cannot reasonably estimate

the actual amounts necessary to successfully complete the development, regulatory approval and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid and any future product candidates. Additional financing may not be available when we need it or may not be available on terms that are favorable to us. In addition, we may seek additional capital due to favorable market conditions or strategic considerations, even if we believe we have sufficient funds for our current or future operating plans. If adequate funds are unavailable to us on a timely basis, or at all, we may not be able to continue the development of the bempedoic acid / ezetimibe combination pill and bempedoic acid or any future product candidate, or to commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid or any future product candidate, if approved, unless we find a partner.

***We are an emerging pharmaceutical company and have not generated any revenue from product sales. We have incurred significant operating losses since our inception, and anticipate that we will incur continued losses for the foreseeable future.***

We have a limited operating history on which to base your investment decision. Pharmaceutical product development is a highly speculative undertaking and involves a substantial degree of risk. We were incorporated in January 2008. Our operations to date have been limited primarily to organizing and staffing our company and conducting research and development activities for bempedoic acid. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates. As such, we are subject to all the risks incident to the development, regulatory approval and commercialization of new pharmaceutical products and we may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors.

Since our inception, we have focused substantially all of our efforts and financial resources on developing bempedoic acid, which commenced Phase 3 clinical development in January 2016. We have funded our operations to date primarily through proceeds from sales of preferred stock, public offerings of common stock, convertible promissory notes and warrants and the incurrence of indebtedness, and we have incurred losses in each year since our inception. Our net losses were \$167.0 million, \$75.0 million and \$49.8 million for the years ended December 31, 2017, 2016 and 2015, respectively. As of December 31, 2017, we had an accumulated deficit of \$396.3 million. Substantially all of our operating losses resulted from costs incurred in connection with our development program and from general and administrative costs associated with our operations. We expect to incur increasing levels of operating losses over the next several years and for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our stockholders' equity and working capital. We expect to continue incurring research and development expenses in connection with our ongoing and additional clinical studies of bempedoic acid, particularly our Phase 3 program and CLEAR Outcomes CVOT, as well as any clinical studies that we undertake to develop the bempedoic acid / ezetimibe combination pill, including our ongoing global pivotal Phase 3 1002FDC-053 clinical study, and development of any other product candidates we may choose to pursue. In addition, if we obtain marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, we will also incur significant sales, marketing and outsourced manufacturing expenses and expect a significant increase in our research and development expenses in connection with the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid, respectively. As a public company, we have incurred and will continue to incur additional costs associated with operating as a public company, particularly now that we are no longer an "emerging growth company." As a result, we expect to continue to incur significant and increasing operating losses for the foreseeable future. Because of the numerous risks and uncertainties associated with developing pharmaceutical products, we are unable to predict the extent of any future losses or when we will become profitable, if at all. Even if we do become profitable, we may not be able to sustain or increase our profitability on a quarterly or annual basis.

***Changes in regulatory requirements, FDA or EMA guidance or unanticipated events during our global pivotal Phase 3 clinical studies, our pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill, or our CVOT of bempedoic acid may occur, which may result in changes to clinical study protocols or additional clinical study requirements, which could result in increased costs to us and could delay our development timeline.***

Changes in regulatory requirements, FDA or EMA guidance or unanticipated events during our clinical studies may force us to amend clinical study protocols or the FDA or EMA may impose additional clinical study requirements. Significant amendments to our clinical study protocols may require resubmission to the FDA and/or IRBs for review and approval, which may adversely impact the cost, timing and/or successful completion of these studies. If we experience substantial delays completing—or if we terminate—any of our global pivotal Phase 3 clinical studies, our Phase 3 1002FDC-053 clinical study, or our CVOT, or if we are required to conduct additional clinical studies, the commercial prospects for the bempedoic acid / ezetimibe combination pill and bempedoic acid may be harmed and our ability to generate product revenue will be delayed.

Even though we completed enrollment of our global pivotal Phase 3 LDL-C lowering studies, we may not be able to identify and enroll the requisite number of patients in our Phase 3 1002FDC-053 clinical study, our CLEAR Outcomes CVOT, or any study that we undertake to support the development of our product candidates. Even if we are successful in enrolling patients, we may not ultimately be able to demonstrate sufficient clinical benefits from the bempedoic acid / ezetimibe combination pill and bempedoic acid, and our failure to do so may delay or hinder our ability to obtain FDA or EMA approval for these product candidates. We currently intend to submit NDAs in tandem for the bempedoic acid / ezetimibe combination pill and for bempedoic acid for LDL-C lowering indications by the first quarter of 2019 if we successfully complete our Phase 3 1002FDC-053 clinical study and Phase 3 LDL-C lowering program, based on the FDA's recent guidance that these programs are adequate to support approval of an LDL-C lowering. However, the FDA has indicated its position regarding an LDL-C lowering indication could be impacted by potential future changes in their view of LDL-C lowering as a surrogate endpoint or the possibility of a shift in the future standard-of-care for statin intolerant patients with hypercholesterolemia, and there is no guarantee that the FDA will view results from our Phase 3 1002FDC-053 clinical study and global pivotal Phase 3 LDL-C lowering program alone as sufficient to support approvals of an LDL-C lowering indication. Conducting our CLEAR Outcomes CVOT will be costly and time-consuming, and any requirement to complete the CVOT prior to approval of bempedoic acid would adversely affect our development timeline and financial condition.

***We have limited experience using the 505(b)(2) regulatory pathway to submit an NDA or any similar drug approval application to the FDA, and we cannot be certain that any of our product candidates will receive regulatory approval.***

We are developing bempedoic acid / ezetimibe combination pill for which we may seek FDA approval through the Section 505(b)(2) regulatory pathway. The Drug Price Competition and Patent Term Restoration Act of 1984, also known as the Hatch-Waxman Act, added Section 505(b)(2) to the Federal Food, Drug and Cosmetic Act, or FDCA. Section 505(b)(2) permits the submission of an NDA where at least some of the information required for approval comes from studies that were not conducted by or for the applicant and for which the applicant has not obtained a right of reference. Section 505(b)(2) would allow an NDA we submit to FDA to rely in part on data in the public domain or the FDA's prior conclusions regarding the safety and effectiveness of approved compounds, which could expedite the development program for our product candidates by potentially decreasing the amount of clinical data that we would need to generate in order to obtain FDA approval. If the FDA does not allow us to pursue the Section 505(b)(2) regulatory pathway as anticipated, we may need to conduct additional clinical trials, provide additional data and information, and meet additional

standards for regulatory approval. If this were to occur, the time and financial resources required to obtain FDA approval for these product candidates, and complications and risks associated with these product candidates, would likely substantially increase. We could need to obtain additional funding, which could result in significant dilution to the ownership interests of our existing stockholders to the extent we issue equity securities or convertible debt. We cannot assure you that we would be able to obtain such additional financing on terms acceptable to us, if at all. Moreover, inability to pursue the Section 505(b)(2) regulatory pathway could result in new competitive products reaching the market more quickly than our product candidates, which would likely materially adversely impact our competitive position and prospects. Even if we are allowed to pursue the Section 505(b)(2) regulatory pathway, we cannot assure you that the bempedoic acid / ezetimibe combination pill will receive the requisite approvals for commercialization.

In addition, notwithstanding the approval of a number of products by the FDA under Section 505(b)(2) over the last few years, certain brand-name pharmaceutical companies and others have objected to the FDA's interpretation of Section 505(b)(2). If the FDA's interpretation of Section 505(b)(2) is successfully challenged, the FDA may change its 505(b)(2) policies and practices, which could delay or even prevent the FDA from approving any NDA that we submit under Section 505(b)(2). In addition, the pharmaceutical industry is highly competitive, and Section 505(b)(2) NDAs are subject to special requirements designed to protect the patent rights of sponsors of previously approved drugs that are referenced in a Section 505(b)(2) NDA. These requirements may give rise to patent litigation and mandatory delays in approval of our NDAs for up to 30 months or longer depending on the outcome of any litigation. It is not uncommon for a manufacturer of an approved product to file a citizen petition with the FDA seeking to delay approval of, or impose additional approval requirements for, pending competing products. If successful, such petitions can significantly delay, or even prevent, the approval of the new product. However, even if the FDA ultimately denies such a petition, the FDA may substantially delay approval while it considers and responds to the petition. In addition, even if we are able to utilize the Section 505(b)(2) regulatory pathway, there is no guarantee this would ultimately lead to accelerated product development or earlier approval.

Moreover, even if our product candidates are approved under Section 505(b)(2), the approval may be subject to limitations on the indicated uses for which the products may be marketed or to other conditions of approval, or may contain requirements for costly post-marketing testing and surveillance to monitor the safety or efficacy of the products.

***Even if we receive marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, we may still face future development and regulatory difficulties.***

Even if we receive marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, regulatory authorities may still impose significant restrictions on the bempedoic acid / ezetimibe combination pill or bempedoic acid's indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies, such as a CVOT. The bempedoic acid / ezetimibe combination pill and bempedoic acid will also be subject to ongoing FDA requirements governing the packaging, storage, labeling, advertising and promotion of the product, recordkeeping and submission of safety updates and other post-marketing information. The FDA has significant post-marketing authority, including, for example, the authority to require labeling changes based on new safety information and to require post-marketing studies or clinical studies to evaluate serious safety risks related to the use of a drug product. The FDA also has the authority to require, as part of an NDA or post-approval, the submission of a REMS. Any REMS required by the FDA may lead to increased costs to assure compliance with post-approval regulatory requirements and potential requirements or restrictions on the sale of approved products, all of which could lead to lower sales volume and revenue. The EMA and other foreign regulatory authorities may impose similar

requirements on the bempedoic acid / ezetimibe combination pill or bempedoic acid as those described above with respect to the FDA.

Manufacturers of drug products and their facilities are subject to continual review and periodic inspections by the FDA and other regulatory authorities for compliance with current Good Manufacturing Practices and other regulations. If we or a regulatory agency discover problems with the bempedoic acid / ezetimibe combination pill or bempedoic acid, such as adverse events of unanticipated severity or frequency, or problems with the facility where the bempedoic acid / ezetimibe combination pill or bempedoic acid is manufactured, a regulatory agency may impose restrictions on the bempedoic acid / ezetimibe combination pill or bempedoic acid, the manufacturer or us, including requiring withdrawal of the bempedoic acid / ezetimibe combination pill or bempedoic acid from the market or suspension of manufacturing. If we, the bempedoic acid / ezetimibe combination pill or bempedoic acid or the manufacturing facilities for the bempedoic acid / ezetimibe combination pill or bempedoic acid fail to comply with applicable regulatory requirements, a regulatory agency may, among other things:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw marketing approval;
- suspend any ongoing clinical studies;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements; or
- seize or detain products, refuse to permit the import or export of products, or request that we initiate a product recall.

***Even if we receive marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid in the U.S., we may never receive regulatory approval to market the bempedoic acid / ezetimibe combination pill or bempedoic acid outside of the U.S., and vice versa.***

In order to market any product outside of the U.S., we must establish and comply with the numerous and varying efficacy, safety and other regulatory requirements of the countries in which we intend to market our product. Approval procedures vary among countries and can involve additional product candidate testing and additional administrative review periods. The time required to obtain approvals in other countries might differ from that required to obtain FDA approval. The marketing approval processes in other countries may include all of the risks detailed above regarding FDA approval in the U.S. as well as other risks, or vice versa. In particular, in many countries outside of the U.S., products must receive pricing and reimbursement approval before the product can be commercialized. Obtaining this approval can result in substantial delays in bringing products to market in such countries. Marketing approval in one country does not ensure marketing approval in another, but a failure or delay in obtaining marketing approval in one country may have a negative effect on the regulatory process in others. Failure to obtain marketing approval in other countries or any delay or other setback in obtaining such approval would impair our ability to commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid in such foreign markets. Any such impairment would reduce the size of our potential market, which could have a material adverse impact on our business, results of operations and prospects.



***Even if we receive marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, it may not achieve broad market acceptance, which would limit the revenue that we generate from its sales.***

The commercial success of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved by the FDA or other regulatory authorities, will depend upon the awareness and acceptance of the bempedoic acid / ezetimibe combination pill and bempedoic acid among the medical community, including physicians, patients and healthcare payors. Market acceptance of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, will depend on a number of factors, including, among others:

- the bempedoic acid / ezetimibe combination pill and bempedoic acid's demonstrated ability to treat statin intolerant patients for LDL-C lowering or CV risk reduction as an add-on for patients already on statin therapy, as compared with other available therapies;
- the relative convenience and ease of administration of the bempedoic acid / ezetimibe combination pill and bempedoic acid, including as compared with other treatments for patients for LDL-C lowering or CV risk reduction;
- the prevalence and severity of any adverse side effects such as muscle pain or weakness;
- limitations or warnings contained in the labeling approved for the bempedoic acid / ezetimibe combination pill or bempedoic acid by the FDA;
- availability of alternative treatments, including a number of competitive therapies already approved for LDL-C lowering or CV risk reduction, including PCSK9 inhibitors, or expected to be commercially launched in the near future;
- pricing and cost effectiveness;
- the effectiveness of our sales and marketing strategies;
- our ability to increase awareness of the bempedoic acid / ezetimibe combination pill or bempedoic acid through marketing efforts;
- our ability to obtain sufficient third-party coverage or reimbursement; and
- the willingness of patients to pay out-of-pocket in the absence of third-party coverage.

If the bempedoic acid / ezetimibe combination pill or bempedoic acid is approved but does not achieve an adequate level of acceptance by patients, physicians and payors, we may not generate sufficient revenue from the bempedoic acid / ezetimibe combination pill and bempedoic acid to become or remain profitable. Our efforts to educate the medical community and third-party payors about the benefits of the bempedoic acid / ezetimibe combination pill and bempedoic acid may require significant resources and may never be successful.

***If we are unable to establish sales and marketing capabilities or enter into agreements with third parties to market and sell the bempedoic acid / ezetimibe combination pill and bempedoic acid, we may not be able to generate any revenue.***

We do not currently have an infrastructure for the sales, marketing and distribution of pharmaceutical products. In order to market the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved by the FDA or any other regulatory body, we must build our sales, marketing, managerial, and other non-technical capabilities or make arrangements with third parties to perform these services. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, or if we are unable to do so on commercially reasonable terms, our business, results of operations, financial condition and prospects will be materially adversely affected.

***Even if we obtain marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, physicians and patients using other LDL-C lowering therapies may choose not to switch to our product.***

Physicians are often reluctant to switch their patients from existing therapies even when new and potentially more effective, safe or convenient treatments enter the market. In addition, patients often acclimate to the brand or type of 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. If physicians or patients are reluctant to switch from existing therapies to the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, our operating results and financial condition would be materially adversely affected.

***The development and, if approved, commercialization of the bempedoic acid / ezetimibe combination pill depends on the availability to and use of ezetimibe by the target patient of this combination therapy.***

The bempedoic acid / ezetimibe combination pill is dependent on the continued availability and use of ezetimibe in the marketplace, and there can be no assurance that the current availability and use of ezetimibe will continue. For example, changes in standard of care or use patterns of ezetimibe could make our bempedoic acid / ezetimibe combination pill therapy obsolete. In addition, ezetimibe could encounter unexpected results in the future and be associated with adverse outcomes during long-term use. Finally, the producers of ezetimibe are under no obligation to continue producing, commercializing or making ezetimibe available to patients, or to continue producing ezetimibe in any particular quantity, which could prevent our ability to obtain ezetimibe for use in our planned clinical trials or impact the number of patients taking ezetimibe who are available to enroll in our clinical trials. For example, such producers may encounter manufacturing or other production issues and fail to produce enough ezetimibe for us to successfully complete our studies and clinical trials, and this could cause our bempedoic acid / ezetimibe combination pill development program or commercialization efforts, if the bempedoic acid / ezetimibe combination pill is approved, to fail or be significantly delayed.

***Guidelines and recommendations published by various organizations may adversely affect the FDA's review of the bempedoic acid / ezetimibe combination pill and bempedoic acid for LDL-C lowering in patients or the use or commercial viability of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved for any indication or patient population.***

Government agencies issue regulations and guidelines directly applicable to us and to the bempedoic acid / ezetimibe combination pill and bempedoic acid, including guidelines generally relating to therapeutically significant LDL-C levels. In addition, professional societies, practice management groups, private health or science foundations and other organizations involved in the research, treatment and prevention of various diseases from time to time publish guidelines or recommendations to the medical and patient communities. These various sorts of recommendations may relate to such matters as product usage and use of related or competing therapies. For example, organizations such as the AHA have made recommendations about therapies in the cardiovascular therapeutics market. In addition, while we recently reached an agreement with the FDA on the definition of statin intolerance, there is no guarantee that the FDA's view of this definition would not change in the future. We expect that the FDA's view of the standard of care for patients with hypercholesterolemia at the time we submit our NDAs for LDL-C lowering indications in patients with hypercholesterolemia will impact the evaluation of such NDAs, including how this standard of care evolves in light of guidelines and recommendations in respect of the use of PCSK9 inhibitors. In addition, following any approval, we expect that changes to these existing recommendations or other guidelines advocating alternative therapies could result in decreased use of the bempedoic acid / ezetimibe combination pill and bempedoic acid, which would adversely affect our results of operations.

***Even if approved, reimbursement policies could limit our ability to sell the bempedoic acid / ezetimibe combination pill or bempedoic acid.***

Market acceptance and sales of the bempedoic acid / ezetimibe combination pill and bempedoic acid will depend on reimbursement policies and may be affected by healthcare reform measures. Government authorities and third-party payors, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels for those medications. Cost containment is a primary concern in the U.S. healthcare industry and elsewhere. Government authorities and these third-party payors have attempted to control costs by limiting coverage and the amount of reimbursement for particular medications. We cannot be sure that reimbursement will be available for the bempedoic acid / ezetimibe combination pill or bempedoic acid and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, the bempedoic acid / ezetimibe combination pill or bempedoic acid. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid.

In some foreign countries, particularly in Canada and European countries, the pricing of prescription pharmaceuticals is subject to strict governmental control. In these countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of regulatory approval and product launch. To obtain favorable reimbursement for the indications sought or pricing approval in some countries, we may be required to conduct a clinical study that compares the cost-effectiveness of the bempedoic acid / ezetimibe combination pill and bempedoic acid with other available therapies. If reimbursement for the bempedoic acid / ezetimibe combination pill or bempedoic acid is unavailable in any country in which we seek reimbursement, if it is limited in scope or amount, if it is conditioned upon our completion of additional clinical studies, or if pricing is set at unsatisfactory levels, our operating results could be materially adversely affected.

***Our future product development programs for candidates other than the bempedoic acid / ezetimibe combination pill or bempedoic acid may require substantial financial resources and may ultimately be unsuccessful.***

In addition to the development of the bempedoic acid / ezetimibe combination pill and bempedoic acid, we may in the future pursue the development of other early-stage development programs. Our potential product candidates have not commenced any clinical studies, and there are a number of FDA requirements that we must satisfy before we can commence such clinical studies. Satisfaction of these requirements will entail substantial time, effort and financial resources. We may never satisfy these requirements. Any time, effort and financial resources we expend on any early-stage development programs that we may pursue may adversely affect our ability to continue development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid, and we may never commence clinical studies of such development programs despite expending significant resources in pursuit of their development. If we do commence clinical studies of our other potential product candidates, such product candidates may never be approved by the FDA.

***Recent federal legislation will increase pressure to reduce prices of pharmaceutical products paid for by Medicare, which could materially adversely affect our revenue, if any, and our results of operations.***

In the United States, the Medicare Prescription Drug, Improvement, and Modernization Act of 2003, also called the MMA, changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by the elderly and introduced a new reimbursement methodology based on average sales prices for physician-administered drugs. In addition, this legislation provided authority for limiting the number of drugs that will be covered in any therapeutic class. As a result of this legislation and the expansion of federal coverage of drug products, we expect that there will be additional pressure to reduce costs. These cost reduction initiatives and

other provisions of this legislation could decrease the scope of coverage and the price that we receive for any approved products and could seriously harm our business. While the MMA applies only to drug benefits for Medicare beneficiaries, private payors often follow Medicare coverage policies and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may cause a similar reduction in payments from private payors. This legislation may pose an even greater risk to the bempedoic acid / ezetimibe combination pill and bempedoic acid than some other pharmaceutical products because a significant portion of the target patient population for the bempedoic acid / ezetimibe combination pill and bempedoic acid would likely be over 65 years of age and, therefore, many such patients will be covered by Medicare.

In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Affordability Reconciliation Act, or collectively, the PPACA, became law in the United States. The goal of the PPACA is to reduce the cost of healthcare and substantially change the way healthcare is financed by both governmental and private insurers. While we cannot predict what impact on federal reimbursement policies this legislation will have in general or on our business specifically, the PPACA may result in downward pressure on pharmaceutical reimbursement, which could negatively affect market acceptance of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, or any of our future products. In 2012, members of the U.S. Congress and some state legislatures sought to overturn certain provisions of the PPACA including those concerning the mandatory purchase of insurance. However, on June 28, 2012, the United States Supreme Court upheld the constitutionality of these provisions. Members of the U.S. Congress have since proposed a number of legislative initiatives, including possible repeal of the PPACA. We cannot predict the outcome or impact of current proposals or whether new proposals will be made or adopted, when they may be adopted or what impact they may have on us if they are adopted. These challenges add to the uncertainty of the legislative changes as part of ACA.

Finally, the availability of generic LDL-C lowering treatments may also substantially reduce the likelihood of reimbursement for branded counterparts or other competitive LDL-C lowering therapies, such as the bempedoic acid / ezetimibe combination pill or bempedoic acid if it is approved for commercial distribution. If we fail to successfully secure and maintain reimbursement coverage for our products or are significantly delayed in doing so, we will have difficulty achieving market acceptance of our products and our business will be harmed.

***Recent federal legislation and actions by state and local governments may permit reimportation of drugs from foreign countries into the United States, including foreign countries where the drugs are sold at lower prices than in the United States, which could materially adversely affect our operating results.***

We may face competition for the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, from cheaper LDL-C lowering therapies sourced from foreign countries that have placed price controls on pharmaceutical products. The MMA contains provisions that may change U.S. importation laws and expand pharmacists' and wholesalers' ability to import cheaper versions of an approved drug and competing products from Canada, where there are government price controls. These changes to U.S. importation laws will not take effect unless and until the Secretary of Health and Human Services certifies that the changes will pose no additional risk to the public's health and safety and will result in a significant reduction in the cost of products to consumers. The Secretary of Health and Human Services has so far declined to approve a reimportation plan. Proponents of drug reimportation may attempt to pass legislation that would directly allow reimportation under certain circumstances. Legislation or regulations allowing the reimportation of drugs, if enacted, could decrease the price we receive for any products that we may develop, including the bempedoic acid / ezetimibe combination pill and bempedoic acid, and adversely affect our future revenues and prospects for profitability.

***The FDA and other regulatory agencies actively enforce the laws and regulations prohibiting the promotion of off-label uses. If we are found to have improperly promoted off-label uses, we may become subject to significant liability.***

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, such as the bempedoic acid / ezetimibe combination pill or bempedoic acid if approved. In particular, a product may not be promoted for uses that are not approved by the FDA or other regulatory agencies as reflected in the product's approved labeling. If we receive marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid as a therapy for lowering LDL-C levels in statin intolerant patients with elevated LDL-C, the first indication we intend to pursue, physicians may nevertheless prescribe the bempedoic acid / ezetimibe combination pill and bempedoic acid to their patients in a manner that is inconsistent with the approved label, potentially including as a therapy in addition to statins. If we are found to have promoted such off-label uses, we may become subject to significant liability. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees, corporate integrity agreements or permanent injunctions under which specified promotional conduct is changed or curtailed. If we cannot successfully manage the promotion of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, we could become subject to significant liability, which would materially adversely affect our business and financial condition.

***Our market is subject to intense competition. If we are unable to compete effectively, our opportunity to generate revenue from the sale of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, will be materially adversely affected.***

The LDL-C lowering therapies market is highly competitive and dynamic and dominated by the sale of inexpensive generic versions of statins. In 2017, generic statins, ezetimibe, and fixed combination drugs accounted for about 93% of U.S. prescriptions within the cholesterol / LDL-C lowering market. Our success will depend, in part, on our ability to obtain a share of the market, initially, for patients with hypercholesterolemia and ASCVD and/or HeFH, including high cardiovascular risk primary prevention patients, whose LDL-C is not adequately controlled despite receiving maximally tolerated lipid-modifying background therapy, or only able to tolerate less than the lowest approved daily starting dose of their statin and are therefore considered to be statin intolerant. Potential competitors in North America, Europe and elsewhere include major pharmaceutical companies, specialty pharmaceutical companies, biotechnology firms, universities and other research institutions and government agencies. Other pharmaceutical companies may develop LDL-C lowering therapies for patients that compete with the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, that do not infringe the claims of our patents, pending patent applications or other proprietary rights, which could materially adversely affect our business and results of operations. The FDA has also indicated to us that approval of other therapies could have an impact on their review of NDAs we submit for the bempedoic acid / ezetimibe combination pill and bempedoic acid for our LDL-C lowering programs in these patients.

LDL-C lowering therapies currently on the market that would compete with the bempedoic acid / ezetimibe combination pill and bempedoic acid include the following:

- Inexpensive generic versions of statins;
- Inexpensive generic versions of ezetimibe, a cholesterol absorption inhibitor;
- PCSK9 inhibitors such as Praluent® (alirocumab) and Repatha® (evolocumab), marketed by Sanofi/Regeneron and Amgen Inc. respectively;
- Bile acid sequestrants such as Welchol® (colesevelam), marketed by Daiichi Sankyo Inc.;

- MTP inhibitors, such as JUXTAPID® (lomitapide), marketed by Novilion Therapeutics, Inc.;
- Apo B Anti-Sense therapy, such as KYNAMRO® (mipomersen), marketed by Kastle Therapeutics LLC;
- Inexpensive generic versions of combination pill therapies, such as ezetimibe and simvastatin; and
- Other lipid-lowering monotherapies (including cheaper generic versions), such as Tricor® (fenofibrate) and Niaspan® (niacin extended release), both of which are marketed by AbbVie, Inc.

Several other pharmaceutical companies have other LDL-C lowering therapies in development that may be approved for marketing in the U.S. or outside of the U.S. Based on publicly available information, we believe the current therapies in development that would compete with the bempedoic acid / ezetimibe combination pill and bempedoic acid include PCSK9 inhibitors in development from Lilly and The Medicines Company/Alnylam.

Many of our potential competitors have substantially greater financial, technical and human resources than we do and significantly greater experience discovering and developing drug candidates, obtaining FDA and other marketing approvals of products and commercializing those products. Accordingly, our competitors may be more successful than we may be in obtaining FDA approval for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective, or more effectively marketed and sold, than the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, and may render the bempedoic acid / ezetimibe combination pill or bempedoic acid obsolete or non-competitive before we can recover the expenses of developing and commercializing it. If approved, the bempedoic acid / ezetimibe combination pill and bempedoic acid may also compete with unapproved and off-label LDL-C lowering treatments, and following the expiration of additional patents covering the LDL-C lowering market, we may also face additional competition from the entry of new generic drugs. We anticipate that we will encounter intense and increasing competition as new drugs enter the market and advanced technologies become available.

***We face potential product liability exposure, and, if claims are brought against us, we may incur substantial liability.***

The use of the bempedoic acid / ezetimibe combination pill and bempedoic acid in clinical studies and the sale of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, exposes us to the risk of product liability claims. Product liability claims might be brought against us by patients, healthcare providers or others selling or otherwise coming into contact with the bempedoic acid / ezetimibe combination pill or bempedoic acid. For example, we may be sued if any product we develop allegedly causes injury or is found to be otherwise unsuitable during product 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, including as a result of interactions with alcohol or other drugs, negligence, strict liability, and a breach of warranties. Claims could also be asserted under state consumer protection acts. If we become subject to product liability claims and cannot successfully defend ourselves against them, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in, among other things:

- withdrawal of patients from our clinical studies;
- substantial monetary awards to patients or other claimants;
- decreased demand for the bempedoic acid / ezetimibe combination pill or bempedoic acid or any future product candidates following marketing approval, if obtained;

- damage to our reputation and exposure to adverse publicity;
- increased FDA warnings on product labels;
- litigation costs;
- distraction of management's attention from our primary business;
- loss of revenue; and
- the inability to successfully commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid or any future product candidates, if approved.

We maintain product liability insurance coverage for our clinical studies with a \$10.0 million annual aggregate coverage limit, in addition to insurance coverage in specific local jurisdictions. Nevertheless, our insurance coverage may be insufficient to reimburse us for any expenses or losses we may suffer. Moreover, in the future, we may not be able to maintain insurance coverage at a reasonable cost or in sufficient amounts to protect us against losses, including if insurance coverage becomes increasingly expensive. If and when we obtain marketing approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid, we intend to expand our insurance coverage to include the sale of commercial products; however, we may not be able to obtain this product liability insurance on commercially reasonable terms. Large judgments have been awarded in class action lawsuits based on drugs that had unanticipated side effects. The cost of any product liability litigation or other proceedings, even if resolved in our favor, could be substantial, particularly in light of the size of our business and financial resources. A product liability claim or series of claims brought against us could cause our stock price to decline and, if we are unsuccessful in defending such a claim or claims and the resulting judgments exceed our insurance coverage, our financial condition, business and prospects could be materially adversely affected.

***We are subject to healthcare laws and regulations, which could expose us to criminal sanctions, civil penalties, contractual damages, reputational harm and diminished profits and future earnings.***

Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved. Our future arrangements with third-party payors will expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute the bempedoic acid / ezetimibe combination pill and bempedoic acid, if we obtain marketing approval. Restrictions under applicable federal and state healthcare laws and regulations include the following:

- The federal healthcare anti-kickback statute prohibits, among other things, persons 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 under federal healthcare programs such as Medicare and Medicaid.
- The federal False Claims Act imposes criminal and civil penalties, including those from civil whistleblower or qui tam actions, against individuals or entities for 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, as amended by the Health Information Technology for Economic and Clinical Health Act, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program and 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 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 PPACA require manufacturers of drugs, devices, biologics, and medical supplies to report to the Department of Health and Human Services information related to physician payments and other transfers of value and physician ownership and investment interests.
- Analogous state laws and regulations, such as state anti-kickback and false claims laws and transparency laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non-governmental third-party payors, including private insurers, and 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 healthcare providers or marketing expenditures and drug pricing.

Ensuring that our future business arrangements with third parties comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices do 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, including anticipated activities to be conducted by our sales team, were 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 and exclusion from government funded healthcare programs, such as Medicare and Medicaid, any of which could substantially disrupt our operations. If any of the physicians or other providers or entities with whom we expect to do business is found not to be in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government funded healthcare programs.

***Our internal computer systems, or those of our third-party clinical research organizations or other contractors or consultants, may fail or suffer security breaches, which could result in a material disruption of our the bempedoic acid / ezetimibe combination pill or bempedoic acid development programs.***

Despite the implementation of security measures, our internal computer systems and those of our third-party clinical research organizations and other contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war, and telecommunication and electrical failures. While we have not experienced any such system failure, accident, or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our programs. For example, the loss of clinical study data for the bempedoic acid / ezetimibe combination pill or bempedoic acid could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach results in a loss of or damage to our data or applications or other data or applications relating to our technology or product candidates, or inappropriate disclosure of confidential or proprietary information, we could incur liabilities and the further development of the bempedoic acid / ezetimibe combination pill or bempedoic acid could be delayed.



***Comprehensive Tax Reform Legislation Could Adversely Affect Our Business And Financial Condition.***

On December 22, 2017, President Trump signed into law the "Tax Cuts and Jobs Act" (TCJA) that significantly reforms the Internal Revenue Code of 1986, as amended (the "Code"). The TCJA, among other things, includes changes to U.S. federal tax rates, imposes significant additional limitations on the deductibility of interest, allows for the expensing of capital expenditures, and puts into effect the migration from a "worldwide" system of taxation to a territorial system. Our net deferred tax assets and liabilities have been revalued at the newly enacted U.S. corporate rate. We continue to examine the impact this tax reform legislation may have on our business. The impact of this tax reform is uncertain and could be adverse.

**Risks Related to our Intellectual Property**

***If we are unable to adequately protect our proprietary technology or maintain issued patents which are sufficient to protect the bempedoic acid / ezetimibe combination pill and bempedoic acid, others could compete against us more directly, which would have a material adverse impact on our business, results of operations, financial condition and prospects.***

Our commercial success will depend in part on our success obtaining and maintaining issued patents and other intellectual property rights in the United States and elsewhere and protecting our proprietary technology. If we do not adequately protect our intellectual property and proprietary technology, competitors may be able to use our technologies and erode or negate any competitive advantage we may have, which could harm our business and ability to achieve profitability.

As of December 31, 2017, our patent estate, including patents we own, on a worldwide basis, included approximately 23 issued United States patents and 6 pending United States patent applications and 18 issued patents and 40 pending patent applications in other foreign jurisdictions. Of our worldwide patents and pending applications, only a subset relates to our small molecule program. Bempedoic acid is claimed in U.S. Patent No. 7,335,799 that is scheduled to expire in December 2025, which includes 711 days of patent term adjustment, and may be eligible for a patent term extension period of up to five years. U.S. Patent Nos. 9,000,041, 8,497,301 and 9,624,152 claim methods of using bempedoic acid. We also have a pending U.S. patent application directed to bempedoic acid. There are currently six issued patents and one pending application in countries outside the United States that relate to bempedoic acid.

A subset of this portfolio relates to our bempedoic acid / ezetimibe combination pill and bempedoic acid and one or more statins. We have 17 pending applications outside the United States, and one inside the U.S., with claims directed to methods of treatment using the bempedoic acid / ezetimibe combination. Additionally, we own one pending U.S. application directed to the manufacturing of our bempedoic acid / ezetimibe combination pill. We also have 17 pending applications outside the U.S., and one inside the U.S., with claims directed to methods of treatment using a fixed dose combination of bempedoic acid and one or more statins.

We may not have identified all patents, published applications or published literature that affect our business either by blocking our ability to commercialize our drug candidates, by preventing the patentability of one or more aspects of our drug candidates to us or our licensors or co-owners, or by covering the same or similar technologies that may affect our ability to market our drug candidates. For example, we (or the licensor of a drug candidate to us) may not have conducted a patent clearance search to identify potentially obstructing third party patents. Moreover, patent applications in the United States are maintained in confidence for up to 18 months after their filing. In some cases, however, patent applications remain confidential in the U.S. Patent and Trademark Office, or the U.S. PTO, for the entire time prior to issuance as a U.S. patent. Patent applications filed in countries outside of the United States are not typically published until at least 18 months from their first filing date. Similarly, publication of discoveries in the scientific or patent literature often lags behind actual

discoveries. We cannot be certain that we or our licensors or co-owners were the first to invent, or the first to file, patent applications covering our drug candidates. We also may not know if our competitors filed patent applications for technology covered by our pending applications or if we were the first to invent the technology that is the subject of our patent applications. Competitors may have filed patent applications or received patents and may obtain additional patents and proprietary rights that block or compete with our patents.

Others may have filed patent applications or received patents that conflict with patents or patent applications that we own, have filed or have licensed, either by claiming the same methods, compounds or uses or by claiming methods, compounds or uses that could dominate those owned by or licensed to us. In addition, we may not be aware of all patents or patent applications that may affect our ability to make, use or sell any of our drug candidates. Any conflicts resulting from third-party patent applications and patents could affect our ability to obtain the necessary patent protection for our products or processes. If other companies or entities obtain patents with conflicting claims, we may be required to obtain licenses to these patents or to develop or obtain alternative technology. We may not be able to obtain any such licenses on acceptable terms or at all. Any failure to obtain such licenses could delay or prevent us from using discovery-related technology to pursue the development or commercialization of our drug candidates, which would adversely affect our business.

We cannot assure you that any of our patents have, or that any of our pending patent applications will mature into issued patents that will include, claims with a scope sufficient to protect the bempedoic acid / ezetimibe combination pill or bempedoic acid or any other product candidates. Others have developed technologies that may be related or competitive to our approach, and may have filed or may file patent applications and may have received or may receive patents that may overlap or conflict with our patent applications, either by claiming the same methods or formulations or by claiming subject matter that could dominate our patent position. The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, the issuance, scope, validity and enforceability of any patent claims that we may obtain cannot be predicted with certainty. Patents, if issued, may be challenged, deemed unenforceable, invalidated, or circumvented. U.S. patents and patent applications may also be subject to interference proceedings, ex parte reexamination, inter partes review and post-grant review proceedings, supplemental examination and may be challenged in district court. Patents granted in certain other countries may be subjected to opposition or comparable proceedings lodged in various national and regional patent offices. These proceedings could result in either loss of the patent or denial of the patent application or loss or reduction in the scope of one or more of the claims of the patent or patent application. In addition, such interference, re-examination, opposition, post-grant review, inter partes review, supplemental examination or revocation proceedings may be costly. Thus, any patents that we may own or exclusively license may not provide any protection against competitors. Furthermore, an adverse decision in an interference proceeding can result in a third-party receiving the patent right sought by us, which in turn could affect our ability to develop, market or otherwise commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid.

Furthermore, the issuance of a patent, while presumed valid and enforceable, is not conclusive as to its validity or its enforceability and it may not provide us with adequate proprietary protection or competitive advantages against competitors with similar products. Competitors may also be able to design around our patents. Other parties may develop and obtain patent protection for more effective technologies, designs or methods. We may not be able to prevent the unauthorized disclosure or use of our technical knowledge or trade secrets by consultants, vendors, former employees and current employees. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the United States, and we may encounter significant problems in protecting our proprietary rights in these countries. If these developments were to occur, they could have a material adverse effect on our sales.

Our ability to enforce our patent rights depends on our ability to detect infringement. It is difficult to detect infringers who do not advertise the components that are used in their products. Moreover, it may be difficult or impossible to obtain evidence of infringement in a competitor's or potential competitor's product. Any litigation to enforce or defend our patent rights, if any, even if we were to prevail, could be costly and time-consuming and would divert the attention of our management and key personnel from our business operations. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded if we were to prevail may not be commercially meaningful.

In addition, proceedings to enforce or defend our patents could put our patents at risk of being invalidated, held unenforceable, or interpreted narrowly. Such proceedings could also provoke third parties to assert claims against us, including that some or all of the claims in one or more of our patents are invalid or otherwise unenforceable. If, in any proceeding, a court invalidated or found unenforceable our patents covering the bempedoic acid / ezetimibe combination pill or bempedoic acid, our financial position and results of operations would be materially and adversely impacted. In addition, if a court found that valid, enforceable patents held by third parties covered the bempedoic acid / ezetimibe combination pill or bempedoic acid, our financial position and results of operations would also be materially and adversely impacted.

The degree of future protection for our proprietary rights is uncertain, and we cannot ensure that:

- any of our patents, or any of our pending patent applications, if issued, will include claims having a scope sufficient to protect the bempedoic acid / ezetimibe combination pill or bempedoic acid;
- any of our pending patent applications will result in issued patents;
- we will be able to successfully commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, before our relevant patents expire;
- we were the first to make the inventions covered by each of our patents and pending patent applications;
- we were the first to file patent applications for these inventions;
- others will not develop similar or alternative technologies that do not infringe our patents;
- any of our patents will be valid and enforceable;
- any patents issued to us will provide a basis for an exclusive market for our commercially viable products, will provide us with any competitive advantages or will not be challenged by third parties;
- we will develop additional proprietary technologies or product candidates that are separately patentable; or
- that our commercial activities or products, or those of our licensors, will not infringe upon the patents of others.

We rely upon unpatented trade secrets, unpatented know-how and continuing technological innovation to develop and maintain our competitive position, which we seek to protect, in part, by confidentiality agreements with our employees and our collaborators and consultants. We also have agreements with our employees and selected consultants that obligate them to assign their inventions to us. It is possible that technology relevant to our business will be independently developed by a person that is not a party to such an agreement. Furthermore, if the employees and consultants who are parties to these agreements breach or violate the terms of these agreements, we may not have adequate remedies for any such breach or violation, and we could lose our trade secrets through such breaches

or violations. Further, our trade secrets could otherwise become known or be independently discovered by our competitors.

***If we are not able to adequately prevent disclosure of trade secrets and other proprietary information, the value of our technology and products could be significantly diminished.***

We rely on trade secrets to protect our proprietary technologies, especially where we do not believe patent protection is appropriate or obtainable. However, trade secrets are difficult to protect. We rely in part on confidentiality agreements with our employees, consultants, outside scientific collaborators, sponsored researchers, contract manufacturers, vendors and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, we cannot guarantee that we have executed these agreements with each party that may have or have had access to our trade secrets.

Moreover, because we acquired certain rights from Pfizer, we must rely on Pfizer's practices, and those of its predecessors, with regard to parties that may have had access to our trade secrets related thereto before our incorporation. Any party with whom we or they 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, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they disclose such trade secrets, 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 or other third-party, our competitive position would be harmed.

***We may infringe the intellectual property rights of others, which may prevent or delay our product development efforts and stop us from commercializing or increase the costs of commercializing the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved.***

Our success will depend in part on our ability to operate without infringing the intellectual property and proprietary rights of third parties. We cannot assure you that our business, products and methods do not or will not infringe the patents or other intellectual property rights of third parties.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Other parties may allege that the bempedoic acid / ezetimibe combination pill or bempedoic acid or the use of our technologies infringes patent claims or other intellectual property rights held by them or that we are employing their proprietary technology without authorization. Patent and other types of intellectual property litigation can involve complex factual and legal questions, and their outcome is uncertain. Any claim relating to intellectual property infringement that is successfully asserted against us may require us to pay substantial damages, including treble damages and attorney's fees if we are found to be willfully infringing another party's patents, for past use of the asserted intellectual property and royalties and other consideration going forward if we are forced to take a license. In addition, if any such claim were successfully asserted against us and we could not obtain such a license, we may be forced to stop or delay developing, manufacturing, selling or otherwise commercializing the bempedoic acid / ezetimibe combination pill or bempedoic acid.

Even if we are successful in these proceedings, we may incur substantial costs and divert management time and attention in pursuing these proceedings, which could have a material adverse effect on us. If we are unable to avoid infringing the patent rights of others, we may be required to seek a license, defend an infringement action or challenge the validity of the patents in court, or

redesign our products. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion. In addition, intellectual property litigation or claims could force us to do one or more of the following:

- cease developing, selling or otherwise commercializing the bempedoic acid / ezetimibe combination pill or bempedoic acid;
- pay substantial damages for past use of the asserted intellectual property;
- obtain a license from the holder of the asserted intellectual property, which license may not be available on reasonable terms, if at all; and
- redesign, or rename in the case of trademark claims, the bempedoic acid / ezetimibe combination pill or bempedoic acid to avoid infringing the intellectual property rights of third parties, which may not be possible and, even if possible, could be costly and time-consuming.

Any of these risks coming to fruition could have a material adverse effect on our business, results of operations, financial condition and prospects.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.***

The United States has enacted and is currently implementing the America Invents Act of 2011, wide-ranging patent reform legislation. The United States 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. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, 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 submission, fee payment and other requirements imposed by governmental patent agencies, and our patent protection could be reduced or eliminated for non-compliance with these requirements.***

The U.S. PTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other provisions during the patent process. There are situations in which noncompliance can result in abandonment or lapse of a patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. In such an event, competitors might be able to enter the market earlier than would otherwise have been the case.

***We could become dependent on licensed intellectual property. If we were to lose our rights to licensed intellectual property, we may not be able to continue developing or commercializing the bempedoic acid / ezetimibe combination pill or bempedoic acid or other product candidates, if approved.***

In the future, we may enter into license(s) to third-party intellectual property that are necessary or useful to our business. Such license agreement(s) will likely impose various obligations upon us, and our licensor(s) have or may have the right to terminate the license thereunder in the event of a material breach or, in some cases, at will. Future licensor(s) may allege that we have breached our license agreement with them or decide to terminate our license at will, and accordingly seek to terminate our license. If successful, this could result in our loss of the right to use the licensed intellectual property, which could materially adversely affect our ability to develop and commercialize a product candidate or product, if approved, as well as harm our competitive business position and our business prospects.

***We do not seek to protect our intellectual property rights in all jurisdictions throughout the world and we may not be able to adequately enforce our intellectual property rights even in the jurisdictions where we seek protection.***

Filing, prosecuting and defending patents on product candidates in all countries and jurisdictions throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States could be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property rights to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. 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, but enforcement is not as strong as that in the United States. 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.

Many companies have encountered significant problems in protecting and defending intellectual property rights in foreign jurisdictions. The legal systems of certain countries, particularly certain developing countries, do not favor the enforcement of patents and other intellectual property protection, particularly those relating to emerging pharmaceuticals, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents at risk of being invalidated or interpreted narrowly, could put our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

***We may be subject to damages resulting from claims that we or our employees have wrongfully used or disclosed alleged trade secrets of their former employers.***

Our employees have been previously employed at other biotechnology or pharmaceutical companies, including our competitors or potential competitors. Although we are not aware of any claims currently pending against us, we may be subject to claims that these employees or we have inadvertently or otherwise used or disclosed trade secrets or other proprietary information of the former employers of our employees. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial costs and be a distraction to management. If we fail in defending such claims, in addition to paying money claims, we may lose valuable intellectual property rights or personnel. A loss of key personnel or their work product could hamper or prevent our ability to commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid, which would materially adversely affect our commercial development efforts.

### Risks Related to our Dependence on Third Parties

*We will be unable to directly control all aspects of our clinical studies due to our reliance on CROs and other third parties that assist us in conducting clinical studies.*

We relied on CROs in our prior clinical studies, and will continue to rely on CROs to conduct our ongoing global pivotal Phase 3 clinical studies, our CLEAR Outcomes CVOT and our pivotal Phase 3 1002FDC-053 clinical study, as well as any future clinical studies we may undertake. As a result, we will have less direct control over the conduct, timing and completion of these clinical studies and the management of data developed through the clinical studies than would be the case if we were relying entirely upon our own staff. Communicating with outside parties can also be challenging, potentially leading to mistakes as well as difficulties in coordinating activities. Outside parties may:

- have staffing difficulties;
- fail to comply with contractual obligations;
- experience regulatory compliance issues;
- undergo changes in priorities or become financially distressed; or
- form relationships with other entities, some of which may be our competitors.

These factors may materially adversely affect the willingness or ability of third parties to conduct our clinical studies and may subject us to unexpected cost increases that are beyond our control.

Moreover, the FDA requires us to comply with standards, commonly referred to as Good Clinical Practices, for conducting, recording, and reporting the results of clinical studies to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of clinical study participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements.

Problems with the timeliness or quality of the work of any CRO may lead us to seek to terminate our relationship with any such CRO and use an alternative service provider. Making this change may be costly and may delay our clinical studies, and contractual restrictions may make such a change difficult or impossible to effect. If we must replace any CRO that is conducting our clinical studies, our clinical studies may have to be suspended until we find another CRO that offers comparable services. The time that it takes us to find alternative organizations may cause a delay in the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid or may cause us to incur significant expenses to replicate data that may be lost. Although we do not believe that any CRO on which we may rely will offer services that are not available elsewhere, it may be difficult to find a replacement organization that can conduct our clinical studies in an acceptable manner and at an acceptable cost. Any delay in or inability to complete our clinical studies could significantly compromise our ability to secure regulatory approval of the bempedoic acid / ezetimibe combination pill or bempedoic acid and preclude our ability to commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid, thereby limiting or preventing our ability to generate revenue from its sales.

*We rely completely on third-party suppliers to manufacture our clinical drug supplies for the bempedoic acid / ezetimibe combination pill and bempedoic acid, and we intend to rely on third parties to produce commercial supplies of the bempedoic acid / ezetimibe combination pill and bempedoic acid and preclinical, clinical and commercial supplies of any future product candidate.*

We do not currently have, nor do we plan to acquire, the infrastructure or capability to internally manufacture our clinical drug supply of the bempedoic acid / ezetimibe combination pill and bempedoic acid, or any future product candidates, for use in the conduct of our preclinical studies and clinical studies, and we lack the internal resources and the capability to manufacture any product

candidates on a clinical or commercial scale. In addition, we have no control over the production of ezetimibe for the bempedoic acid / ezetimibe combination pill. The facilities used by our contract manufacturers to manufacture the active pharmaceutical ingredient and final drug for bempedoic acid, or any future product candidates, must be approved by the FDA and other comparable foreign regulatory agencies pursuant to inspections that would be conducted after we submit our NDA or relevant foreign regulatory submission to the applicable regulatory agency.

We do not control the manufacturing process of, and are completely dependent on, our contract manufacturers to comply with current Good Manufacturing Practices for manufacture of both active drug substances and finished drug products. If our contract manufacturers cannot successfully manufacture material that conforms to our specifications and the strict regulatory requirements of the FDA or applicable foreign regulatory agencies, they will not be able to secure and/or maintain regulatory approval for their manufacturing facilities. In addition, we have no direct control over our contract manufacturers' ability to maintain adequate quality control, quality assurance and qualified personnel. Furthermore, all of our contract manufacturers are engaged with other companies to supply and/or manufacture materials or products for such companies, which exposes our manufacturers to regulatory risks for the production of such materials and products. As a result, failure to satisfy the regulatory requirements for the production of those materials and products may affect the regulatory clearance of our contract manufacturers' facilities generally. If the FDA or a comparable foreign regulatory agency does not approve these facilities for the manufacture of our product candidates or if it withdraws its approval in the future, we may need to find alternative manufacturing facilities, which would adversely impact our ability to develop, obtain regulatory approval for or market our product candidates.

***If we do not establish successful collaborations, we may have to alter our development and commercialization plans for the bempedoic acid / ezetimibe combination pill and bempedoic acid.***

Our drug development programs and commercialization plans for the bempedoic acid / ezetimibe combination pill and bempedoic acid will require substantial additional cash to fund expenses. We may develop and initially commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid in the United States without a partner. However, in order to pursue the broader statin resistant market in the United States, we may also enter into a partnership or co-promotion arrangement with an established pharmaceutical company that has a larger sales force and we may enter into collaborative arrangements to develop and commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid outside of the United States. We will face significant competition in seeking appropriate collaborators and these collaboration agreements are complex and time-consuming to negotiate. We may not be able to negotiate collaborations on acceptable terms, or at all. If that were to occur, we may have to curtail the development or delay commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid in certain geographies, reduce the scope of our sales or marketing activities, reduce the scope of our commercialization plans, 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 outside of the United States on our own, we may need to obtain additional capital, which may not be available to us on acceptable terms, or at all.

***If a collaborative partner terminates or fails to perform its obligations under an agreement with us, the commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid could be delayed or terminated.***

We are not currently party to any collaborative arrangements for the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid or similar arrangements, although we may pursue such arrangements before any commercialization of the bempedoic acid / ezetimibe



combination pill or bempedoic acid outside of the United States or to further commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid in the broader statin resistant market in the United States, if approved. If we are successful in entering into collaborative arrangements for the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid or similar arrangements and any of our collaborative partners does not devote sufficient time and resources to a collaboration arrangement with us, we may not realize the potential commercial benefits of the arrangement, and our results of operations may be materially adversely affected. In addition, if any such future collaboration partner were to breach or terminate its arrangements with us, the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid could be delayed, curtailed or terminated because we may not have sufficient financial resources or capabilities to continue commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid on our own in such locations.

Much of the potential revenue from future collaborations may consist of contingent payments, such as payments for achieving regulatory milestones or royalties payable on sales of drugs. The milestone and royalty revenue that we may receive under these collaborations will depend upon our collaborators' ability to successfully develop, introduce, market and sell new products. In addition, collaborators may decide to enter into arrangements with third parties to commercialize products developed under collaborations using our technologies, which could reduce the milestone and royalty revenue that we may receive, if any. Future collaboration partners may fail to develop or effectively commercialize products using our products or technologies because they:

- decide not to devote the necessary resources due to internal constraints, such as limited personnel with the requisite expertise, limited cash resources or specialized equipment limitations, or the belief that other drug development programs may have a higher likelihood of obtaining marketing approval or may potentially generate a greater return on investment;
- decide to pursue other technologies or develop other product candidates, either on their own or in collaboration with others, including our competitors, to treat the same diseases targeted by our own collaborative programs;
- do not have sufficient resources necessary to carry the product candidate through clinical development, marketing approval and commercialization; or
- cannot obtain the necessary marketing approvals.

Competition may negatively impact a partner's focus on and commitment to the bempedoic acid / ezetimibe combination pill or bempedoic acid and, as a result, could delay or otherwise negatively affect the commercialization of the bempedoic acid / ezetimibe combination pill or bempedoic acid outside of the United States or in the broader statin resistant market in the United States. If future collaboration partners fail to develop or effectively commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid for any of these reasons, our sales of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, may be limited, which would have a material adverse effect on our operating results and financial condition.

#### **Risks Related to General Business, Employee Matters and Managing Growth**

***We will need to develop and expand our company, and we may encounter difficulties in managing this development and expansion, which could disrupt our operations.***

We expect that we will continue to increase our workforce and the scope of our operations. To manage our anticipated development and expansion, 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. Also, our management may need to divert a disproportionate amount of its attention away from its day-to-day activities and devote a substantial amount of time to managing

these development activities. Due to our limited resources, we may not be able to effectively manage the expansion of our operations or recruit and train additional qualified personnel. This may result in weaknesses in our infrastructure; or give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs and may divert financial resources from other projects, such as the development of the bempedoic acid / ezetimibe combination pill or bempedoic acid. If our management is unable to effectively manage our expected development and expansion, our expenses may increase more than anticipated, our ability to generate or increase our revenue could be reduced and we may not be able to implement our business strategy. Our future financial performance and our ability to commercialize the bempedoic acid / ezetimibe combination pill or bempedoic acid, if approved, and compete effectively will depend, in part, on our ability to effectively manage the future development and expansion of our company.

***Our future success depends on our ability to retain members of our senior management team, and to attract, retain and motivate qualified personnel.***

We are highly dependent on members of our senior management team. We have entered into employment agreements with these individuals, but any employee may terminate his or her employment with us. Although we do not have any reason to believe that we will lose the services of these individuals in the foreseeable future, the loss of the services of these individuals might impede the achievement of our research, development and commercialization objectives. We rely on consultants and advisors, including scientific and clinical advisors, to assist us in formulating our development and commercialization strategy. Our consultants and advisors may be employed by employers other than us and may have commitments under consulting or advisory contracts with other entities that may limit their availability to us. Recruiting and retaining qualified scientific personnel and sales and marketing personnel will also be critical to our success. We may not be able to attract and retain these personnel on acceptable terms given the competition among numerous pharmaceutical and biotechnology companies for similar personnel. We also experience competition for the hiring of scientific personnel from universities and research institutions. Failure to succeed in clinical studies may make it more challenging to recruit and retain qualified scientific personnel.

***Our company lacks experience commercializing products, which may have a material adverse effect on our business.***

We will need to transition from a company with a development focus to a company capable of supporting commercial activities. We may be unsuccessful in making such a transition. Our company has never filed an NDA and has not yet demonstrated an ability to obtain marketing approval for or commercialize a product candidate. Therefore, our clinical development and regulatory approval process may involve more inherent risk, take longer, and cost more than it would if we were a company with a more significant operating history and had experience obtaining marketing approval for and commercializing a product candidate.

***Our employees may engage in misconduct or other improper activities, including violating applicable regulatory standards and requirements or engaging in insider trading, which could significantly harm our business.***

We are exposed to the risk of employee fraud or other misconduct. Misconduct by employees could include intentional failures to comply with the regulations of the FDA and applicable non-U.S. regulators, provide accurate information to the FDA and applicable non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the United States and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations

intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Employee misconduct could also involve the improper use of, including trading on, information obtained in the course of clinical studies, which could result in regulatory sanctions and serious harm to our reputation. We have adopted a code of conduct, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent this activity may be ineffective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

***In order to satisfy our obligations as a publicly traded company, we may need to hire qualified accounting and financial personnel with appropriate public company experience.***

As a relatively new public company, we need to establish and maintain effective disclosure and financial controls and our corporate governance practices that we have adopted. We may need to hire additional accounting and financial personnel with appropriate public company experience and technical accounting knowledge, and it may be difficult to recruit and maintain such personnel. Even if we are able to hire appropriate personnel, our existing operating expenses and operations will be impacted by the direct costs of their employment and the indirect consequences related to the diversion of management resources from product development efforts.

### **Risks Related to our Financial Position and Capital Requirements**

***We have not generated any revenue from the bempedoic acid / ezetimibe combination pill or bempedoic acid and may never be profitable.***

Our ability to become profitable depends upon our ability to generate revenue. To date, we have not generated any revenue from the bempedoic acid / ezetimibe combination pill or bempedoic acid, and we do not know when, or if, we will generate any revenue. We do not expect to generate significant revenue unless and until we obtain marketing approval of, and begin to sell, the bempedoic acid / ezetimibe combination pill and bempedoic acid. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- successfully complete our CLEAR Outcomes CVOT;
- successfully complete our global pivotal Phase 3 LDL-C lowering program for bempedoic acid;
- successfully complete our pivotal Phase 3 clinical study for the bempedoic acid / ezetimibe combination pill;
- commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid, if approved, by developing a sales force or entering into collaborations with third parties; and
- achieve market acceptance of the bempedoic acid / ezetimibe combination pill and bempedoic acid in the medical community and with third-party payors.

Absent our entering into a collaboration or partnership agreement, we expect to incur significant sales and marketing costs as we prepare to commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid. Even if we initiate and successfully complete our clinical program of the bempedoic acid / ezetimibe combination pill and bempedoic acid and achieve all clinical endpoints and the bempedoic acid / ezetimibe combination pill and bempedoic acid is approved for commercial sale, and despite expending these costs, the bempedoic acid / ezetimibe combination pill or bempedoic acid

may not be a commercially successful drug. We may not achieve profitability soon after generating product sales, if ever. If we are unable to generate product revenue, we will not become profitable and may be unable to continue operations without continued funding.

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

We may seek additional capital through a combination of private and public equity offerings, debt financings, royalty-based financings, collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of common stock or securities convertible or exchangeable into common stock, your ownership interest in our company will be diluted. In addition, the terms of any such securities may include liquidation or other preferences that materially adversely affect your rights as a stockholder. Debt financing, if available, would increase our fixed payment obligations. Debt or royalty-based financings may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic partnerships and licensing arrangements with third parties, we may have to relinquish valuable rights to the bempedoic acid / ezetimibe combination pill or bempedoic acid, our intellectual property, future revenue streams or grant licenses on terms that are not favorable to us.

***Our ability to use our net operating loss carryforwards may be subject to limitation.***

At December 31, 2017, we had United States federal net operating loss carryforwards of approximately \$347.4 million and state net operating loss carryforwards of approximately \$327.8 million. Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, if a corporation undergoes an "ownership change," the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes, such as research tax credits, to offset its post-change income may be limited. In general, an "ownership change" will occur if there is a cumulative change in our ownership by "5-percent shareholders" that exceeds 50 percentage points over a rolling three-year period. Similar rules may apply under state tax laws. As a result of prior equity issuances and other transactions in our stock, we have previously experienced "ownership changes" under section 382 of the Code and comparable state tax laws. We may also experience ownership changes in the future as a result of future transactions in our stock. As a result, if we earn net taxable income, our ability to use our pre-change net operating loss carryforwards or other pre-change tax attributes to offset United States federal and state taxable income is subject to limitations. The effect of the enactment of the TCJA was to reduce our corporate statutory income tax rate from 34% to 21%. This may cause a reduction in the economic benefit of our net operating loss carryforwards and other deferred tax assets available to the Company.

***Complying with public company reporting and other obligations may strain our financial and managerial resources. Additionally, we are obligated to develop and maintain proper and effective internal control over financial reporting, but we may not complete our analysis of our internal control over financial reporting in a timely manner or these internal controls may not be determined to be effective, either of which may harm investor confidence in us and the value of our common stock.***

As a public company, we are required to comply with applicable provisions of the Sarbanes-Oxley Act of 2002, as well as other rules and regulations promulgated by the SEC and the NASDAQ Stock Market LLC, or NASDAQ, which results in significant continuing legal, accounting, administrative and other costs and expenses. The listing requirements of The NASDAQ Global Market require that we satisfy certain corporate governance requirements relating to director independence, distributing annual and interim reports, stockholder meetings, approvals and voting, soliciting proxies, conflicts of interest

and a code of conduct. Our management and other personnel need to devote a substantial amount of time to ensure that we comply with all of these requirements.

We are subject to Section 404 of the Sarbanes-Oxley Act of 2002, or Section 404, and the related rules of the SEC that generally require our management and independent registered public accounting firm to report on the effectiveness of our internal control over financial reporting. Section 404 requires an annual management assessment, as well as an opinion from our independent registered public accounting firm, on the effectiveness of our internal control over financial reporting.

We are in the costly and challenging process of evaluating and testing our internal controls for the purpose of providing the reports required by these rules. We may not be able to complete our evaluation, testing and any required remediation in a timely fashion. During the course of our review and testing, we may identify deficiencies and be unable to remediate them before we must provide the required reports. Furthermore, if we have a material weakness in our internal control over financial reporting, we may not detect errors on a timely basis and our financial statements may be materially misstated. We or our independent registered public accounting firm may not be able to conclude on an ongoing basis that we have effective internal control over financial reporting, which could harm our operating results, cause investors to lose confidence in our reported financial information and cause the trading price of our stock to fall. In addition, we are required to timely file accurate quarterly and annual reports with the SEC under the Securities Exchange Act of 1934, or the Exchange Act, as amended. In order to report our results of operations and financial statements on an accurate and timely basis, we depend on CROs to provide timely and accurate notice of their costs to us. Any failure to report our financial results on an accurate and timely basis could result in sanctions, lawsuits, delisting of our shares from The NASDAQ Global Market or other adverse consequences that would materially harm our business.

### **Risks Related to the Securities Markets and Investment in our Common Stock**

***Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

At December 31, 2017, our executive officers, directors and entities affiliated with certain of our directors beneficially owned approximately 17.7% of our outstanding voting common stock. These stockholders have the ability to influence us through their ownership position. These stockholders may be able to determine the outcome of all matters requiring stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

***We may be at an increased risk of securities class action litigation.***

Historically, 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 biotechnology and pharmaceutical companies have experienced significant stock price volatility in recent years. For example, a purported securities class action lawsuit was filed in January 2016 naming us and certain of our officers as defendants. In December 2016, the court granted our motion to dismiss with prejudice and entered judgment in our favor. In May 2017, the court denied plaintiffs' motion to alter or amend that judgment. On June 19, 2017, plaintiffs filed a notice of appeal to the Sixth Circuit Court of Appeals and on September 14, 2017, they filed their opening brief in support of the appeal. The appeal was fully briefed on December 7, 2017. Additionally, in December 2016, a purported derivative action was filed in Delaware against certain of our directors and officers. Any lawsuit to which we or our directors or officers are a party, with or without merit, may result in an unfavorable judgment. We also

may decide to settle lawsuits on unfavorable terms. Any such negative outcome could result in payments of substantial damages or fines, damage to our reputation or adverse changes to our offerings or business practices. Any of these results could adversely affect our business. In addition, defending claims is costly and can impose a significant burden on our management. This proceeding and any others in which we may become involved could result in substantial costs and a diversion of management's attention and resources, which could harm our business.

***If securities or industry analysts cease publishing research or reports or publish misleading, inaccurate or unfavorable research about us, our business or our market, our stock price and trading volume could decline.***

The trading market for our common stock is influenced by the research and reports that securities or industry analysts publish about us, our business, our market or our competitors. We only recently started receiving research coverage by securities and industry analysts. If one or more of the industry analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, or provides more favorable relative recommendations about our competitors, our stock price would likely decline. If one or more of these analysts ceases coverage of us or fails to publish reports on us regularly, demand for our stock could decrease, which could cause our stock price or trading volume to decline.

***Anti-takeover provisions in our charter documents and under Delaware law could make an acquisition of us, even one that 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 certificate of incorporation and bylaws may delay or prevent an acquisition of us or a change in our management. These provisions include a classified board of directors, a prohibition on actions by written consent of our stockholders and the ability of our board of directors to issue preferred stock without stockholder approval. In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporation Law, which limits the ability of stockholders owning in excess of 15% of our outstanding voting stock to merge or combine with us. Although we believe these provisions collectively provide for an opportunity to obtain greater value for stockholders by requiring potential acquirors to negotiate with our board of directors, they would apply even if an offer rejected by our board were considered beneficial by some stockholders. In addition, 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, which is responsible for appointing the members of our management.

***We do not intend to pay dividends on our common stock and, consequently, your ability to achieve a return on your investment will depend on appreciation in the price of our common stock.***

We have never declared or paid any cash dividend on our common stock and do not currently intend to do so in the foreseeable future. We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends in the foreseeable future. Therefore, the success of an investment in shares of our common stock will depend upon any future appreciation in their value. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which you purchased them. Additionally, our ability to pay dividends on our common stock is limited by restrictions under the terms of our Credit Facility with Oxford Finance LLC.

**Item 1B. Unresolved Staff Comments**

None.

**Item 2. Properties**

Our corporate headquarters are located in Ann Arbor, Michigan where we lease and occupy approximately 7,900 square feet of office space. We lease and occupy an additional 5,500 square feet of office space in Ann Arbor, Michigan to support our clinical development operations. We believe our current facilities will be sufficient to meet our needs until expiration.

**Item 3. Legal Proceedings**

On January 12, 2016, a purported stockholder of the Company filed a putative class action lawsuit in the United States District Court for the Eastern District of Michigan, against us and Tim Mayleben, captioned *Kevin L. Dougherty v. Esperion Therapeutics, Inc., et al.* (No. 16-cv-10089). The lawsuit alleges that we and Mr. Mayleben violated Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and SEC Rule 10b-5 by allegedly failing to disclose in an August 17, 2015, public statement that the FDA would require a cardiovascular outcomes trial before approving our lead product candidate. The lawsuit seeks, among other things, compensatory damages in connection with an allegedly inflated stock price between August 18, 2015, and September 28, 2015, as well as attorneys' fees and costs. On May 20, 2016, an amended complaint was filed in the lawsuit and on July 5, 2016, we filed a motion to dismiss the amended complaint. On December 27, 2016, the court granted our motion to dismiss with prejudice and entered judgment in our favor. On January 24, 2017, the plaintiffs in this lawsuit filed a motion to alter or amend the judgment. In May 2017, the court denied the plaintiff's motion to alter or amend the judgment. On June 19, 2017, the plaintiffs filed a notice of appeal to the Sixth Circuit Court of Appeals and on September 14, 2017, they filed their opening brief in support of the appeal. The appeal was fully briefed on December 7, 2017.

On December 15, 2016, a purported stockholder of the Company filed a derivative lawsuit in the Court of Chancery of the State of Delaware against Tim Mayleben, Roger Newton, Mary McGowan, Nicole Vitullo, Dov Goldstein, Daniel Janney, Antonio Gotto Jr., Mark McGovern, Gilbert Omenn, Scott Braunstein, and Patrick Enright. The Company is named as a nominal defendant. The lawsuit alleges that the defendants breached their fiduciary duties to the Company when they made or approved improper statements on August 17, 2015, regarding our lead product candidate's path to FDA approval, and failed to ensure that reliable systems of internal controls were in place at the Company. The lawsuit seeks, among other things, any damages sustained by the Company as a result of the defendants' alleged breaches of fiduciary duties, including damages related to the above-referenced securities class action, an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures, restitution from the defendants, and attorneys' fees and costs. In light of, among other things, the early stage of the litigation, we are unable to predict the outcome of this matter and are unable to make a meaningful estimate of the amount or range of loss, if any, that could result from an unfavorable outcome.

In the future, we may become party to legal matters and claims arising in the ordinary course of business, the resolution of which we do not anticipate would have a material adverse impact on our financial position, results of operations or cash flows.

**Item 4. Mine Safety Disclosures**

Not applicable.

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

Our common stock began trading on the NASDAQ Global Market on June 26, 2013, under the symbol "ESPR". Prior to that time there was no public market for our common stock. Shares sold in our initial public offering which closed on July 1, 2013, were priced at \$14.00 per share.

On December 31, 2017, the closing price for our common stock as reported on the NASDAQ Global Market was \$65.84. The following table sets forth the high and low sales prices per share of our common stock as reported on the NASDAQ Global Market for the period indicated:

<u>Year Ended December 31, 2017</u>	<u>High</u>	<u>Low</u>
First Quarter	\$ 48.21	\$ 10.71
Second Quarter	\$ 49.69	\$ 30.95
Third Quarter	\$ 57.38	\$ 43.06
Fourth Quarter	\$ 68.60	\$ 42.55

<u>Year Ended December 31, 2016</u>	<u>High</u>	<u>Low</u>
First Quarter	\$ 22.43	\$ 12.61
Second Quarter	\$ 20.19	\$ 9.58
Third Quarter	\$ 14.85	\$ 9.75
Fourth Quarter	\$ 14.33	\$ 9.40

**Stockholders**

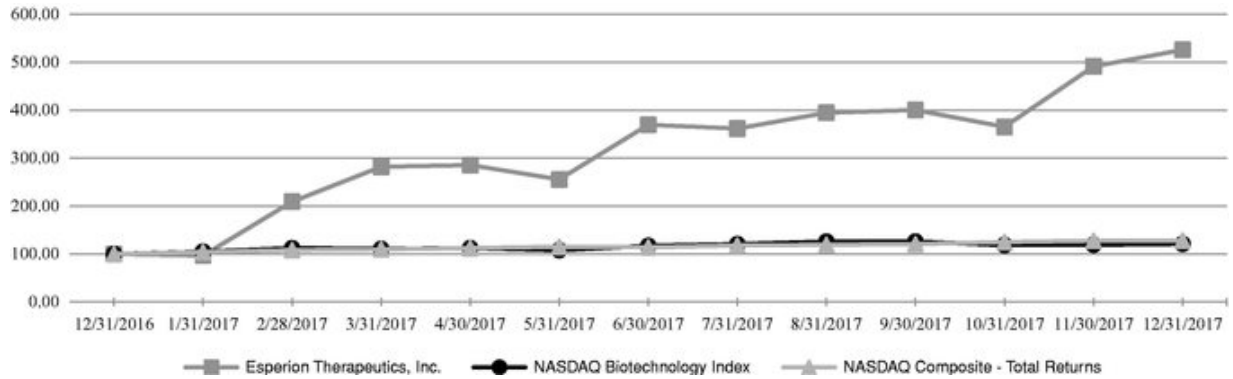
As of February 1, 2018, there were 11 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers.

**Performance Graph**

The following graph illustrates a comparison of the total cumulative stockholder return for our common stock since January 1, 2017, to two indices: the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The graph assumes an initial investment of \$100 on January 1, 2017, in our common stock, the stocks comprising the NASDAQ Composite Index, and the stocks comprising the NASDAQ Biotechnology Index. Historical stockholder return is not necessarily indicative of the performance to be expected for any future periods.



**Comparison of 1 Year Cumulative Total Return\***  
**Among Esperion Therapeutics, Inc., the NASDAQ Composite Index and**  
**the NASDAQ Biotechnology Index**



\* \$100 invested on January 1, 2017, in stock or index. Fiscal Year ending December 31.

The performance graph shall not be deemed to be incorporated by reference by means of any general statement incorporating by reference this Form 10-K into any filing under the Securities Act of 1933, as amended or the Exchange Act, except to the extent that we specifically incorporate such information by reference, and shall not otherwise be deemed filed under such acts.

**Dividend Policy**

We have never paid or declared any cash dividends on our common stock, and we do not anticipate paying any cash dividends on our common stock in the foreseeable future. We intend to retain all available funds and any future earnings to fund the development and expansion of our business. Any future determination to pay dividends will be at the discretion of our board of directors and will depend upon a number of factors, including our results of operations, financial condition, future prospects, contractual restrictions, restrictions imposed by applicable law and other factors our board of directors deems relevant. Additionally, our ability to pay dividends on our common stock is limited by restrictions under the terms of our Credit Facility with Oxford Finance LLC.

**Equity Compensation Plans**

The information required by Item 5 of Form 10-K regarding equity compensation plans is incorporated herein by reference to Item 11 of Part III of this Annual Report.

**Issuer 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.

**Purchases of Equity Securities by the Issuer and Affiliated Purchasers**

None.

**Item 6. Selected Financial Data**

The selected financial data set forth below is derived from our audited financial statements and may not be indicative of future operating results. The following selected financial data should be read in conjunction with Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations" and the financial statements and the notes thereto included elsewhere in this report. The selected financial data in this section are not intended to replace our financial statements and the related notes. Our historical results are not necessarily indicative of our future results.

	Three Months Ended December 31,					Years Ended December 31,				
	2017	2016	2015	2014	2013	2017	2016	2015	2014	2013
	(in thousands, except share and per share data)									
Operating expenses:										
Research and development	\$ 33,439	\$ 24,881	\$ 7,956	\$ 6,200	\$ 7,338	\$ 147,603	\$ 57,868	\$ 29,802	\$ 25,302	\$ 16,014
General and administrative	5,257	4,404	5,278	3,180	2,398	21,379	18,282	20,238	10,922	6,745
Total operating expenses	38,696	29,285	13,234	9,380	9,736	168,982	76,150	50,040	36,224	22,759
Loss from operations	(38,696)	(29,285)	(13,234)	(9,380)	(9,736)	(168,982)	(76,150)	(50,040)	(36,224)	(22,759)
Total other income (expense)	805	329	112	(77)	46	1,994	1,172	256	(151)	(3,329)
Net loss	\$ (37,891)	\$ (28,956)	\$ (13,122)	\$ (9,457)	\$ (9,690)	\$ (166,988)	\$ (74,978)	\$ (49,784)	\$ (36,375)	\$ (26,088)
Net loss per common share (basic and diluted)	\$ (1.44)	\$ (1.29)	\$ (0.58)	\$ (0.49)	\$ (0.63)	\$ (6.98)	\$ (3.33)	\$ (2.26)	\$ (2.22)	\$ (3.31)
Weighted average shares outstanding (basic and diluted)	26,222,397	22,554,418	22,515,136	19,276,639	15,340,713	23,933,273	22,544,475	22,019,818	16,374,102	7,885,921

The table below presents a summary of our balance sheet data as of December 31, 2017, 2016, 2015, 2014 and 2013:

	As of December 31,				
	2017	2016	2015	2014	2013
	(in thousands)				
<b>Balance Sheet Data:</b>					
Cash and cash equivalents	\$ 34,468	\$ 38,165	\$ 77,336	\$ 85,038	\$ 56,537
Working capital	170,780	197,988	208,769	101,208	56,417
Investments	239,151	204,324	215,240	56,544	21,062
Total assets	277,835	245,213	295,572	143,276	78,294
Total long-term debt	—	1,022	2,688	4,231	—
Common stock	26	23	23	20	15
Accumulated deficit	(396,291)	(229,200)	(154,222)	(104,438)	(68,063)
Total stockholders' equity	244,691	228,602	287,259	133,554	74,091

## Item 7. Management's Discussion and Analysis of Financial Condition and Results of Operation

*You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes appearing elsewhere in this Annual Report on Form 10-K. In addition to historical information, this discussion and analysis contains forward-looking statements that involve risks, uncertainties and assumptions. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of certain factors. We discuss factors that we believe could cause or contribute to these differences below and elsewhere in this report, including those set forth under Item 1A. "Risk Factors" and under "Forward-Looking Statements" in this Annual Report on Form 10-K.*

### Overview

#### Corporate Overview

We are the Lipid Management Company, a late-stage pharmaceutical company focused on developing and commercializing complementary, convenient, cost-effective, once-daily, oral therapies for the treatment of patients with elevated LDL-C. Through scientific and clinical excellence, and a deep understanding of cholesterol biology, the experienced lipid management team at Esperion is committed to developing new LDL-C lowering therapies that will make a substantial impact on reducing global cardiovascular disease, or CVD; the leading cause of death around the world. Bempedoic acid and our lead product candidate, the bempedoic acid / ezetimibe combination pill, are targeted therapies that have been shown to significantly reduce elevated LDL-C levels in patients with hypercholesterolemia, including patients inadequately treated with current lipid-modifying therapies.

The clinical development program for the bempedoic acid / ezetimibe combination pill consists of a single pivotal Phase 3 clinical study (1002FDC-053) in patients with hypercholesterolemia and with atherosclerotic cardiovascular disease, or ASCVD, and/or heterozygous familial hypercholesterolemia, or HeFH, including high CVD risk primary prevention patients, whose LDL-C is not adequately controlled despite receiving maximally tolerated lipid-modifying background therapy. 1002FDC-053 initiated in November 2017 and we expect to report top-line results in August 2018.

The global pivotal Phase 3 clinical development program for bempedoic acid, consisting of four clinical studies, fully enrolled approximately 3,600 high CVD risk patients with hypercholesterolemia and ASCVD and/or HeFH, or who are high CVD risk primary prevention, on optimized background lipid-modifying therapy and with elevated levels of LDL-C. These patients are on two distinct types of background lipid-modifying therapy: 1) patients on their maximally tolerated statin therapy, and 2) patients who are only able to tolerate less than the lowest approved daily starting dose of a statin, and can be considered statin intolerant. In March 2018, we expect to report top-line results from the first of the Phase 3 studies, Study 4 (1002-048). In May 2018, we expect to report top-line results from the 52-week long-term safety study, Study 1 (1002-040) and top-line results from Study 3 (1002-046). In September 2018, top-line results are expected from Study 2 (1002-047).

We intend to use positive results from our Phase 3 bempedoic acid / ezetimibe combination pill and bempedoic acid programs with a total of 4,000 patients to support our global regulatory submissions for tandem LDL-C lowering indications in the U.S. by the first quarter of 2019 and in Europe by the second quarter of 2019.

We are also conducting a global cardiovascular outcomes trial, or CVOT,—known as **C**holesterol **L**owering via **B**empedoic Acid, an **A**CL-inhibiting **R**egimen (CLEAR) Outcomes, for bempedoic acid in patients with hypercholesterolemia and high CVD risk and who can be considered statin intolerant. We initiated the CLEAR Outcomes CVOT in December 2016, and intend to use positive results from this CVOT to support our submissions for a CV risk reduction indication in the U.S. and Europe by 2022.

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In December 2017, we submitted an investigational new drug, or IND, application to the Food and Drug Administration, or FDA, for a reformulated tablet of bempedoic acid for a nonalcoholic steatohepatitis, or NASH, indication, which was accepted in January 2018.

We were incorporated in Delaware in January 2008, and commenced our operations in April 2008. Since our inception, we have focused substantially all of our efforts and financial resources on developing bempedoic acid. We have funded our operations to date primarily through proceeds from sales of preferred stock, convertible promissory notes and warrants, public offerings of common stock and the incurrence of indebtedness, and we have incurred losses in each year since our inception. We own the exclusive worldwide rights to bempedoic acid.

On August 15, 2017, we completed an underwritten public offering of 3,100,000 shares of common stock. We also granted the underwriters a 30-day option to purchase up to 465,000 additional shares of our common stock, which was exercised in full in September 2017. All the shares were offered by us at a price to the public of \$49.00 per share. The aggregate net proceeds received by us from the offering were \$164.0 million, net of underwriting discounts and commissions and expenses payable by us.

We have not commenced principal operations and do not have any products approved for sale. To date, we have not generated any revenue. We have never been profitable and our net losses were \$167.0 million, \$75.0 million, and \$49.8 million for the years ended December 31, 2017, 2016 and 2015, respectively. Substantially all of our net losses resulted from costs incurred in connection with research and development programs, general and administrative costs associated with our operations. We expect to incur significant additional research and development expenses and operating losses for the foreseeable future. We expect our expenses to increase in connection with our ongoing activities, including, among others:

- completing the clinical development activities for bempedoic acid, including the completion of the global pivotal Phase 3 LDL-C lowering program and the CLEAR Outcomes CVOT;
- completing the clinical development activities for the bempedoic acid / ezetimibe combination pill;
- seeking regulatory approval for the bempedoic acid / ezetimibe combination pill and bempedoic acid;
- commercializing the bempedoic acid / ezetimibe combination pill and bempedoic acid; and
- operating as a public company.

Accordingly, we will need additional financing to support our continuing operations. We will seek to fund our operations through public or private equity or debt financings or through other sources, which may include collaborations with third parties. Adequate additional financing may not be available to us on acceptable terms, or at all. Our failure to raise capital as and when needed would have a material adverse effect on our financial condition and our ability to pursue our business strategy or continue operations. We will need to generate significant revenues to achieve profitability, and we may never do so.

### ***Product Overview***

Through the complementary mechanisms of action of inhibition of cholesterol synthesis (bempedoic acid) and inhibition of cholesterol absorption (ezetimibe), the bempedoic acid / ezetimibe combination pill is our lead, non-statin, orally available, once-daily, LDL-C lowering therapy. Inhibition of ATP Citrate Lyase, or ACL, by bempedoic acid reduces cholesterol biosynthesis and lowers LDL-C by up-regulating the LDL receptor. Inhibition of Niemann-Pick C1-Like 1 by ezetimibe results in reduced absorption of cholesterol from the gastrointestinal tract, thereby reducing delivery of cholesterol to the liver, which in turn upregulates the LDL receptors. Previously completed Phase 2

data demonstrated that this safe and well tolerated combination results in a 48 percent lowering of LDL-C, a 26 percent reduction in high sensitivity C-reactive protein, or hsCRP, and may potentially be associated with a lower occurrence of muscle-related side effects. The bempedoic acid / ezetimibe combination pill is being developed for patients at high CVD risk with hypercholesterolemia.

With a targeted mechanism of action, bempedoic acid is a first-in-class, complementary, orally available, once-daily ACL inhibitor that reduces cholesterol biosynthesis and lowers LDL-C by up-regulating the LDL receptor, and may potentially be associated with a lower occurrence of muscle-related side effects. Completed Phase 1 and 2 studies conducted in more than 1,300 patients and over 800 patients treated with bempedoic acid have produced clinically relevant LDL-C lowering results of up to 30 percent as monotherapy and an incremental 20+ percent when added to stable statin therapy. Bempedoic acid is being developed for patients at high CVD risk with hypercholesterolemia. We acquired the rights to bempedoic acid from Pfizer in 2008. We own the exclusive worldwide rights to bempedoic acid and we are not obligated to make any royalty or milestone payments to Pfizer.

During the year ended December 31, 2017, we incurred \$111.8 million in expenses related to the four studies in our global pivotal Phase 3 LDL-C lowering program, our Phase 3 (1002FDC-053) efficacy and safety study of the bempedoic acid / ezetimibe combination pill, our CLEAR Outcomes CVOT, our Phase 2 (1002-038) clinical study of the bempedoic acid / ezetimibe combination plus statin oral therapy, our Phase 2 (1002-39) clinical study of bempedoic acid when added-on to an injectable proprotein convertase subtilisin/kexin type 9 inhibitor, or PCSK9i, and other clinical pharmacology studies.

During the year ended December 31, 2016, we incurred \$36.2 million in expenses related to the four studies in our global pivotal Phase 3 LDL-C lowering program, our CLEAR Outcomes CVOT, our Phase 2 (1002-035) PK/PD clinical study of bempedoic acid in patients treated with atorvastatin 80 mg and our Phase 1 (1002-037) clinical pharmacology study to assess the safety and tolerability of bempedoic acid, as well as the effects of bempedoic acid on the PK of single doses of four high-dose statins, and other clinical pharmacology studies

During the year ended December 31, 2015, we incurred \$12.0 million in expenses related to our Phase 2 (1002-009) clinical study in patients with elevated LDL-C already receiving statin therapy, our Phase 2 (1002-014) exploratory clinical safety study in patients with both elevated LDL-C and hypertension, our 52-week global pivotal Phase 3 long-term safety and tolerability study (Study 1), and other clinical pharmacology studies.

## **Financial Operations Overview**

### ***Revenue***

To date, we have not generated any revenue. In the future, we may never generate revenue from the sale of the bempedoic acid / ezetimibe combination pill or bempedoic acid or other product candidates. If we fail to complete the development of the bempedoic acid / ezetimibe combination pill or bempedoic acid or any other product candidates and secure approval from regulatory authorities, our ability to generate future revenue and our results of operations and financial position will be adversely affected.

### ***Research and Development Expenses***

Since our inception, we have focused our resources on our research and development activities, including conducting nonclinical, preclinical and clinical studies. Our research and development

expenses consist primarily of costs incurred in connection with the development of the bempedoic acid / ezetimibe combination pill and bempedoic acid, which include:

- expenses incurred under agreements with consultants, contract research organizations, or CROs, and investigative sites that conduct our preclinical and clinical studies;
- the cost of acquiring, developing and manufacturing clinical study materials, including the procurement of ezetimibe in our continued development of our bempedoic acid / ezetimibe combination pill;
- employee-related expenses, including salaries, benefits, stock-based compensation and travel expenses;
- allocated expenses for rent and maintenance of facilities, insurance and other supplies; and
- costs related to compliance with regulatory requirements.

We expense research and development costs as incurred. To date, substantially all of our research and development work has been related to the bempedoic acid / ezetimibe combination pill and bempedoic acid. Costs for certain development activities, such as clinical studies, are recognized based on an evaluation of the progress to completion of specific tasks using data such as patient enrollment, clinical site activations or information provided to us by our vendors. Our direct research and development expenses consist principally of external costs, such as fees paid to investigators, consultants, central laboratories and CROs in connection with our clinical studies. We do not allocate acquiring and manufacturing clinical study materials, salaries, stock-based compensation, employee benefits or other indirect costs related to our research and development function to specific programs.

We expect to continue to incur significant research and development expenses in the foreseeable future. Costs associated with bempedoic acid will continue to accumulate as we further its clinical development, including in connection with the continuation of our global pivotal Phase 3 LDL-C lowering program and our CLEAR Outcomes CVOT. We also expect to continue to incur significant research and development expenses as we pursue the clinical development of the bempedoic acid / ezetimibe combination pill. We cannot determine with certainty the duration and completion costs associated with the ongoing or future clinical studies of the bempedoic acid / ezetimibe combination pill and bempedoic acid. Also, we cannot conclude with certainty if, or when, we will generate revenue from the commercialization and sale of the bempedoic acid / ezetimibe combination pill or bempedoic acid, if ever. We may never succeed in obtaining regulatory approval for the bempedoic acid / ezetimibe combination pill or bempedoic acid. The duration, costs and timing associated with the development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid will depend on a variety of factors, including uncertainties associated with the results of our clinical studies and our ability to obtain regulatory approval. For example, if the FDA or another regulatory authority were to require us to conduct clinical studies beyond those that we currently anticipate will be required for the completion of clinical development or post-commercialization clinical studies of the bempedoic acid / ezetimibe combination pill or bempedoic acid, or if we experience significant delays in enrollment in any of our clinical studies, we could be required to expend significant additional financial resources and time on the completion of clinical development or post-commercialization clinical studies of the bempedoic acid / ezetimibe combination pill and bempedoic acid.

#### ***General and Administrative Expenses***

General and administrative expenses primarily consist of salaries and related costs for personnel, including stock-based compensation, associated with our executive, accounting and finance, operational and other administrative functions. Other general and administrative expenses include facility-related costs, communication expenses and professional fees for legal, patent prosecution, protection and review, consulting and accounting services.

We anticipate that our general and administrative expenses will increase in the future in connection with the continued research and development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid, increases in our headcount, expansion of our information technology infrastructure, and increased expenses associated with being a public company and complying with exchange listing and Securities and Exchange Commission, or SEC, requirements. These increases will likely include higher legal, compliance, accounting and investor and public relations expenses.

### ***Interest Expense***

Interest expense consists primarily of cash interest costs associated with our credit facility and non-cash interest costs associated with the amortization of the related debt discount, deferred issuance costs and final payment fee.

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

Our discussion and analysis of our financial condition and results of operations is based on our financial statements, which have been prepared in accordance with generally accepted accounting principles in the United States. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities and expenses and the disclosure of contingent assets and liabilities in our financial statements. We evaluate our estimates and judgments on an ongoing basis, including those related to accrued expenses and stock-based compensation. We base our estimates on historical experience, known trends and events, contractual milestones and other various factors that are believed to be reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Our actual results may differ from these estimates under different assumptions or conditions.

Our significant accounting policies are described in more detail in Note 2 to our audited financial statements appearing elsewhere in this Annual Report on Form 10-K. We believe the following accounting policies to be most critical to understanding our results and financial operations.

### ***Accrued Clinical Development Costs***

As part of the process of preparing our financial statements we are required to estimate our accrued expenses. We base our accrued expenses related to clinical studies on estimates of patient enrollment and related expenses at clinical investigator sites as well as estimates for the services received and efforts expended pursuant to contracts with multiple research institutions and CROs that conduct and manage clinical studies on our behalf. We generally accrue expenses related to clinical studies based on contracted amounts applied to the level of patient enrollment and activity according to the protocol. If timelines or contracts are modified based upon changes in the clinical study protocol or scope of work to be performed, we modify our estimates of accrued expenses accordingly on a prospective basis. If we do not identify costs that we have begun to incur or if we underestimate or overestimate the level of services performed or the costs of these services, our actual expenses could differ from our estimates. We do not anticipate the future settlement of existing accruals to differ materially from our estimates.

### ***Stock-Based Compensation***

We typically grant stock-based compensation to new employees in connection with their commencement of employment and to existing employees in connection with annual performance reviews. We account for all stock-based compensation payments issued to employees, consultants and directors using an option-pricing model for estimating fair value. Accordingly, stock-based

compensation expense is measured based on the estimated fair value of the awards on the date of grant. In accordance with authoritative guidance, the fair value of non-employee stock-based awards is remeasured as the awards vest, and the resulting value, if any, is recognized as expense during the period the related services are rendered.

*Significant Factors, Assumptions and Methodologies Used in Determining Fair Value*

We estimate the fair value of our stock-based awards to employees, consultants and directors using the Black-Scholes option-pricing model. The Black-Scholes model requires the input of subjective assumptions, including (a) the per share fair value of our common stock, (b) the expected stock price volatility, (c) the calculation of the expected term of the award, (d) the risk free interest rate and (e) expected dividends. Due to our limited operating history and a lack of company-specific historical and implied volatility data, we have based our estimate of expected volatility on the historical volatility of a group of similar companies, which are publicly traded. When selecting these public companies on which we have based our expected stock price volatility, we selected companies with comparable characteristics to us, including enterprise value, risk profiles, position within the industry, and with historical share price information sufficient to meet the expected life of our stock-based awards. The historical volatility data was computed 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 have estimated the expected life of our employee stock options using the "simplified" method, whereby, the expected life equals the arithmetic 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 are based on the U.S. Treasury yield curve in effect during the period the options were granted. We have never paid, and do not expect to pay, dividends in the foreseeable future.

In accordance with the adoption of Accounting Standards Update, or ASU, 2016-09 on January 1, 2017, we elected to account for forfeitures as they occur. Prior to January 1, 2017, we were required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differed from our estimates. We used historical data to estimate pre-vesting option forfeitures and recorded stock-based compensation expense only for those awards that were expected to vest. To the extent that actual forfeitures differed from our estimates, the difference was recorded as a cumulative adjustment in the period the estimates were revised.

*Fair Value Estimate*

We are required to estimate the fair value of the common stock underlying our stock-based awards when performing the fair value calculations with the Black-Scholes option-pricing model. All options to purchase shares of our common stock are intended to be granted with an exercise price per share no less than the fair value per share of our common stock underlying those options on the date of grant, based on the information known to us on the date of grant.

***Recent Accounting Pronouncements***

In February 2016, the Financial Accounting Standards Board, or FASB, issued ASU 2016-02 which is intended to improve financial reporting about leasing transactions. The updated guidance will require a lessee to recognize assets and liabilities for leases with lease terms of more than twelve months. Consistent with current GAAP, the recognition, measurement and presentation of expenses and cash flows arising from a lease by a lessee primarily will depend on its classification as a capital or operating lease. Unlike current GAAP—which requires only capital leases to be recognized on the balance sheet—the updated guidance will require both types of leases to be recognized on the balance sheet. The standard is effective for public companies for fiscal years beginning after December 15, 2018, and interim periods within those years. Early adoption is permitted for annual or interim reporting periods for which the financial statements have not previously been issued. We do not believe the adoption of this standard will have a material impact on our financial position, results of operations or related financial statement disclosures.



In March 2016, the FASB issued ASU 2016-09 which includes provisions intended to simplify the various aspects related to how share-based payments are accounted for and presented in the financial statements. The updated guidance requires all income tax effects of awards to be recognized in the income statement when the awards vest or are settled. Additionally, under the updated guidance companies have to elect whether to account for forfeitures of share-based payments by (1) recognizing forfeitures as they occur or (2) estimating the number of awards expected to be forfeited and adjusting the estimate when it is likely to change, as was previously required. We adopted ASU 2016-09 effective January 1, 2017, and recognized approximately \$4.5 million of deferred tax assets that were not previously recognized on our balance sheet under the prior accounting guidance. The increase in the deferred tax assets was fully offset by an increase in the Company's valuation allowance. In addition, we made a policy election to account for forfeitures as they occur. The cumulative effect of adoption was an increase of \$0.1 million to both additional paid-in capital and accumulated deficit as of January 1, 2017. The remaining provisions adopted in ASU 2016-09 did not have a material impact to our balance sheets, statements of operations or statements of cash flows.

## Results of Operations

### *Comparison of the Years Ended December 31, 2017 and 2016*

The following table summarizes our results of operations for the years ended December 31, 2017 and 2016:

	Year Ended December 31,		Change
	2017	2016	
	(in thousands)		
<b>Operating Expenses:</b>			
Research and development	\$ 147,603	\$ 57,868	\$ 89,735
General and administrative	21,379	18,282	3,097
<b>Loss from operations</b>	<u>(168,982)</u>	<u>(76,150)</u>	<u>(92,832)</u>
Interest expense	(198)	(376)	178
Other income, net	2,192	1,548	644
<b>Net loss</b>	<u>\$ (166,988)</u>	<u>\$ (74,978)</u>	<u>\$ (92,010)</u>

### *Research and development expenses*

Research and development expenses for the year ended December 31, 2017, were \$147.6 million compared to \$57.9 million for the year ended December 31, 2016, an increase of \$89.7 million. The increase in research and development expenses was primarily related to the further clinical development of the bempedoic acid / ezetimibe combination pill and bempedoic acid, including costs to support the global pivotal Phase 3 studies, the CVOT, and increases in our headcount and stock-based compensation expense.

### *General and administrative expenses*

General and administrative expenses for the year ended December 31, 2017, were \$21.4 million compared to \$18.3 million for the year ended December 31, 2016, an increase of approximately \$3.1 million. The increase in general and administrative expenses was primarily attributable to costs to support public company operations, further increases in our headcount and stock-based compensation expense, and other costs to support our growth.

**Interest expense**

Interest expense for the year ended December 31, 2017, was \$0.2 million compared to \$0.4 million for the year ended December 31, 2016. Interest expense was related to our credit facility with Oxford Finance LLC.

**Other income, net**

Other income, net for the year ended December 31, 2017, was \$2.2 million compared to \$1.5 million for the year ended December 31, 2016. This increase was primarily related to a reduction in expense for the amortization of premiums and discounts on our investments.

**Results of Operations****Comparison of the Years Ended December 31, 2016 and 2015**

The following table summarizes our results of operations for the years ended December 31, 2016 and 2015:

	Year Ended December 31,		Change
	2016	2015	
	(in thousands)		
<b>Operating Expenses:</b>			
Research and development	\$ 57,868	\$ 29,802	\$ 28,066
General and administrative	18,282	20,238	(1,956)
<b>Loss from operations</b>	(76,150)	(50,040)	(26,110)
Other income (expense):			
Interest expense	(376)	(520)	144
Other income, net	1,548	776	772
<b>Net loss</b>	<u>\$ (74,978)</u>	<u>\$ (49,784)</u>	<u>\$ (25,194)</u>

**Research and development expenses**

Research and development expenses for the year ended December 31, 2016, were \$57.9 million compared to \$29.8 million for the year ended December 31, 2015, an increase of \$28.1 million. The increase in research and development expenses was primarily related to the further clinical development of bempedoic acid, including costs to support the initiation of the three global pivotal Phase 3 studies and the CVOT, and further increases in our headcount and stock-based compensation expense.

**General and administrative expenses**

General and administrative expenses for the year ended December 31, 2016, were \$18.3 million compared to \$20.2 million for the year ended December 31, 2015, a decrease of approximately \$1.9 million. The decrease in general and administrative expenses was primarily related to a reduction in pre-commercialization activities, partially offset by increases in costs to support public company operations, increases in our headcount, and other costs to support our growth.

**Interest expense**

Interest expense for the year ended December 31, 2016, was \$0.4 million compared to \$0.5 million for the year ended December 31, 2015. Interest expense was related to our credit facility with Oxford Finance LLC.

**Other income, net**

Other income, net for the year ended December 31, 2016, was \$1.5 million compared to \$0.8 million for the year ended December 31, 2015. This increase was primarily related to an increase in interest income earned on our cash, cash equivalents and investment securities.

**Liquidity and Capital Resources**

We have funded our operations to date primarily through proceeds from sales of preferred stock, convertible promissory notes and warrants, public offerings of common stock and the incurrence of indebtedness. In June 2014, we entered into a loan and security agreement (the credit facility) with Oxford Finance LLC whereby we received net proceeds of \$4.9 million from the issuance of secured promissory notes under a term loan as part of the facility. In October 2014, we sold 4,887,500 shares of common stock at a price of \$20.00 per share, less underwriting discounts and commissions, for net proceeds of \$91.6 million. In March 2015, we sold 2,012,500 shares of common stock at a price of \$100.00 per share, less underwriting discounts and commissions, for net proceeds of \$190.0 million. In August 2017, we completed an underwritten public offering of 3,100,000 shares of common stock. We also granted the underwriters a 30-day option to purchase up to 465,000 additional shares of our common stock, which was exercised in full in September 2017. All of the shares were offered by us at a price to the public of \$49.00 per share for net proceeds of \$164.0 million. To date, we have not generated any revenue and we anticipate that we will continue to incur losses for the foreseeable future.

As of December 31, 2017, our primary sources of liquidity were our cash and cash equivalents and available-for-sale investments, which totaled \$34.5 million and \$239.2 million, respectively. We invest our cash equivalents and investments in highly liquid, interest-bearing investment-grade and government securities to preserve principal.

The following table summarizes the primary sources and uses of cash for the periods presented below:

	Year Ended December 31,	
	2017	2016
	(in thousands)	
Cash used in operating activities	\$ (131,302)	\$ (47,730)
Cash (used in) provided by investing activities	(35,853)	10,118
Cash provided by (used in) financing activities	163,458	(1,559)
<b>Net decrease in cash and cash equivalents</b>	<b>\$ (3,697)</b>	<b>\$ (39,171)</b>

**Operating Activities**

We have incurred, and expect to continue to incur, significant costs in the areas of research and development, regulatory and other clinical study costs, associated with our development of the bempedoic acid / ezetimibe combination pill and bempedoic acid and our operations.

Net cash used in operating activities totaled \$131.3 million and \$47.7 million for the years ended December 31, 2017 and 2016, respectively. The primary use of our cash was to fund the development of the bempedoic acid / ezetimibe combination pill and bempedoic acid, adjusted for non-cash expenses such as stock-based compensation expense, depreciation and changes in working capital.

***Investing Activities***

Net cash used in investing activities of \$35.9 million for the year ended December 31, 2017, consisted primarily of purchases of highly liquid, interest bearing investment grade and government securities. Net cash provided by investing activities of \$10.1 million for the year ended December 31, 2016, consisted primarily of proceeds from maturities of highly liquid, interest bearing investment-grade and government securities.

***Financing Activities***

Net cash provided by financing activities of \$163.5 million for the year ended December 31, 2017, related primarily to the proceeds of our underwritten public offering of common stock. Net cash used in financing activities of \$1.6 million for the year ended December 31, 2016, related primarily to payments on our credit facility.

***Plan of Operations and Funding Requirements***

We expect to continue to incur significant expenses and operating losses for the foreseeable future as we progress through the clinical development programs for the bempedoic acid / ezetimibe combination pill and bempedoic acid. We estimate that current cash resources are sufficient to fund operations through the expected approvals of the bempedoic acid / ezetimibe combination pill and bempedoic acid in the first quarter of 2020. We will likely need to raise additional capital to continue to fund the further development and commercialization efforts for the bempedoic acid / ezetimibe combination pill and bempedoic acid and our operations and to complete the CLEAR Outcomes CVOT. We have based these estimates 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 and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid and the extent to which we may enter into collaborations with pharmaceutical partners regarding the development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development and commercialization of the bempedoic acid / ezetimibe combination pill and bempedoic acid. Our future funding requirements will depend on many factors, including, but not limited to:

- our ability to successfully develop and commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid or other product candidates;
- the costs, timing and outcomes of our ongoing and planned clinical studies of the bempedoic acid / ezetimibe combination pill and bempedoic acid;
- the time and cost necessary to obtain regulatory approvals for the bempedoic acid / ezetimibe combination pill and bempedoic acid, if at all;
- our ability to establish a sales, marketing and distribution infrastructure to commercialize the bempedoic acid / ezetimibe combination pill and bempedoic acid or our ability to establish any future collaboration or commercialization arrangements on favorable terms, if at all;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims; and
- the implementation of operational and financial information technology.

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, the ownership

interest of our stockholders will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect your rights as a common stockholder. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through collaborations, strategic alliances or licensing arrangements with pharmaceutical partners or royalty-based financing arrangements, we may have to relinquish valuable rights to our technologies, future revenue streams or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings or through collaborations, strategic alliances or licensing arrangements or royalty-based financing arrangements 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 the bempedoic acid / ezetimibe combination pill or bempedoic acid that we would otherwise prefer to develop and market ourselves.

### Contractual Obligations and Commitments

In February 2014, we signed a lease to move our principal executive offices to Ann Arbor, Michigan. The Ann Arbor lease has a term of 63 months and provides for fixed monthly rent of approximately \$7,900, with monthly rent increasing every 12 months, and also provides for certain rent adjustments to be paid as determined by the landlord. In August 2015, we signed a new lease to increase our office space in Ann Arbor, Michigan to support our growing company and clinical development operations. The second Ann Arbor lease has a term of 49 months and provides for fixed monthly rent of approximately \$7,100, with monthly rent increasing every 12 months.

In June 2014, we entered into a Credit Facility which provided for initial borrowings of \$5.0 million and additional borrowings of \$15.0 million until March 2015. We received proceeds of \$4.9 million, net of issuance costs, from the issuance of secured promissory notes under a term loan as part of the Credit Facility and we have not drawn upon any additional borrowings. Under the Credit Facility we are obligated to make monthly, interest-only payments on any term loans funded until July 1, 2015, and, thereafter, to pay 36 consecutive, equal monthly installments of principal and interest from August 1, 2015, through July 1, 2018. The term loan outstanding under the Credit Facility bears interest at an annual rate of 6.40%. In addition, a final payment equal to 8.0% of any amounts drawn under the Credit Facility is due upon the earlier of the maturity date or prepayment of the term loans.

The following table summarizes our future minimum contractual obligations as of December 31, 2017:

	<u>Total</u>	<u>Less than 1 Year</u>	<u>1 - 3 Years</u>	<u>3 - 5 Years</u>	<u>More than 5 Years</u>
			(in thousands)		
Operating leases	\$ 329	\$ 197	\$ 132	\$ —	\$ —
Debt commitments <sup>(1)</sup>	1,471	1,471	—	—	—
<b>Total</b>	<b>\$ 1,800</b>	<b>\$ 1,668</b>	<b>\$ 132</b>	<b>\$ —</b>	<b>\$ —</b>

- (1) The amounts in the table reflect the contractually required principal and fixed interest payments in accordance with the payment schedule. The projected fixed interest payment obligations are based upon debt outstanding as of the balance sheet date and assume retirement at the scheduled maturity date of the loan.

There have been no material changes to our contractual obligations and commitments outside the ordinary course of business from those disclosed above.

### Off-Balance Sheet Arrangements

We do not currently have, nor did we have during the periods presented, any off-balance sheet arrangements as defined by Securities and Exchange Commission rules.

**Item 7A. Quantitative and Qualitative Disclosures about Market Risk**

We had cash and cash equivalents and available-for-sale investments of approximately \$34.5 million and \$239.2 million, respectively, at December 31, 2017. The primary objectives of our investment activities are to preserve principal, provide liquidity and maximize income without significantly increasing risk. Our primary exposure to market risk relates to fluctuations in interest rates which are affected by changes in the general level of U.S. interest rates. Given the short-term nature of our cash equivalents, we believe that a sudden change in market interest rates would not be expected to have a material impact on our financial condition and/or results of operation. We do not have any foreign currency or other derivative financial instruments.

We do not believe that our cash, cash equivalents and available-for-sale investments have significant risk of default or illiquidity. While we believe our cash and cash equivalents do not contain excessive risk, we cannot provide absolute assurance that in the future our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash and cash equivalents at one or more financial institutions that are in excess of federally insured limits.

We contract with CROs and investigational sites globally. We are therefore subject to fluctuations in foreign currency rates in connection with these agreements. We do not hedge our foreign currency exchange rate risk.

Inflation generally affects us by increasing our cost of labor and clinical study costs. We do not believe that inflation has had a material effect on our results of operations during the year ended December 31, 2017.

**Item 8. Financial Statements and Supplementary Data**

The financial statements required to be filed pursuant to this Item 8 are appended to this report. An index of those financial statements is found in Item 15.

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

None.

**Item 9A. Controls and Procedures**

**Evaluation of Disclosure Controls and Procedures**

We maintain disclosure controls and procedures that are designed to ensure that information required to be disclosed in the reports that we file or submit under the Securities and Exchange Act of 1934 is (1) recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms and (2) accumulated and communicated to our management, including our President and Chief Executive Officer, who is our principal executive officer, and our Chief Financial Officer, who is our principal financial officer, to allow timely decisions regarding required disclosure.

As of December 31, 2017, our management, with the participation of our principal executive officer and principal financial officer, evaluated the effectiveness of our disclosure controls and procedures (as defined in Rules 13a-15(e) and 15d-15(e) under the Securities and Exchange Act of 1934). 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, 2017, our disclosure controls and procedures were effective at the reasonable assurance level.

## **Management's Report on Internal Control over Financial Reporting**

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for our company. Internal control over financial reporting is defined in Rule 13a-15(f) or 15d-15(f) promulgated under the Exchange Act as a process designed by, or under the supervision of, the company's principal executive officer and principal financial officer and effected by the company's board of preparation of financial statements for external purposes in accordance with GAAP and directors, management and other personnel, to provide reasonable assurance regarding the reliability of financial reporting and the includes those policies and procedures that: (i) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (ii) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of our company are being made only in accordance with authorizations of management and directors of the company; and (iii) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of our company's assets that could have a material effect on the financial statements.

Internal control over financial reporting is designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements prepared for external purposes in accordance with generally accepted accounting principles. Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Our management, with the participation of our principal executive officer and principal financial officer, assessed the effectiveness of our internal control over financial reporting as of December 31, 2017, based on criteria for effective internal control over financial reporting established in Internal Control—Integrated Framework (2013), issued by the Committee of Sponsoring Organizations of the Treadway Commission (COSO). Based on its assessment, management concluded that our internal control over financial reporting was effective as of December 31, 2017, based on those criteria.

The effectiveness of our internal control over financial reporting as of December 31, 2017, has been audited by Ernst & Young LLP, an independent registered public accounting firm, as stated in their report which is included herein.

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

There were no changes to our internal control over financial reporting that occurred during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## **Report of Independent Registered Public Accounting Firm**

**To the Stockholders and the Board of Directors of Esperion Therapeutics, Inc.**

### **Opinion on Internal Control over Financial Reporting**

We have audited Esperion Therapeutics, Inc.'s internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) (the COSO criteria). In our opinion, Esperion Therapeutics, Inc. (the Company) maintained, in all material respects, effective internal control over financial reporting as of December 31, 2017, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the balance sheets of Esperion Therapeutics, Inc. as of December 31, 2017 and 2016, and the related statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes and our report dated February 20, 2018 expressed an unqualified opinion thereon.

### **Basis for Opinion**

The Company's management is responsible for maintaining effective internal control over financial reporting and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the Company's internal control over financial reporting based on our audit. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audit in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects.

Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

### **Definition and Limitations of Internal Control Over Financial Reporting**

A company's internal control over financial reporting is a process designed to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles. A company's internal control over financial reporting includes those policies and procedures that (1) pertain to the maintenance of records that, in reasonable detail, accurately and fairly reflect the transactions and dispositions of the assets of the company; (2) provide reasonable assurance that transactions are recorded as necessary to permit preparation of financial statements in accordance with generally accepted accounting principles, and that receipts and expenditures of the company are being made only in accordance with authorizations of management and directors of the company; and (3) provide reasonable assurance regarding prevention or timely detection of unauthorized acquisition, use, or disposition of the company's assets that could have a material effect on the financial statements.



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Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Also, projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

/s/ Ernst & Young LLP

Detroit, Michigan

February 20, 2018

**Item 9B. Other Information**

None.

## PART III

### **Item 10. Directors, Executive Officers and Corporate Governance**

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2018 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission within 120 days of the end of our fiscal year pursuant to General Instruction G(3) of Form 10-K.

### **Item 11. Executive Compensation**

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2018 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission within 120 days of the end of our fiscal year pursuant to General Instruction G(3) of Form 10-K.

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

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2018 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission within 120 days of the end of our fiscal year pursuant to General Instruction G(3) of Form 10-K.

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

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2018 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission within 120 days of the end of our fiscal year pursuant to General Instruction G(3) of Form 10-K.

### **Item 14. Principal Accounting Fees and Services**

The information required by this Item is incorporated herein by reference to the information that will be contained in our proxy statement related to the 2018 Annual Meeting of Stockholders, which we intend to file with the Securities and Exchange Commission within 120 days of the end of our fiscal year pursuant to General Instruction G(3) of Form 10-K.

**PART IV**

**Item 15. Exhibits and Financial Statement Schedules**

(a) The following documents are filed as part of this report:

(1) Financial Statements:

<a href="#">Report of Independent Registered Public Accounting Firm</a>	<a href="#">F-2</a>
<a href="#">Balance Sheets</a>	<a href="#">F-3</a>
<a href="#">Statements of Operations and Comprehensive Loss</a>	<a href="#">F-4</a>
<a href="#">Statements of Stockholders' Equity</a>	<a href="#">F-5</a>
<a href="#">Statements of Cash Flows</a>	<a href="#">F-6</a>
<a href="#">Notes to Financial Statements</a>	<a href="#">F-7</a>

(2) Financial Statement Schedules:

All financial statement schedules have been omitted because they are not applicable, not required or the information required is shown in the financial statements or the notes thereto.

(3) Exhibits. The exhibits filed as part of this Annual Report on Form 10-K are set forth on the Exhibit Index included herein. The Exhibit Index is incorporated herein by reference.

**Item 16. Form 10-K Summary.**

None.

## Exhibit List

<u>Exhibit No.</u>	<u>Exhibit Index</u>
3.1	<a href="#"><u>Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.2 to the Registrant's Amendment No. 2 to the Registration Statement on Form S-1, File No. 333-188595, filed on June 12, 2013)</u></a>
3.2	<a href="#"><u>Amended and Restated By-laws of the Registrant (incorporated by reference to Exhibit 3.4 to the Registrant's Amendment No. 1 to the Registration Statement on Form S-1, File No. 333-188595, filed on June 7, 2013)</u></a>
4.1	<a href="#"><u>Specimen Common Stock Certificate (incorporated by reference to Exhibit 4.1 to the Registrant's Amendment No. 2 to the Registration Statement on Form S-1, File No. 333-188595, filed on June 12, 2013)</u></a>
4.2	<a href="#"><u>Form of Warrant to Purchase Preferred Stock dated September 4, 2012 (incorporated by reference to Exhibit 4.3 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
4.3	<a href="#"><u>Investor Rights Agreement by and between the Registrant and certain of its stockholders dated April 28, 2008 (incorporated by reference to Exhibit 4.4 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
4.4	<a href="#"><u>Amendment No. 1 to Investor Rights Agreement by and between the Registrant and certain of its stockholders dated April 11, 2013 (incorporated by reference to Exhibit 4.5 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
4.5	<a href="#"><u>Warrant dated June 30, 2014 issued to Oxford Finance LLC (incorporated by reference to Exhibit 4.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on July 2, 2014)</u></a>
10.1*	<a href="#"><u>License Agreement between Pfizer Inc. and the Registrant dated April 28, 2008 and amended on November 17, 2010 (incorporated by reference to Exhibit 10.7 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
10.2	<a href="#"><u>Termination Agreement, dated December 2, 2015, by and between the Registrant and Michigan Land Bank Fast Track Authority (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on December 3, 2015)</u></a>
10.3	<a href="#"><u>Valley Ranch Business Park Lease by and between the Registrant and McMullen SPE, LLC, dated February 4, 2014 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on February 7, 2014)</u></a>
10.4	<a href="#"><u>Form of Officer Indemnification Agreement entered into between the Registrant and its officers (incorporated by reference to Exhibit 10.8 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
10.5	<a href="#"><u>Form of Director Indemnification Agreement entered into between the Registrant and its directors (incorporated by reference to Exhibit 10.9 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>
10.6#	<a href="#"><u>2008 Incentive Stock Option and Restricted Stock Plan and forms of agreements thereunder (incorporated by reference to Exhibit 10.1 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)</u></a>

- 10.7# [Amended and Restated 2013 Stock Option and Incentive Plan and forms of agreements thereunder \(incorporated by reference to Exhibit 10.1 of the Registrant's Quarterly Report on Form 10-Q, File No. 001-35986, filed on November 3, 2016\).](#)
- 10.8# [Senior Executive Cash Bonus Plan \(incorporated by reference to Exhibit 10.11 to the Registrant's Amendment No. 1 to the Registration Statement on Form S-1, File No. 333-188595, filed on June 7, 2013\)](#)
- 10.9# [Employment Agreement by and between the Registrant and Dr. Roger S. Newton dated December 4, 2012 \(incorporated by reference to Exhibit 10.3 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013\)](#)
- 10.10 [Loan and Security Agreement, dated June 30, 2014, by and between the Registrant and Oxford Finance LLC \(incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on July 2, 2014\).](#)
- 10.11# [Employment Agreement, dated May 14, 2015, between the Registrant and Tim M. Mayleben \(incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on May 20, 2015\).](#)
- 10.12# [Employment Agreement, dated May 14, 2015, between the Registrant and Narendra D. Lalwani \(incorporated by reference to Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q, File No. 001-35986, filed on August 6, 2015\).](#)
- 10.13# [Employment Agreement, effective June 15, 2015, between the Registrant and Mary P. McGowan \(incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on June 15, 2015\).](#)
- 10.14# [Advisor Agreement, dated December 8, 2016, between the Company and Roger Newton, Ph.D. \(incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on December 9, 2016\).](#)
- 10.15# [2017 Inducement Equity Plan and form of award agreement thereunder \(incorporated by reference to Exhibit 99.1 to the Registrant's Registration Statement on Form S-8, File No. 333-218084, filed on May 18, 2017\)](#)
- 21.1 [Subsidiaries of the Registrant \(incorporated by reference to Exhibit 21.1 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013\)](#)
- 23.1\*\* [Consent of Ernst & Young LLP](#)
- 31.1\*\* [Certification of Principal Executive Officer and Principal Financial Officer pursuant to Rule 13a-14\(a\) and Rule 15d-14\(a\) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002](#)
- 31.2\*\* [Certification of Principal Financial Officer pursuant to Rule 13a-14\(a\) and Rule 15d-14\(a\) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002](#)
- 32.1\*\*\* [Certification of Principal Executive Officer and Principal 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 Extension Calculation Document

101.DEF\*\* XBRL Taxonomy Extension Definition Linkbase Document

101.LAB\*\* XBRL Taxonomy Extension Labels Linkbase Document.

101.PRE\*\* XBRL Taxonomy Extension Presentation Link Document.

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(#) Management contract or compensatory plan or arrangement.

(\*) Confidential treatment has been granted by the Securities and Exchange Commission as to certain portions.

(\*\*) Filed herewith.

(\*\*\*) The certifications furnished in Exhibit 32.1 hereto are deemed to accompany this Annual Report on Form 10-K and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended. Such certifications will not be deemed to be incorporated by reference into any filings under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference.

**SIGNATURES**

Pursuant to the requirements of the Section 13 or 15(d) of the Securities Exchange Act of 1934, the Registrant has duly caused this Form 10-K to be signed on its behalf by the undersigned, thereunto duly authorized.

**ESPERION THERAPEUTICS, INC.**

Date: February 20, 2018

By:   /s/ TIM M. MAYLEBEN    
Tim M. Mayleben  
President and Chief Executive Officer  
(Principal Executive Officer)

Pursuant to the requirements of the Securities Exchange Act of 1934, this Annual Report on Form 10-K has been signed by the following persons in the capacities indicated below and on the dates indicated:

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>  /s/ TIM M. MAYLEBEN  </u> Tim M. Mayleben	President, Chief Executive Officer and Director (Principal Executive Officer)	February 20, 2018
<u>  /s/ RICHARD B. BARTRAM  </u> Richard B. Bartram	Chief Financial Officer (Principal Financial Officer and Principal Accounting Officer)	February 20, 2018
<u>  /s/ JEFFREY BERKOWITZ, J.D.  </u> Jeffrey Berkowitz, J.D.	Director	February 20, 2018
<u>  /s/ SCOTT BRAUNSTEIN, M.D.  </u> Scott Braunstein, M.D.	Director	February 20, 2018
<u>  /s/ DOV A. GOLDSTEIN, M.D.  </u> Dov A. Goldstein, M.D.	Director	February 20, 2018
<u>  /s/ ANTONIO M. GOTTO, M.D., D. PHIL  </u> Antonio M. Gotto, M.D., D. Phil	Director	February 20, 2018

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<hr/> <u>/s/ DANIEL JANNEY</u> Daniel Janney	Director	February 20, 2018
<hr/> <u>/s/ MARK E. MCGOVERN, M.D.</u> Mark E. McGovern, M.D.	Director	February 20, 2018
<hr/> <u>/s/ ROGER S. NEWTON, PH.D., FAHA, FACN</u> Roger S. Newton, Ph.D., FAHA, FACN	Director	February 20, 2018
<hr/> <u>/s/ GILBERT S. OMENN, M.D., PH.D.</u> Gilbert S. Omenn, M.D., Ph.D.	Director	February 20, 2018
<hr/> <u>/s/ NICOLE VITULLO</u> Nicole Vitullo	Director	February 20, 2018



**Esperion Therapeutics, Inc.  
Index to the Financial Statements**

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**Report of Independent Registered Public Accounting Firm**

**To the Stockholders and the Board of Directors of Esperion Therapeutics, Inc.**

**Opinion on the Financial Statements**

We have audited the accompanying balance sheets of Esperion Therapeutics, Inc. (the Company) as of December 31, 2017 and 2016, and the related statements of operations and comprehensive loss, stockholders' equity and cash flows for each of the three years in the period ended December 31, 2017, and the related notes (collectively referred to as the "financial statements"). In our opinion, the financial statements present fairly, in all material respects, the financial position of the Company at December 31, 2017 and 2016, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2017, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States) (PCAOB), the Company's internal control over financial reporting as of December 31, 2017, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (2013 framework) and our report dated February 20, 2018 expressed an unqualified opinion thereon.

**Basis for Opinion**

These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on the Company's financial statements based on our audits. We are a public accounting firm registered with the PCAOB and are required to be independent with respect to the Company in accordance with the U.S. federal securities laws and the applicable rules and regulations of the Securities and Exchange Commission and the PCAOB.

We conducted our audits in accordance with the standards of the PCAOB. Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement, whether due to error or fraud. Our audits included performing procedures to assess the risks of material misstatement of the financial statements, whether due to error or fraud, and performing procedures that respond to those risks. Such procedures included examining, on a test basis, evidence regarding the amounts and disclosures in the financial statements. Our audits also included evaluating the accounting principles used and significant estimates made by management, as well as evaluating the overall presentation of the financial statements. We believe that our audits provide a reasonable basis for our opinion.

/s/ Ernst & Young LLP

We have served as the Company's auditor since 2008.

Detroit, Michigan

February 20, 2018

**Esperion Therapeutics, Inc.**

**Balance Sheets**

**(in thousands, except share data)**

	<b>December 31, 2017</b>	<b>December 31, 2016</b>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents	\$ 34,468	\$ 38,165
Short-term investments	165,731	173,418
Prepaid clinical development costs	2,072	560
Other prepaid and current assets	1,653	1,434
Total current assets	<u>203,924</u>	<u>213,577</u>
Property and equipment, net	435	674
Intangible assets	56	56
Long-term investments	73,420	30,906
Total assets	<u>\$ 277,835</u>	<u>\$ 245,213</u>
<b>Liabilities and stockholders' equity</b>		
Current liabilities:		
Accounts payable	\$ 20,375	\$ 4,595
Current portion of long-term debt	1,045	1,709
Accrued clinical development costs	10,506	8,138
Other accrued liabilities	1,218	1,147
Total current liabilities	<u>33,144</u>	<u>15,589</u>
Long-term debt, net of discount	<u>—</u>	<u>1,022</u>
Total liabilities	<u>33,144</u>	<u>16,611</u>
Commitments and contingencies (Note 5)		
<b>Stockholders' equity:</b>		
Preferred stock, \$0.001 par value; 5,000,000 shares authorized and no shares issued or outstanding as of December 31, 2017 and December 31, 2016	—	—
Common stock, \$0.001 par value; 120,000,000 shares authorized as of December 31, 2017 and December 31, 2016; 26,304,669 shares issued and outstanding at December 31, 2017; 22,555,413 shares issued and outstanding at December 31, 2016	26	23
Additional paid-in capital	641,801	457,951
Accumulated other comprehensive loss	(845)	(172)
Accumulated deficit	<u>(396,291)</u>	<u>(229,200)</u>
Total stockholders' equity	<u>244,691</u>	<u>228,602</u>
Total liabilities and stockholders' equity	<u>\$ 277,835</u>	<u>\$ 245,213</u>

See accompanying notes to the financial statements.

## Esperion Therapeutics, Inc.

## Statements of Operations and Comprehensive Loss

(in thousands, except share and per share data)

	Year Ended December 31,		
	2017	2016	2015
<b>Operating expenses:</b>			
Research and development	\$ 147,603	\$ 57,868	\$ 29,802
General and administrative	21,379	18,282	20,238
Total operating expenses	168,982	76,150	50,040
<b>Loss from operations</b>	<b>(168,982)</b>	<b>(76,150)</b>	<b>(50,040)</b>
Interest expense	(198)	(376)	(520)
Other income, net	2,192	1,548	776
<b>Net loss</b>	<b>\$ (166,988)</b>	<b>\$ (74,978)</b>	<b>\$ (49,784)</b>
Net loss per common share (basic and diluted)	<b>\$ (6.98)</b>	<b>\$ (3.33)</b>	<b>\$ (2.26)</b>
Weighted-average shares outstanding (basic and diluted)	23,933,273	22,544,475	22,019,818
<b>Other comprehensive loss:</b>			
Unrealized (loss) gain on investments	\$ (673)	\$ 310	\$ (423)
<b>Total comprehensive loss</b>	<b>\$ (167,661)</b>	<b>\$ (74,668)</b>	<b>\$ (50,207)</b>

See accompanying notes to the financial statements.

**Esperion Therapeutics, Inc.**

**Statements of Stockholders' Equity**

(in thousands, except share data)

	Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Loss	Total Stockholders' Equity
	Shares	Amount				
Balance December 31, 2014	20,352,876	\$ 20	\$ 238,031	\$ (104,438)	\$ (59)	\$ 133,554
Issuance of common stock from public offering, net of issuance costs (\$199)	2,012,500	3	189,980	—	—	189,983
Early exercise of stock options and vesting of restricted stock	—	—	26	—	—	26
Exercise of stock options	128,086	—	1,177	—	—	1,177
Exercise of warrants	25,445	—	—	—	—	—
Stock-based compensation	—	—	12,726	—	—	12,726
Other comprehensive loss	—	—	—	—	(423)	(423)
Net loss	—	—	—	(49,784)	—	(49,784)
Balance December 31, 2015	22,518,907	23	441,940	(154,222)	(482)	287,259
Early exercise of stock options and vesting of restricted stock	—	—	9	—	—	9
Exercise of stock options	27,757	—	45	—	—	45
Vesting of restricted stock units	8,749	—	—	—	—	—
Stock-based compensation	—	—	15,957	—	—	15,957
Other comprehensive gain	—	—	—	—	310	310
Net loss	—	—	—	(74,978)	—	(74,978)
Balance December 31, 2016	22,555,413	23	457,951	(229,200)	(172)	228,602
Adoption of accounting standard 2016-09 (Note 2)	—	—	103	(103)	—	—
Issuance of common stock from public offering, net of issuance costs (\$226)	3,565,000	3	163,975	—	—	163,978
Exercise of stock options	115,483	—	1,167	—	—	1,167
Exercise of warrants	62,525	—	—	—	—	—
Vesting of restricted stock units	6,248	—	—	—	—	—
Stock-based compensation	—	—	18,605	—	—	18,605
Other comprehensive loss	—	—	—	—	(673)	(673)
Net loss	—	—	—	(166,988)	—	(166,988)
Balance December 31, 2017	<u>26,304,669</u>	<u>\$ 26</u>	<u>\$ 641,801</u>	<u>\$ (396,291)</u>	<u>\$ (845)</u>	<u>\$ 244,691</u>

See accompanying notes to the financial statements.

**Esperion Therapeutics, Inc.**

**Statements of Cash Flows**

(in thousands)

	Year Ended December 31,		
	2017	2016	2015
<b>Operating activities</b>			
Net loss	\$ (166,988)	\$ (74,978)	\$ (49,784)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation expense	258	252	236
Amortization of debt discount	11	21	29
Amortization of debt issuance costs	12	23	32
Amortization of premiums and discounts on investments	334	1,014	647
Stock-based compensation expense	18,605	15,957	12,726
Loss on sale of assets	—	—	47
Changes in assets and liabilities:			
Prepays and other assets	(1,731)	139	(1,275)
Accounts payable	15,758	3,888	(1,333)
Other accrued liabilities	2,439	5,954	519
Net cash used in operating activities	(131,302)	(47,730)	(38,156)
<b>Investing activities</b>			
Purchases of investments	(219,577)	(197,230)	(280,559)
Proceeds from sales/maturities of investments	183,743	207,442	120,792
Proceeds from sale of assets	—	—	24
Purchase of property and equipment	(19)	(94)	(325)
Net cash (used in) provided by investing activities	(35,853)	10,118	(160,068)
<b>Financing activities</b>			
Proceeds from issuance of common stock, net of issuance costs	164,000	—	189,983
Proceeds from exercise of common stock options	1,167	45	1,177
Payments on long-term debt	(1,709)	(1,604)	(638)
Net cash provided by (used in) financing activities	163,458	(1,559)	190,522
Net decrease in cash and cash equivalents	(3,697)	(39,171)	(7,702)
Cash and cash equivalents at beginning of period	38,165	77,336	85,038
Cash and cash equivalents at end of period	<u>\$ 34,468</u>	<u>\$ 38,165</u>	<u>\$ 77,336</u>
<b>Supplemental disclosure of cash flow information:</b>			
Offering costs not yet paid	<u>\$ 22</u>	<u>\$ —</u>	<u>\$ —</u>

See accompanying notes to the financial statements.

**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements**

**1. The Company and Basis of Presentation**

The Company is the Lipid Management Company, a late-stage pharmaceutical company focused on developing and commercializing complementary, convenient, cost-effective, once-daily, oral therapies for the treatment of patients with elevated low density lipoprotein cholesterol ("LDL-C"). Through scientific and clinical excellence, and a deep understanding of cholesterol biology, the experienced lipid management team at Esperion is committed to developing new LDL-C lowering therapies that will make a substantial impact on reducing global cardiovascular disease ("CVD"); the leading cause of death around the world. Bempedoic acid and the Company's lead product candidate, the bempedoic acid / ezetimibe combination pill, are targeted therapies that have been shown to significantly reduce elevated LDL-C levels in patients with hypercholesterolemia, including patients inadequately treated with current lipid-modifying therapies.

The clinical development program for the bempedoic acid / ezetimibe combination pill consists of a single pivotal Phase 3 clinical study (1002FDC-053) in patients with hypercholesterolemia and with atherosclerotic cardiovascular disease ("ASCVD") and/or heterozygous familial hypercholesterolemia ("HeFH"), including high CVD risk primary prevention patients, whose LDL-C is not adequately controlled despite receiving maximally tolerated lipid-modifying background therapy. 1002FDC-053 initiated in November 2017 and the Company expects to report top-line results in August 2018.

The global pivotal Phase 3 clinical development program for bempedoic acid, consisting of four clinical studies, fully enrolled approximately 3,600 high CVD risk patients with hypercholesterolemia and ASCVD and/or HeFH, or who are high CVD risk primary prevention, on optimized background lipid-modifying therapy and with elevated levels of LDL-C. These patients are on two distinct types of background lipid-modifying therapy: 1) patients on their maximally tolerated statin therapy, and 2) patients who are only able to tolerate less than the lowest approved daily starting dose, and can be considered statin intolerant. In March 2018, the Company expects to report top-line results from the first of the Phase 3 studies, Study 4 (1002-048). In May 2018, the Company expects to report top-line results from the 52-week long-term safety study, Study 1 (1002-040) and top-line results from Study 3 (1002-046). In September 2018, top-line results are expected from Study 2 (1002-047).

The Company intends to use positive results from the Phase 3 bempedoic acid / ezetimibe combination pill and bempedoic acid programs with a total of 4,000 patients to support global regulatory submissions for tandem LDL-C lowering indications in the U.S. by the first quarter of 2019 and in Europe by the second quarter of 2019.

The Company is also conducting a global cardiovascular outcomes trial ("CVOT")—known as **C**holesterol **L**owering via **B**empedoic Acid, an **A**CL-inhibiting **R**egimen (CLEAR) Outcomes, for bempedoic acid in patients with hypercholesterolemia and high CVD risk and who can be considered statin intolerant. The Company initiated the CLEAR Outcomes CVOT in December 2016, and intends to use positive results from this CVOT to support submissions for a CV risk reduction indication in the U.S. and Europe by 2022.

In December 2017, the Company submitted an investigational new drug ("IND") application to the Food and Drug Administration ("FDA"), for a reformulated tablet of bempedoic acid for nonalcoholic steatohepatitis ("NASH") indication, which was accepted in January 2018.

The Company's primary activities since incorporation have been conducting research and development activities, including nonclinical, preclinical and clinical testing, performing business and financial planning, recruiting personnel, and raising capital. Accordingly, the Company has not

**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**1. The Company and Basis of Presentation (Continued)**

commenced principal operations and is subject to risks and uncertainties which include the need to research, develop, and clinically test potential therapeutic products; obtain regulatory approvals for its products and commercialize them, if approved; expand its management and scientific staff; and finance its operations with an ultimate goal of achieving profitable operations.

The Company has sustained operating losses since inception and expects such losses to continue over the foreseeable future. Management plans to continue to fund operations through public or private equity or debt financings or through other sources, which may include collaborations with third parties. If adequate funds are not available, the Company may not be able to continue the development of its current or future product candidates, or to commercialize its current or future product candidates, if approved.

**Follow On Offerings**

On March 24, 2015, the Company completed an underwritten public offering of 2,012,500 shares of common stock, including 262,500 shares sold pursuant to the full exercise of an over-allotment option granted to the underwriters. All the shares were offered by the Company at a price to the public of \$100.00 per share. The aggregate net proceeds received by the Company from the offering were \$190.0 million, net of underwriting discounts and commissions and expenses payable by the Company.

On August 15, 2017, the Company completed an underwritten public offering of 3,100,000 shares of common stock. The Company also granted the underwriters a 30-day option to purchase up to 465,000 additional shares of its common stock which was exercised in full in September 2017. All the shares were offered by the Company at a price to the public of \$49.00 per share. The aggregate net proceeds received by the Company from the offering were \$164.0 million, net of underwriting discounts and commissions and expenses payable by the Company.

**2. Summary of Significant Accounting Policies**

**Use of Estimates**

The preparation of financial statements in accordance with generally accepted accounting principles in the United States requires management to make estimates and assumptions that affect the reported amounts of assets, liabilities, expenses and related disclosures. Actual results could differ from those estimates.

**Cash and Cash Equivalents**

The Company invests its excess cash in bank deposits, money market accounts, and short-term investments. The Company considers all highly liquid investments with an original maturity of 90 days or less at the time of purchase to be cash equivalents. Cash equivalents are reported at fair value.

**Investments**

Investments are considered to be available-for-sale and are carried at fair value. Unrealized gains and losses, if any, are reported as a separate component of stockholders' equity. The cost of investments classified as available-for-sale are adjusted for the amortization of premiums and accretion of discounts to maturity and recorded in other income, net. Realized gains and losses, if any, are determined using the specific identification method and recorded in other income, net.

Investments



**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**2. Summary of Significant Accounting Policies (Continued)**

with original maturities beyond 90 days at the date of purchase and which mature at, or less than twelve months from, the balance sheet date are classified as current. Investments with a maturity beyond twelve months from the balance sheet date are classified as long-term.

**Concentration of Credit Risk**

Cash, cash equivalents, and marketable securities consist of financial instruments that potentially subject the Company to concentrations of credit risk. The Company has established guidelines for investment of its excess cash and believes the guidelines maintain safety and liquidity through diversification of counterparties and maturities.

**Segment Information**

The Company views its operations and manages its business in one operating segment, which is the business of researching, developing and commercializing therapies for the treatment of patients with elevated LDL-C.

**Fair Value of Financial Instruments**

The Company's cash, cash equivalents and investments are carried at fair value. Financial instruments, including other prepaid and current assets, accounts payable and accrued liabilities are carried at cost, which approximates fair value. Debt is carried at amortized cost, which approximates fair value.

**Property and Equipment, Net**

Property and equipment are recorded at cost, less accumulated depreciation. Depreciation is provided using the straight-line method over the estimated useful lives of the respective assets, generally three to ten years. Leasehold improvements are amortized over the lesser of the lease term or the estimated useful lives of the related assets.

**Impairment of Long-Lived Assets**

The Company reviews long-lived assets, including property and equipment, for impairment whenever events or changes in business circumstances indicate that the carrying amount of the assets may not be fully recoverable. An impairment loss would be recognized when estimated undiscounted future cash flows expected to result from the use of the asset and its eventual disposition are less than its carrying amount. The impairment loss, if recognized, would be based on the excess of the carrying value of the impaired asset over its respective fair value. No impairment losses have been recorded through December 31, 2017.

**Research and Development**

Research and development expenses consist of costs incurred to further the Company's research and development activities and include salaries and related benefits, costs associated with clinical activities, nonclinical activities, regulatory activities, manufacturing activities to support clinical activities, research-related overhead expenses and fees paid to external service providers that conduct

**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**2. Summary of Significant Accounting Policies (Continued)**

certain research and development, clinical, and manufacturing activities on behalf of the Company. Research and development costs are expensed as incurred.

**Accrued Clinical Development Costs**

Outside research costs are a component of research and development expense. These expenses include fees paid to clinical research organizations and other service providers that conduct certain clinical and product development activities on behalf of the Company. Depending upon the timing of payments to the service providers, the Company recognizes prepaid expenses or accrued expenses related to these costs. These accrued or prepaid expenses are based on management's estimates of the work performed under service agreements, milestones achieved and experience with similar contracts. The Company monitors each of these factors and adjusts estimates accordingly.

**Income Taxes**

The Company utilizes the liability method of accounting for income taxes as required by ASC 740, Income Taxes. Under this method, deferred tax assets and liabilities are determined based on differences between financial reporting and the tax basis of assets and liabilities and are measured using enacted tax rates and laws that will be in effect when the differences are expected to reverse. The Company has incurred operating losses since inception. Accordingly, it is not more likely than not that the Company will realize a tax benefit from its deferred tax assets and as such, it has recorded a full valuation allowance.

**Warrants**

The Company accounts for its warrants issued in connection with its various financing transactions based upon the characteristics and provisions of the instrument. Warrants classified as additional-paid-in-capital are recorded on the Company's balance sheet at their fair value on the date of issuance. The warrants are measured using the Black-Scholes option-pricing model subsequent to the pricing of the Company's IPO and a Monte Carlo valuation model for previous periods which are based, in part, upon inputs where there is little or no market data, requiring the Company to develop its own independent assumptions (see Note 4).

**Stock-Based Compensation**

The Company accounts for stock-based compensation in accordance with the provisions of ASC 718, Compensation—Stock Compensation. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value calculated using a Black-Scholes option-pricing model. In accordance with the adoption of Accounting Standards Update ("ASU") 2016-09 on January 1, 2017, the Company accounts for forfeitures as they occur. Prior to January 1, 2017, under the provisions of ASC 718, the Company was required to include an estimate of the number of awards that will be forfeited in calculating compensation costs. Any changes to the estimated forfeiture rates were accounted for prospectively. Stock-based compensation arrangements with non-employees are recognized at the grant-date fair value and then re-measured at each reporting period. Expense is recognized during the period the related services are rendered.

**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**2. Summary of Significant Accounting Policies (Continued)**

**Recent Accounting Pronouncements**

In February 2016, the Financial Accounting Standards Board ("FASB") issued Accounting Standards Update ("ASU") 2016-02 which is intended to improve financial reporting about leasing transactions. The updated guidance will require a lessee to recognize assets and liabilities for leases with lease terms of more than twelve months. Consistent with current GAAP, the recognition, measurement and presentation of expenses and cash flows arising from a lease by a lessee primarily will depend on its classification as a capital or operating lease. Unlike current GAAP—which requires only capital leases to be recognized on the balance sheet—the updated guidance will require both types of leases to be recognized on the balance sheet. The standard is effective for public companies for fiscal years beginning after December 15, 2018, and interim periods within those years. Early adoption is permitted for annual or interim reporting periods for which the financial statements have not previously been issued. The Company does not believe the adoption of this standard will have a material impact on its financial position, results of operations or related financial statement disclosures.

In March 2016, the FASB issued ASU 2016-09 which includes provisions intended to simplify the various aspects related to how share-based payments are accounted for and presented in the financial statements. The updated guidance requires all income tax effects of awards to be recognized in the income statement when the awards vest or are settled. Additionally, under the updated guidance companies have to elect whether to account for forfeitures of share-based payments by (1) recognizing forfeitures as they occur or (2) estimating the number of awards expected to be forfeited and adjusting the estimate when it is likely to change, as was previously required. The Company adopted ASU 2016-09 effective January 1, 2017, and recognized approximately \$4.5 million of deferred tax assets that were not previously recognized on the Company's balance sheet under the prior accounting guidance. The increase in the deferred tax assets was fully offset by an increase in the Company's valuation allowance. In addition, the Company made a policy election to account for forfeitures as they occur. The cumulative effect of adoption was an increase of \$0.1 million to both additional paid-in capital and accumulated deficit as of January 1, 2017. The remaining provisions adopted in ASU 2016-09 did not have a material impact to the Company's balance sheets, statements of operations or statements of cash flows.

**3. Debt**

**Credit Facility**

In June 2014, the Company entered into a loan and security agreement (the "Credit Facility") with Oxford Finance LLC which provided for borrowings of \$5.0 million under the term loan (the "Term A Loan"). On June 30, 2014, the Company received proceeds of \$5.0 million from the issuance of secured promissory notes under the Term A Loan. The secured promissory notes issued under the Credit Facility are due on July 1, 2018, and are collateralized by substantially all of the Company's personal property, other than its intellectual property.

The Company is obligated to make monthly, interest-only payments on the Term A Loan until July 1, 2015, and, thereafter, to pay 36 consecutive, equal monthly installments of principal and interest from August 1, 2015, through July 1, 2018. The Term A Loan bears interest at an annual rate of 6.40%. In addition, a final payment equal to 8.0% of the Term A Loan is due upon the earlier of the maturity date or prepayment of the term loan. The Company is recognizing the final payment as interest expense using the effective interest method over the life of the Credit Facility.

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****3. Debt (Continued)**

There are no financial covenants associated to the Credit Facility. However, so long as the Credit Facility is outstanding, there are negative covenants that limit or restrict the Company's activities, which include limitations on incurring indebtedness, granting liens, mergers or acquisitions, dispositions of assets, making certain investments, entering into certain transactions with affiliates, paying dividends or distributions, encumbering or pledging interest in its intellectual property and certain other business transactions. Additionally, the Credit Facility includes events of default, the occurrence and continuation of any of which provides the lenders the right to exercise remedies against the Company and the collateral securing the loans under the Credit Facility, which includes cash. These events of default include, among other things, non-payment of any amounts due under the Credit Facility, insolvency, the occurrence of a material adverse event, inaccuracy of representations and warranties, cross default to material indebtedness and a material judgment against the Company. Upon the occurrence of an event of default, all obligations under the Credit Facility shall accrue interest at a rate equal to the fixed annual rate plus five percentage points.

In connection with the borrowing of the Term A Loan, the Company issued a warrant to purchase 8,230 shares of common stock at an exercise price of \$15.19 (see Note 4). The warrant resulted in a debt discount of \$0.1 million which is amortized into interest expense using the effective interest method over the life of the Term A Loan. In addition, the Company incurred debt issuance costs of \$0.1 million in connection with the borrowing of the Term A Loan. The debt issuance costs were capitalized and included in long-term debt on the condensed balance sheet at the inception of the Term A Loan, and are amortized to interest expense using the effective interest method over the same term. As of December 31, 2017, the remaining unamortized discount and debt issuance costs associated with the debt were less than \$0.1 million and less than \$0.1 million, respectively.

Estimated future principal payments due under the Credit Facility are as follows:

<u>Years Ending December 31,</u>	<u>(in thousands)</u>
<u>2018</u>	<u>\$ 1,049</u>
Total	<u>\$ 1,049</u>

During the years ended December 31, 2017 and 2016, the Company recognized \$0.2 million and \$0.4 million of interest expense and made cash interest payments of \$0.1 million and \$0.2 million related to the Credit Facility, respectively.

**4. Warrants**

In connection with the Credit Facility entered into in June 2014, the Company issued a warrant to purchase 8,230 shares of common stock at an exercise price of \$15.19. The warrant will terminate on the earlier of June 30, 2019, and the closing of a merger or consolidation transaction in which the Company is not the surviving entity. The warrant was recorded at fair value of \$0.1 million to additional-paid-in-capital in accordance with ASC 815-10 based upon the allocation of the debt proceeds. The Company estimated the fair value of the warrant using a Black-Scholes option-pricing model, which is based, in part, upon subjective assumptions including but not limited to stock price volatility, the expected life of the warrant, the risk-free interest rate and the fair value of the common stock underlying the warrant. The Company estimates the volatility of its stock based on public company peer group historical volatility that is in line with the expected remaining life of the warrant.

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****4. Warrants (Continued)**

The risk-free interest rate is based on the U.S. Treasury zero-coupon bond for a maturity similar to the expected remaining life of the warrant. The expected remaining life of the warrant is assumed to be equivalent to its remaining contractual term.

Upon the closing of the Company's IPO, all warrants exercisable for 1,940,000 shares of Series A preferred stock, at an exercise price of \$1.00 per share (unadjusted for stock splits), were automatically converted into warrants exercisable for 277,690 shares of common stock, at an exercise price of \$6.99 per share. As a result, the Company concluded the warrants outstanding no longer met the criteria to be classified as liabilities and were reclassified to additional paid-in capital at fair value on the date of reclassification. During the year ended December 31, 2017, 71,237 warrants were net exercised for 62,525 shares of the Company's common stock. During the year ended December 31, 2015, 29,330 warrants were net exercised for 25,445 shares of the Company's common stock. The remaining 177,123 warrants outstanding as of December 31, 2017, expire in February 2018.

As of December 31, 2017, the Company had warrants outstanding that were exercisable for a total of 185,353 shares of common stock at a weighted-average exercise price of \$7.35 per share.

**5. Commitments and Contingencies**

In February 2014, the Company entered into an operating lease agreement for its principal executive offices located in Ann Arbor, Michigan commencing in April 2014, with a term of 63 months. The Company's lease provides for fixed monthly rent for the term of the lease, with monthly rent increasing every 12 months subsequent to the first three months of the lease, and also provides for certain rent adjustments to be paid as determined by the landlord.

In August 2015, the Company entered into an operating lease agreement to increase its office space and support its clinical development operations located in Ann Arbor, Michigan, commencing September 2015, with a term of 49 months. The Company's lease provides for fixed monthly rent for the term of the lease, with monthly rent increasing every 12 months subsequent to the first month of the lease.

The total rent expense for the years ended December 31, 2017, 2016 and 2015, was approximately \$0.2 million, \$0.2 million, and \$0.2 million, respectively. The following table summarizes the Company's future minimum lease payments as of December 31, 2017:

	<u>Total</u>	<u>Less than 1 Year</u>	<u>1 - 3 Years</u>	<u>3 - 5 Years</u>	<u>More than 5 Years</u>
	(in thousands)				
Operating lease	\$ 329	\$ 197	\$ 132	\$ —	\$ —
Total	<u>\$ 329</u>	<u>\$ 197</u>	<u>\$ 132</u>	<u>\$ —</u>	<u>\$ —</u>

**Legal Proceedings**

On January 12, 2016, a purported stockholder of the Company filed a putative class action lawsuit in the United States District Court for the Eastern District of Michigan, against the Company and Tim Mayleben, captioned *Kevin L. Dougherty v. Esperion Therapeutics, Inc., et al.* (No. 16-cv-10089). The lawsuit alleges that the Company and Mr. Mayleben violated Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and SEC Rule 10b-5 by allegedly failing to disclose in an August 17, 2015, public

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****5. Commitments and Contingencies (Continued)**

statement that the FDA would require a cardiovascular outcomes trial before approving the Company's lead product candidate. The lawsuit seeks, among other things, compensatory damages in connection with an allegedly inflated stock price between August 18, 2015, and September 28, 2015, as well as attorneys' fees and costs. On May 20, 2016, an amended complaint was filed in the lawsuit and on July 5, 2016, the Company filed a motion to dismiss the amended complaint. On December 27, 2016, the court granted the Company's motion to dismiss with prejudice and entered judgment in the Company's favor. On January 24, 2017, the plaintiffs in this lawsuit filed a motion to alter or amend the judgment. In May 2017, the court denied the plaintiff's motion to alter or amend the judgment. On June 19, 2017, the plaintiffs filed a notice of appeal to the Sixth Circuit Court of Appeals and on September 14, 2017, they filed their opening brief in support of the appeal. The appeal was fully briefed on December 7, 2017.

On December 15, 2016, a purported stockholder of the Company filed a derivative lawsuit in the Court of Chancery of the State of Delaware against Tim Mayleben, Roger Newton, Mary McGowan, Nicole Vitullo, Dov Goldstein, Daniel Janney, Antonio Gotto Jr., Mark McGovern, Gilbert Omenn, Scott Braunstein, and Patrick Enright. The Company is named as a nominal defendant. The lawsuit alleges that the defendants breached their fiduciary duties to the Company when they made or approved improper statements on August 17, 2015, regarding the Company's lead product candidate's path to FDA approval, and failed to ensure that reliable systems of internal controls were in place at the Company. The lawsuit seeks, among other things, any damages sustained by the Company as a result of the defendants' alleged breaches of fiduciary duties, including damages related to the above-referenced securities class action, an order directing the Company to take all necessary actions to reform and improve its corporate governance and internal procedures, restitution from the defendants, and attorneys' fees and costs. In light of, among other things, the early stage of the litigation, the Company is unable to predict the outcome of this matter and is unable to make a meaningful estimate of the amount or range of loss, if any, that could result from an unfavorable outcome.

**6. Property and Equipment**

Property and equipment consist of the following:

	<b>December 31,</b>	
	<b>2017</b>	<b>2016</b>
	<b>(in thousands)</b>	
Lab equipment	\$ 232	\$ 232
Computer equipment	114	135
Software	206	73
Furniture and fixtures	568	568
Leasehold improvements	159	159
Assets in Progress	—	114
Subtotal	<u>1,279</u>	<u>1,281</u>
Less accumulated depreciation and amortization	844	607
Property and equipment, net	<u>\$ 435</u>	<u>\$ 674</u>

Depreciation expense was \$0.3 million, \$0.3 million, and \$0.2 million for the years ended December 31, 2017, 2016 and 2015, respectively.

Esperion Therapeutics, Inc.

Notes to the Financial Statements (Continued)

7. Other Accrued Liabilities

Other accrued liabilities consist of the following:

	December 31,	
	2017	2016
	(in thousands)	
Accrued compensation	\$ 582	\$ 456
Accrued professional fees	153	158
Accrued franchise and property taxes	38	40
Accrued interest	397	350
Accrued other	48	143
Total other accrued liabilities	<u>\$ 1,218</u>	<u>\$ 1,147</u>

8. Investments

The following table summarizes the Company's cash equivalents and investments:

	December 31, 2017			
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Estimated Fair Value
	(in thousands)			
<b>Cash equivalents:</b>				
Money market funds	\$ 27,302	\$ —	\$ —	\$ 27,302
U.S treasury notes	2,999	—	—	2,999
<b>Short-term investments:</b>				
Certificates of deposit	12,429	1	(13)	12,417
U.S treasury notes	97,537	—	(225)	97,312
U.S. government agency securities	56,143	—	(141)	56,002
<b>Long-term investments:</b>				
Certificates of deposit	3,863	—	(10)	3,853
U.S. treasury notes	27,983	—	(209)	27,774
U.S. government agency securities	42,041	—	(248)	41,793
Total	<u>\$ 270,297</u>	<u>\$ 1</u>	<u>\$ (846)</u>	<u>\$ 269,452</u>

Esperion Therapeutics, Inc.

Notes to the Financial Statements (Continued)

8. Investments (Continued)

	December 31, 2016			Estimated Fair Value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
(in thousands)				
<b>Cash equivalents:</b>				
Money market funds	\$ 33,661	\$ —	\$ —	\$ 33,661
<b>Short-term investments:</b>				
Certificates of deposit	25,586	1	(20)	25,567
U.S. treasury notes	47,547	2	(30)	47,519
U.S. government agency securities	100,356	13	(37)	100,332
<b>Long-term investments:</b>				
Certificates of deposit	3,432	—	(15)	3,417
U.S. treasury notes	22,575	—	(72)	22,503
U.S. government agency securities	5,000	—	(14)	4,986
<b>Total</b>	<b>\$ 238,157</b>	<b>\$ 16</b>	<b>\$ (188)</b>	<b>\$ 237,985</b>

At December 31, 2017, remaining contractual maturities of available-for-sale investments classified as current on the balance sheet were less than 12 months, and remaining contractual maturities of available-for-sale investments classified as long-term were less than two years.

During the years ended December 31, 2017, 2016 and 2015, other income, net in the statements of operations includes interest income on available-for-sale investments of \$2.5 million, \$2.6 million and \$1.5 million, and expense for the amortization of premiums and discounts on investments of \$0.3 million, \$1.0 million and \$0.6 million, respectively.

There were no unrealized gains or losses on investments reclassified from accumulated other comprehensive loss to other income, net in the statements of operations during the year ended December 31, 2017.

9. Fair Value Measurements

The Company follows accounting guidance that emphasizes that fair value is a market-based measurement, not an entity-specific measurement. Fair value is defined as "the price that would be received to sell an asset or paid to transfer a liability in an orderly transaction between market participants at the measurement date." Fair value measurements are defined on a three level hierarchy:

- Level 1 inputs: Quoted prices for identical assets or liabilities in active markets;
- Level 2 inputs: Observable inputs other than Level 1 prices, such as quoted market prices for similar assets or liabilities or other inputs that are observable or can be corroborated by market data; and
- Level 3 inputs: Unobservable inputs that are supported by little or no market activity and require the reporting entity to develop assumptions that market participants would use when pricing the asset or liability.



**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**9. Fair Value Measurements (Continued)**

The following table presents the Company's financial assets and liabilities that have been measured at fair value on a recurring basis:

<u>Description</u>	<u>Total</u>	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>
		(in thousands)		
<b>December 31, 2017</b>				
Assets:				
Money market funds	\$ 27,302	\$ 27,302	\$ —	\$ —
Available-for-sale securities:				
Certificates of deposit	16,270	16,270	—	—
U.S. treasury notes	128,085	128,085	—	—
U.S. government agency securities	97,795	—	97,795	—
Total assets at fair value	<u>\$ 269,452</u>	<u>\$ 171,657</u>	<u>\$ 97,795</u>	<u>\$ —</u>

<u>Description</u>	<u>Total</u>	<u>Level 1</u>	<u>Level 2</u>	<u>Level 3</u>
		(in thousands)		
<b>December 31, 2016</b>				
Assets:				
Money market funds	\$ 33,661	\$ 33,661	\$ —	\$ —
Available-for-sale securities:				
Certificates of deposit	28,984	28,984	—	—
U.S. treasury notes	70,022	70,022	—	—
U.S. government agency securities	105,318	—	105,318	—
Total assets at fair value	<u>\$ 237,985</u>	<u>\$ 132,667</u>	<u>\$ 105,318</u>	<u>\$ —</u>

There were no transfers between Levels 1, 2 or 3 during the years ended December 31, 2017 or December 31, 2016.

**10. Stock Compensation**

**2017 Inducement Equity Plan**

In May 2017, the Company's board of directors approved the 2017 Inducement Equity Plan (the "2017 Plan"). The number of shares of common stock available for awards under the 2017 Plan was set to 750,000, with any shares of common stock that are forfeited, cancelled, held back upon the exercise or settlement of an award to cover the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of common stock, or otherwise terminated (other than by exercise) under the 2017 Plan added back to the shares of common stock available for issuance under the 2017 Plan. The 2017 Plan provides for the granting of stock options, stock appreciation rights, restricted stock awards, restricted stock units ("RSUs"), unrestricted stock awards and dividend equivalent rights.

**2013 Stock Option and Incentive Plan**

In May 2015, the Company's stockholders approved the amended and restated 2013 Stock Option and Incentive Plan (as amended, the "2013 Plan") which, among other things, increased the number of

**Esperion Therapeutics, Inc.**

**Notes to the Financial Statements (Continued)**

**10. Stock Compensation (Continued)**

shares of common stock reserved for issuance thereunder. The number of shares of common stock available for awards under the 2013 Plan was increased by 923,622 shares from 2,051,378 shares to 2,975,000 shares, plus (i) shares of common stock that are forfeited, cancelled, held back upon the exercise or settlement of an award to cover the exercise price or tax withholding, reacquired by the Company prior to vesting, satisfied without the issuance of common stock or otherwise terminated (other than by exercise) under the 2013 Plan and the Company's 2008 Incentive Stock Option and Restricted Stock Plan are added back to the shares of common stock available for issuance under the 2013 Plan, and (ii) on January 1, 2016, and each January 1, thereafter, the number of shares of common stock reserved and available for issuance under the 2013 Plan will be cumulatively increased by 2.5% of the number of shares of common stock outstanding on the immediately preceding December 31, or such lesser number of shares of common stock determined by the compensation committee. The 2013 Plan provides for the granting of stock options, stock appreciation rights, restricted stock awards, RSUs, unrestricted stock awards, cash-based awards, performance share awards and dividend equivalent rights.

**2008 Stock Option and Restricted Stock Plan**

In April 2008, the Company adopted the 2008 Plan, administered by the Board of Directors or a committee appointed by the Board of Directors. The 2008 Plan provides for the granting of stock options and restricted stock to employees and nonemployees of the Company. Options granted under the 2008 Plan may either be incentive stock options, restricted stock awards or nonqualified stock options. Stock options and restricted stock grants may be granted to employees, directors and consultants. Stock awards under the 2008 Plan may be granted for up to ten years from the adoption of the 2008 Plan at prices no less than 100 percent of the fair value of the shares on the date of the grant as determined by (i) the closing price of the Company's common stock on any national exchange, (ii) the National Association of Securities Dealers Inc. Automated Quotation System ("NASDAQ"), if so authorized for quotation as a NASDAQ security, or (iii) by reasonable application of a reasonable valuation method. The valuation methods utilized by the Company are consistent with the AICPA Technical Practice Aid.

The Company incurs stock-based compensation expense related to stock options and RSUs. The fair value of RSUs is determined by the closing market price of the Company's common stock on the date of grant. The fair value of stock options is calculated using a Black-Scholes option-pricing model. The Company accounts for stock-based compensation in accordance with the provisions of ASC 718, Compensation—Stock Compensation. Accordingly, compensation costs related to equity instruments granted are recognized over the requisite service periods of the awards on a straight-line basis at the grant-date fair value. In accordance with the adoption of ASU 2016-09, effective January 1, 2017, the Company accounts for forfeitures as they occur. Prior to January 1, 2017, under the provisions of ASC 718, the Company was required to include an estimate of the number of awards that will be forfeited in calculating compensation costs. Any changes to the estimated forfeiture rates were accounted for prospectively.

Under the 2017 Plan, 2013 Plan and the 2008 Plan the vesting of options granted or restricted awards given will be determined individually with each option grant. Generally, 25 percent of the granted amount will vest upon the first anniversary of the option grant with the remainder vesting ratably on the first day of each calendar quarter for the following three years. Stock options have a

Esperion Therapeutics, Inc.

Notes to the Financial Statements (Continued)

10. Stock Compensation (Continued)

10-year life and expire if not exercised within that period, or if not exercised within 90 days of cessation of providing service to the Company.

The following table summarizes the activity relating to the Company's options to purchase common stock for the year ended December 31, 2017:

	Number of Options	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Outstanding at December 31, 2016	3,255,987	\$ 28.53	7.73	\$ 5,214
Granted	1,327,400	\$ 24.30		
Forfeited or cancelled (vested and unvested)	(308,753)	\$ 22.07		
Exercised	(115,483)	\$ 11.58		
Outstanding at December 31, 2017	<u>4,159,151</u>	\$ 28.13	7.39	\$ 165,385

The following table summarizes information about the Company's stock option plan as of December 31, 2017:

	Number of Options	Weighted-Average Exercise Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value (in thousands)
Vested and expected to vest at December 31, 2017	4,159,151	\$ 28.13	7.39	\$ 165,385
Exercisable at December 31, 2017	<u>2,421,364</u>	\$ 27.05	6.53	\$ 100,329

The total intrinsic value of stock options exercised during the years ended December 31, 2017, 2016 and 2015, was \$4.0 million, \$0.4 million and \$7.4 million, respectively.

The following table shows the weighted-average assumptions used to compute the stock-based compensation costs for the stock options granted to employees and non-employees during each of the three years ending December 31, 2017, using the Black-Scholes option-pricing model:

	Year ended December 31,		
	2017	2016	2015
Risk-free interest rate	2.04%	1.47%	1.65%
Dividend yield	—	—	—
Weighted-average expected life of options (years)	6.19	6.22	6.11
Volatility	73%	71%	70%

The risk-free interest rate assumption was based on the United States Treasury's rates for U.S. Treasury zero-coupon bonds with maturities similar to those of the expected term of the award being valued. The assumed dividend yield was based on the Company's expectation of not paying dividends in the foreseeable future. The weighted-average expected life of the options was calculated using the

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****10. Stock Compensation (Continued)**

simplified method as prescribed by the Securities and Exchange Commission Staff Accounting Bulletin No. 107 ("SAB No. 107"). This decision was based on the lack of relevant historical data due to the Company's limited historical experience. In addition, due to the Company's limited historical data, the estimated volatility also reflects the application of SAB No. 107, incorporating the historical volatility of comparable companies whose share prices are publicly available.

The weighted-average grant-date fair values of stock options granted during the years ended December 31, 2017, 2016 and 2015, were \$15.99, \$9.78 and \$38.44, respectively. During the years ended December 31, 2017, 2016 and 2015, the Company recognized stock-based compensation expense related to stock options of \$18.2 million, \$15.6 million and \$12.6 million, respectively.

As of December 31, 2017, there was approximately \$29.0 million of unrecognized compensation cost related to unvested options, which will be recognized over a weighted-average period of approximately 2.2 years.

The following table summarizes the activity relating to the Company's RSUs for the year ended December 31, 2017:

	Number of RSUs	Weighted-Average Fair Value Per Share
Outstanding and unvested at December 31, 2016	16,251	\$ 57.54
Vested	(6,248)	\$ 57.54
Outstanding and unvested at December 31, 2017	<u>10,003</u>	<u>\$ 57.54</u>

During the years ended December 31, 2017, 2016 and 2015, the Company recognized approximately \$0.4 million, \$0.4 million and \$0.1 million, respectively, of stock-based compensation expense recognized related to RSUs. As of December 31, 2017, there was approximately \$0.5 million of unrecognized stock-based compensation expense related to unvested RSUs, which will be recognized over a weighted-average period of approximately 1.5 years.

**11. Employee Benefit Plan**

During 2008, the Company adopted the Esperion Therapeutics, Inc. 401(k) Plan (the "401(k) Plan"), which qualifies as a deferred salary arrangement under Section 401(k) of the Internal Revenue Code. Under the 401(k) Plan, participating employees may defer a portion of their pretax earnings. The Company may, at its sole discretion, contribute for the benefit of eligible employees. Company contributions to the 401(k) Plan during the years ended December 31, 2017, 2016 and 2015, were \$0.3 million, \$0.2 million and \$0.1 million, respectively.

**12. Income Taxes**

There was no provision for income taxes for the years ended December 31, 2017, 2016 and 2015, because the Company has incurred operating losses since inception. At December 31, 2017, the Company concluded that it is not more likely than not that the Company will realize the benefit of its deferred tax assets due to its history of losses. Accordingly, a full valuation allowance has been applied against the net deferred tax assets.

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****12. Income Taxes (Continued)**

On December 22, 2017, the Tax Cuts and Jobs Act of 2017 ("TCJA") was signed into law making significant changes to the Internal Revenue Code. Changes include, but are not limited to, a corporate tax decrease from 34% to 21% effective for tax years after December 31, 2017, the transition of U.S. Tax from a worldwide to a territorial system, and potential additional limitations on deductions related to interest expense and executive compensation. The Company has calculated the impact of TCJA in its year end income tax provision in accordance with its current understanding of the TCJA and guidance currently available as of this filing and recorded a provisional reduction to its gross deferred tax assets of \$50.4 million in the fourth quarter of 2017, the period in which the legislation was enacted. The provisional reduction in the Company's gross deferred tax assets was fully offset by an equal reduction in the Company's valuation allowance, resulting in no additional net income tax expense from the tax law change.

In addition, on January 1, 2017, upon the Company's adoption of ASU 2016-09, the Company recognized approximately \$4.5 million of deferred tax assets that were not previously recognized on the Company's balance sheet under the prior accounting guidance. The increase in the deferred tax assets was fully offset by an increase in the Company's valuation allowance.

As of December 31, 2017, 2016 and 2015, the Company had deferred tax assets, before valuation allowance, of approximately \$99.8 million, \$75.3 million and \$50.6 million, respectively. Realization of the deferred assets is dependent upon future taxable income, if any, the amount and timing of which are uncertain. Accordingly, the net deferred tax assets have been fully offset by a valuation allowance. As of December 31, 2017, 2016 and 2015, the Company had federal net operating loss ("NOL") carryforwards of approximately \$347.4 million, \$196.4 million and \$137.4 million, respectively. The federal NOL carryforwards will expire at various dates beginning in 2028, if not utilized. The Company filed certain amended state tax returns for tax years 2012-2015 during 2017 that resulted in increasing the Company's state NOL carryforward. As of December 31, 2017, 2016 and 2015, the Company had state NOL carryforwards of approximately \$327.8 million, \$18.1 million and \$15.4 million, respectively. The state NOL carryforwards will expire at various dates beginning in 2022, if not utilized.

A reconciliation of the U.S. statutory income tax rate to the Company's effective tax rate is as follows:

	December 31,		
	2017	2016	2015
Federal income tax (benefit) at statutory rate	(34.0)%	(34.0)%	(34.0)%
Change in tax rate	29.6%	0.1%	0.3%
Permanent items	0.1%	0.9%	1.3%
Other	(0.9)%	0.2%	0.0%
Amended Tax Returns	(4.5)%	0.0%	0.0%
Change in valuation allowance	9.7%	32.8%	32.4%
Effective income tax rate	<u>0.0%</u>	<u>0.0%</u>	<u>0.0%</u>

If the Company experiences a greater than 50 percentage point aggregate change in ownership of certain significant stockholders over a three-year period, a Section 382 ownership change could be deemed to have occurred. If a section 382 change occurs, the Company's future utilization of the net operating loss carryforwards and credits as of the ownership change will be subject to an annual

**Esperion Therapeutics, Inc.****Notes to the Financial Statements (Continued)****12. Income Taxes (Continued)**

limitation under Section 382 of the Internal Revenue Code of 1986, as amended, and similar state provisions. Such an annual limitation may result in the expiration of net operating losses before utilization.

The Company's reserves related to taxes are based on a determination of whether and how much of a tax benefit taken by the Company in its tax filings or positions is more likely than not to be realized following resolution of any potential contingencies present related to the tax benefit. The Company recognized no material adjustment for unrecognized income tax benefits. Through December 31, 2017, the Company had no unrecognized tax benefits or related interest and penalties accrued.

Significant components of the Company's deferred tax assets are summarized in the table below:

	<u>December 31,</u>	
	<u>2017</u>	<u>2016</u>
	(in thousands)	
Deferred tax assets:		
Federal and state operating loss carryforwards	\$ 88,637	\$ 65,972
Equity compensation	10,809	9,067
Temporary differences	402	226
Total deferred tax assets	<u>99,848</u>	<u>75,265</u>
Valuation allowance	(99,848)	(75,265)
Net deferred tax assets	<u>\$ —</u>	<u>\$ —</u>

**13. Net Loss Per Common Share**

Basic net loss per share is calculated by dividing net loss by the weighted-average number of common shares outstanding during the period, without consideration for common stock equivalents. Diluted net loss per share is computed by dividing net loss by the weighted-average number of common stock equivalents outstanding for the period determined using the treasury-stock method. For purposes of this calculation, warrants for common stock, stock options and unvested restricted stock and RSUs are considered to be common stock equivalents and are only included in the calculation of diluted net loss per share when their effect is dilutive.

The shares outstanding at the end of the respective periods presented below were excluded from the calculation of diluted net loss per share due to their anti-dilutive effect:

	<u>December 31,</u>		
	<u>2017</u>	<u>2016</u>	<u>2015</u>
Warrants for common stock	185,353	256,590	256,590
Common shares under option	4,159,151	3,255,987	2,662,862
Unvested restricted stock and RSUs	10,003	16,251	27,399
Total potential dilutive shares	<u>4,354,507</u>	<u>3,528,828</u>	<u>2,946,851</u>

Esperion Therapeutics, Inc.

Notes to the Financial Statements (Continued)

14. Selected Quarterly Financial Data (Unaudited)

The following table summarizes the unaudited quarterly financial data for the last two years:

	2017			
	March 31	June 30	September 30	December 31
	(in thousands, except share and per share data)			
<b>Operating expenses:</b>				
Research and development	\$ 35,860	\$ 38,248	\$ 40,056	\$ 33,439
General and administrative	5,029	5,412	5,681	5,257
Total operating expenses	40,889	43,660	45,737	38,696
<b>Loss from operations:</b>	(40,889)	(43,660)	(45,737)	(38,696)
Interest expense	(67)	(55)	(44)	(32)
Other income, net	415	378	562	837
Net loss	\$ (40,541)	\$ (43,337)	\$ (45,219)	\$ (37,891)
Net loss per common share (basic and diluted) <sup>(1)</sup>	\$ (1.80)	\$ (1.92)	\$ (1.86)	\$ (1.44)
Weighted-average shares outstanding (basic and diluted)	22,563,152	22,591,326	24,311,844	26,222,397

	2016			
	March 31	June 30	September 30	December 31
	(in thousands, except share and per share data)			
<b>Operating expenses:</b>				
Research and development	\$ 9,791	\$ 9,698	\$ 13,498	\$ 24,881
General and administrative	5,031	4,633	4,214	4,404
Total operating expenses	14,822	14,331	17,712	29,285
<b>Loss from operations:</b>	(14,822)	(14,331)	(17,712)	(29,285)
Interest expense	(110)	(99)	(89)	(78)
Other income, net	347	395	399	407
Net loss	\$ (14,585)	\$ (14,035)	\$ (17,402)	\$ (28,956)
Net loss per common share (basic and diluted)	\$ (0.65)	\$ (0.62)	\$ (0.77)	\$ (1.29)
Weighted-average shares outstanding (basic and diluted)	22,532,031	22,541,455	22,550,438	22,554,418

(1) Due to the use of weighted average shares outstanding for each quarter for calculating net loss per common share, the sum of the quarterly net loss per common share amounts may not equal the net loss per common share amount for the full year.





**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in the following Registration Statements:

- Registration Statement (Form S-8 No. 333-218084) pertaining to the 2017 Inducement Equity Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-216169) pertaining to the Amended and Restated 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-3 No. 333-208701) of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-208702) pertaining to the Amended and Restated 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-206180) pertaining to the Amended and Restated 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-201378) pertaining to the 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-194536) pertaining to the 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.
- Registration Statement (Form S-8 No. 333-189738) pertaining to the 2008 Incentive Stock Option and Restricted Stock Plan and the 2013 Stock Option and Incentive Plan of Esperion Therapeutics, Inc.

of our reports dated February 20, 2018, with respect to the financial statements of Esperion Therapeutics, Inc. and the effectiveness of internal control over financial reporting of Esperion Therapeutics, Inc. included in this Annual Report (Form 10-K) for the year ended December 31, 2017.

/s/ Ernst & Young LLP

Detroit, Michigan  
February 20, 2018

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QuickLinks

[Exhibit 23.1](#)

[Consent of Independent Registered Public Accounting Firm](#)

**CERTIFICATIONS UNDER SECTION 302**

I, Tim M. Mayleben, certify that:

1. I have reviewed this annual report on Form 10-K of Esperion Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 20, 2018

/s/ TIM M. MAYLEBEN

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Tim M. Mayleben  
*President and Chief Executive Officer*  
*(Principal Executive Officer)*

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QuickLinks

[Exhibit 31.1](#)

[CERTIFICATIONS UNDER SECTION 302](#)

**CERTIFICATIONS UNDER SECTION 302**

I, Richard B. Bartram, certify that:

1. I have reviewed this annual report on Form 10-K of Esperion Therapeutics, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
  - a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
  - b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
  - c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
  - d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
  - a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
  - b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: February 20, 2018

/s/ RICHARD B. BARTRAM

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Richard B. Bartram  
*Chief Financial Officer*  
*(Principal Financial Officer and*  
*Principal Accounting Officer)*

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QuickLinks

[Exhibit 31.2](#)

[CERTIFICATIONS UNDER SECTION 302](#)

[QuickLinks](#) -- Click here to rapidly navigate through this document

Exhibit 32.1

**CERTIFICATIONS UNDER SECTION 906**

Pursuant to section 906 of the Sarbanes-Oxley Act of 2002 (subsections (a) and (b) of section 1350, chapter 63 of title 18, United States Code), each of the undersigned officers of Esperion Therapeutics, Inc., a Delaware corporation (the "Company"), does hereby certify, to such officer's knowledge, that:

The Annual Report for the year ended December 31, 2017 (the "Form 10-K") of the Company fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, and the information contained in the Form 10-K fairly presents, in all material respects, the financial condition and results of operations of the Company.

Dated: February 20, 2018

/s/ TIM M. MAYLEBEN

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Tim M. Mayleben  
*President and Chief Executive Officer*  
*(Principal Executive Officer)*

/s/ RICHARD B. BARTRAM

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Richard B. Bartram  
*Chief Financial Officer*  
*(Principal Financial Officer and*  
*Principal Accounting Officer)*

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QuickLinks

[Exhibit 32.1](#)

[CERTIFICATIONS UNDER SECTION 906](#)