



**Esperion**Therapeutics<sup>®</sup>  
Beyond Statins

2013 ANNUAL REPORT



## TO OUR SHAREHOLDERS AND COLLEAGUES:

2013 was a highly productive and important year for Esperion with more successes, both clinically and financially, than in any other year since we were founded in April 2008. All these achievements would not have been possible without your support of the Esperion team. Among our 2013 business highlights, we completed a \$33 million private financing last April and an \$80 million initial public offering (IPO) in June, leaving Esperion well-funded to rapidly advance development of our lead program, ETC-1002. In the second half of 2013, Esperion (ESPR) was added to the NASDAQ Biotechnology Index, the Russell Global, Russell 3000, Russell 2000 and Russell Microcap Indices, as well as the MSCI Micro Cap Indices.

Our clinical and scientific colleagues were especially productive in 2013, publishing and presenting results from multiple Phase 2a clinical studies that, in aggregate, included data from 242 patients treated with ETC-1002. Results demonstrated consistent and significant reductions in LDL-cholesterol as high as 43 percent, and statin-like reductions in levels of high sensitivity C-reactive protein (hsCRP), a key marker of inflammation associated with cardiovascular disease. Esperion published a study in the Journal of Lipid Research that demonstrated, for the first time, the effectiveness of ETC-1002 in reducing chronic inflammation in preclinical models of inflammation. In June, the Journal of the American College of Cardiology published full results from our ETC-1002-003 study in patients with hypercholesterolemia and in September, we announced positive top-line results from our ETC-1002-007 clinical study demonstrating that ETC-1002 provided incremental LDL-cholesterol lowering when added on to statin therapy in patients with hypercholesterolemia. In November, Paul Thompson, M.D., of Hartford Hospital, made an oral presentation of the full results from our ETC-1002-006 study in patients with hypercholesterolemia and a history of statin intolerance at the 2013 American Heart Association Scientific Sessions.

Looking into 2014, we plan to deliver on a number of important clinical and non-clinical milestones including completing our ETC-1002-008 Phase 2b study and reporting top-line results in the fourth quarter. The ETC-1002-008 clinical study is focused on treating patients with hypercholesterolemia and a history with or without statin intolerance, which is defined as intolerance to two or more statins due to muscle-related adverse events. The goals of this study are to compare the LDL-cholesterol lowering efficacy of 1002 with ezetimibe, the active control and a common therapy for statin intolerance, and to further characterize the tolerability of ETC-1002.

In addition, Esperion will initiate and complete the ETC-1002-009 Phase 2b clinical study in 2014. We expect to report top-line results by the end of 2014. This study is designed to demonstrate the ability of ETC-1002 to achieve incremental LDL-cholesterol lowering on top of statin therapy in patients with elevated levels of LDL-cholesterol. The ETC-1002-009 study will use parallel doses of ETC-1002 for 12 weeks added on to low and moderate doses of statin therapy.

Finally, in the second quarter of 2014, and then again in the fourth quarter, we expect to report results from several non-clinical studies, including results from our long-term, 12-month toxicology study and our 2-year carcinogenicity studies. We believe 2014 will prove to be pivotal for ETC-1002 with both clinical and nonclinical results to be announced throughout the year. We look forward to providing you regular updates of our progress.



TIM MAYLEBEN | President And Chief Executive Officer

### Esperion Headquarters

3891 Ranchero Drive  
Suite 150  
Ann Arbor, MI 48108  
Phone: (734) 862-4840  
[www.esperion.com](http://www.esperion.com)





**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, 2013

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)

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

**46701 Commerce Center Drive**  
**Plymouth, Michigan 48170**  
(Address of Principal Executive Offices)

**48170**  
(Zip Code)

**(743) 862-4840**

(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 Global Market

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

Indicate by check mark if the registrant is a well-known seasoned issuer, as defined in Rule 405 of the Securities Act.

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, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act. (Check one):

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

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

An initial public offering, or the IPO, of the registrant's common stock, which is listed on The NASDAQ Global Market, closed on July 1, 2013. Upon the closing of the IPO, the registrant issued 5,000,000 shares of common stock in the IPO and an additional 9,210,999 shares of common stock upon the conversion of preferred stock. As of that date, the aggregate market value of the stock held by non-affiliates of the registrant computed by reference to the price of the registrant's common stock (based on the last reported sale price on The Nasdaq Global Market as of the last business day of the registrant's most recently completed second fiscal quarter) was \$204.6 million. As of June 28, 2013, the last business day of the registrant's most recently completed second fiscal quarter, which does not include the 5,000,000 shares of common stock issued in the IPO and the additional 9,210,999 shares of common stock issued upon the conversion of preferred stock, the aggregate market value of common stock held by non-affiliates of the registrant was \$5.6 million.

As of March 1, 2014, there were 15,394,226 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 2014 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, 2013.

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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 ETC-1002;
- the timing and outcome of our Phase 2 clinical studies of ETC-1002;
- the timing and outcome of our Phase 3 clinical program of ETC-1002, including two Phase 3 clinical studies and one long-term safety 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 ETC-1002, including as compared to statins, the standard of care for LDL-C lowering therapies, other currently available therapies or therapies in development;
- our ability to respond and adhere to changes in regulatory requirements, including any requirement to conduct additional, unplanned clinical studies, such as a cardiovascular outcomes study in connection with our pursuit of ETC-1002 as an LDL-C lowering therapy in the statin intolerant or other patient populations;
- the progress, timing and amount of expenses associated with our development of ETC-1002;
- 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 sell ETC-1002, if approved;
- the accuracy of our estimates of the size and growth potential of the statin intolerant market and the rate and degree of ETC-1002’s market acceptance, if it is approved;
- our ability to obtain and maintain intellectual property protection for ETC-1002 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 ETC-1002, 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 1.A. 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 a clinical stage biopharmaceutical company focused on developing and commercializing first-in-class, oral, low-density lipoprotein cholesterol (LDL-C) lowering therapies for the treatment of patients with hypercholesterolemia and other cardiometabolic risk markers. ETC-1002, our lead product candidate, is a unique, first-in-class, orally available, once-daily small molecule designed to lower LDL-C levels and avoid the side effects associated with other LDL-C lowering therapies currently available. ETC-1002 is being developed primarily for patients intolerant of statins with elevated levels of LDL-C. Phase 2b clinical trials for ETC-1002 are currently underway and build upon a successful and comprehensive Phase 1 and Phase 2 program. We own the exclusive worldwide rights to ETC-1002 and our other product candidates.

Statins are the current standard of care for LDL-C lowering for approximately 34 million patients in the United States. However, it is estimated that 2 - 7 million U.S. adults are intolerant of statin therapy due to muscle pain or weakness associated with statin therapy. We believe that ETC-1002, if approved, has the potential to become the preferred once-daily, oral therapy for patients who are unable to tolerate statin therapy. We also believe, because symptoms of muscle pain or weakness occur in up to 20% of patients on statin therapy in clinical practice, that the size of the statin intolerant market is poised to grow as effective non-statin therapies become available.

In October 2013, we initiated our Phase 2b clinical study in hypercholesterolemic patients with or without statin intolerance (ETC-1002-008), the first clinical study in our Phase 2b program. The ETC-1002-008 study is a 12-week Phase 2b study in approximately 322 patients who are either statin intolerant or statin tolerant. Patients enrolled in the ETC-1002-008 study will complete a five week placebo run-in period and will then be randomized to one of five arms: 1) 120 mg dose of ETC-1002, 2) 180 mg dose of ETC-1002, 3) an active comparator, 10 mg dose of ezetimibe, 4) a combination of 120 mg of ETC-1002 and ezetimibe, or 5) a combination of 180 mg of ETC-1002 and ezetimibe. This Phase 2b clinical study is a parallel dose design with a 12-week duration. The primary objective is to assess the LDL-C lowering efficacy of ETC-1002 monotherapy versus ezetimibe monotherapy in patients with elevated LDL-C levels with or without statin intolerance. In addition, the study will assess the LDL-C lowering efficacy of ETC-1002 in combination with ezetimibe versus ezetimibe monotherapy. We expect to complete the study by the end of 2014.

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 cholesterol, or HDL-C. After successfully completing a Phase 2a clinical study with its synthetic HDL therapy, the original Esperion was acquired by Pfizer Inc. in 2004. ETC-1002 was first discovered at the original Esperion and we subsequently acquired the rights to the product from Pfizer in 2008.

## Our Strategy

Our objective is to be a leader in the discovery, development and commercialization of novel therapies for the treatment of patients with hypercholesterolemia and intolerance to statin therapy. The core elements of our strategy include:

- ***Rapidly advance the clinical development of ETC-1002 as a novel, first in class, orally available, once-daily, small molecule therapy for hypercholesterolemic patients who are statin intolerant.*** In November 2013 at the Scientific Sessions of the American Heart Association, we presented efficacy and safety results from ETC-1002-006, our Phase 2a clinical study in patients with elevated LDL-C and a history of intolerance to two or more statins. We initiated a Phase 2b clinical study in approximately 322 statin intolerant and statin tolerant patients in October 2013 and expect to report top-line results by the end of 2014. This Phase 2b clinical study includes a comparison with Zetia® (ezetimibe), which we believe is currently the most prescribed non-statin LDL-C lowering therapy. Zetia's worldwide sales total more than \$2.5 billion, approximately half of which are estimated to be for the treatment of statin intolerant patients. While we have not yet completed any comparative clinical studies, Zetia has reported LDL-C lowering of up to an average of 18% in two pivotal clinical studies and ETC-1002 has demonstrated LDL-C lowering up to an average of 43% in clinical studies to date. Because of its superior LDL-C lowering and an attractive safety and tolerability profile, we believe that ETC-1002, if approved, has the potential to become the preferred orally available, once-daily LDL-C lowering small molecule therapy for hypercholesterolemic patients who are unable to tolerate statin therapy.
- ***Demonstrate ETC-1002's potential as an add-on therapy for hypercholesterolemic patients who cannot achieve their LDL-C goals despite the use of statin therapy.*** In September 2013, we announced top-line safety, tolerability, pharmacokinetics and efficacy results from ETC-1002-007, our Phase 2a clinical study using increasing doses of ETC-1002 as an add-on to atorvastatin calcium. In March 2014, we expect to initiate a Phase 2b clinical study (ETC-1002-009) in approximately 132 patients with hypercholesterolemia who will also be taking a statin. Patients in our Phase 2b clinical study will receive two dose strengths of ETC-1002 as an add-on to low to moderate doses of the four most commonly prescribed statins, which include, atorvastatin, pravastatin, simvastatin and rosuvastatin.
- ***Develop ETC-1002 for LDL-C lowering in targeted patient populations, and develop our other product candidates to treat other cardiometabolic risk markers in additional patient populations.*** We may initiate additional clinical studies to explore ETC-1002 as a potential therapy for patients with multiple cardiometabolic risk markers, including elevated levels of hsCRP, blood glucose, and blood pressure. In addition, we may advance the clinical development of two early-stage product candidates to which we own the exclusive worldwide rights: ESP41091, a small molecule oral therapy; and 4WF, a synthetic apoA-I mimetic targeted for patients with acute coronary syndrome.
- ***Maintain flexibility in commercializing and maximizing the value of our development programs.*** We may enter into strategic relationships with biotechnology or pharmaceutical companies to optimize the value of ETC-1002 or our other earlier-stage development programs. For ETC-1002, we may enter into one or more strategic relationships to access broader geographic markets, pursue broader LDL-C lowering indications and populations or pursue indications outside of LDL-C lowering.

## ETC-1002

ETC-1002 is a novel, first in class, orally available, once-daily LDL-C lowering small molecule therapy with a unique dual mechanism of action. ETC-1002 is differentiated from statins because it acts at an earlier step in the cholesterol biosynthetic pathway. ETC-1002 works by inhibiting the ATP citrate



lyase (ACL) enzyme and activating 5'-adenosine monophosphate-activated protein kinase (AMPK), whereas statins have a mechanism of action that directly inhibits the rate-limiting enzyme, HMG-CoA reductase, in the cholesterol biosynthetic pathway. Reductions in LDL-C levels resulting from statin therapy are ultimately due to reduced cholesterol synthesis and an increase in the number of LDL receptors in the liver. By inhibiting the ACL enzyme, ETC-1002 achieves LDL-C lowering comparable to moderate-dose statins and we believe provide will provide incremental lowering of LDL-C when used in combination with statins.

Dr. Newton and his scientific team first discovered ETC-1002 at the original Esperion, and we subsequently acquired its exclusive worldwide rights from Pfizer in 2008. Initially, we intend to seek approval of ETC-1002 as a therapy for patients with elevated levels of LDL-C who are unable to tolerate statin therapy due to muscle pain or weakness. Subsequently, we may seek approval of ETC-1002 in a broader population of patients who are unable to achieve their LDL-C goals, despite being on statin therapy, and therefore remain at an increased risk for cardiovascular disease.

### ***Cardiovascular Disease and Hypercholesterolemia***

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 estimates that approximately 800,000 deaths in the United States were caused by cardiovascular disease in 2009.

Elevated LDL-C is well-accepted as a significant risk factor for cardiovascular disease and the CDC estimates that 71 million U.S. adults have elevated levels of LDL-C. A consequence of elevated LDL-C is atherosclerosis, which is a disease that is 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 the 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 and elevated blood pressure were identified early on as key risk factors 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 based on its ability to significantly 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 hypercholesterolemia. Over the subsequent 20 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 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 "lower is better" hypothesis. This hypothesis was tested and proven in the PROVE-IT (Pravastatin or Atorvastatin Evaluation and Infection Therapy) study where an on-treatment LDL-C level of 62 mg/dL associated

with atorvastatin treatment translated into a statistically significant 16% reduction in risk of major cardiovascular events as compared with the 95 mg/dL on-treatment LDL-C level associated with pravastatin.

The direct relationship between lower LDL-C levels and reduced risk for major cardiovascular events has been consistently demonstrated for more than a decade in 14 clinical studies 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, increasing attention has been placed on aggressive LDL-C management by organizations such as the National Cholesterol Education Program, or NCEP, the American Heart Association, and the American College of Cardiology. 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 Zetia.

In July 2004, the NCEP issued an update to its Adult Treatment Panel III (ATP 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, expanding the number of potential patients for LDL-C lowering therapy.

**NCEP ATP III Clinical Practice Guidelines**

<u>Patient Cardiovascular Disease Risk</u>	<u>LDL-C Goal</u>
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 and the American Heart Association issued new guidelines for the treatment of elevated cholesterol. For the first time in more than 20 years, the new guidelines do not include specific, numerical LDL-C treatment goals for hypercholesterolemic patients. However, the guidelines strongly recommend the use of more potent statins and intensive statin therapy in patients with hypercholesterolemia. The new 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.

### Currently Approved Therapies

The following table illustrates common therapies used to treat hypercholesterolemia:

Class of Therapy	Labeled Indication	Average LDL-C Change from Baseline	Key Side Effects
Statins	Reduction in LDL-C	Up to 63%	<ul style="list-style-type: none"> <li>Skeletal muscle effects (e.g., myopathy and rhabdomyolysis)</li> <li>FDA recently warned that people being treated with statins may have an increased risk of raised blood sugar levels and the development of type 2 diabetes</li> </ul>
Fixed combination therapies	Reduction in LDL-C	Up to 63%	<ul style="list-style-type: none"> <li>Includes a statin as one of the underlying therapies and therefore contains the same side effects outlined above</li> </ul>
Bile acid sequestrants	Reduction in LDL-C(1)	Up to 20%	<ul style="list-style-type: none"> <li>Gastrointestinal disorders</li> </ul>
Cholesterol absorption inhibitors	Reduction in LDL-C	Up to 18%	<ul style="list-style-type: none"> <li>Limited</li> </ul>
Niacin	Reduction in LDL-C; Reduction in recurrent myocardial infarction	Up to 17%	<ul style="list-style-type: none"> <li>Flushing (i.e., warmth or redness) hepatic toxicity and skeletal muscle effects</li> </ul>
Fibrates	Reduction in triglycerides and LDL-C	Up to 21%	<ul style="list-style-type: none"> <li>Gallstones, skeletal muscle effects and liver disorders</li> </ul>

- (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 300 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 receptors and as a result, 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. MTP inhibitors and ApoB antisense drugs are approved therapies to treat patients with a clinical or laboratory diagnosis of HoFH. Given the serious safety concerns with these therapies, specifically hepatotoxicity, the FDA has restricted their usage to this narrow subpopulation.

### Statin Therapy

Statins are the cornerstone of lipid treatment today and are highly effective at lowering LDL-C. This class of drugs includes atorvastatin calcium, marketed as Lipitor®, the most prescribed LDL-C drug in the world and the best-selling pharmaceutical in history. Approximately 25% of Americans over the age of 45 from 2005 to 2008 were treated for elevated LDL-C levels with a statin therapy, according to a National Health and Nutrition Examination Survey.

Statins are selective, competitive inhibitors of HMG-CoA reductase, a rate-limiting enzyme in the cholesterol biosynthesis pathway, and work primarily 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 enhances uptake of LDL particles into liver cells from the circulation, thus lowering LDL-C levels.

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 37 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 novel therapies to treat patients with hypercholesterolemia.

#### ***Statin Intolerance—Initial Market Opportunity for ETC-1002***

We are initially pursuing the clinical development of ETC-1002 as a therapy for patients with hypercholesterolemia who are statin intolerant. Based upon our communications with the FDA, statin intolerance is defined as the inability to tolerate at least two statins, one of which was taken at the lowest approved dose, due to skeletal muscle pain, aches, weakness or cramping, that manifested or increased during statin therapy and stopped upon the discontinuation of statin usage.

Muscle pain or weakness is the most common side effect experienced by statin users and the most common cause for discontinuing therapy. According to the USAGE survey, an approximately 10,000 patient academic study of current and former statin users published during 2012 in the Journal of Clinical Lipidology, 12% of patients on statins discontinue therapy and 62% of these patients cited side effects as the reason for discontinuation. More than 86% of patients who discontinued therapy because of side effects cited muscle pain or weakness as the reason. Based upon these data, approximately 6% of statin users, or more than 2 million adults in the United States, ceased therapy because of muscle pain or weakness and are therefore statin intolerant.

Moreover, a significant proportion of patients remain on statin therapy despite experiencing muscle-related side effects. The rate of occurrence in the clinical setting, as highlighted by the USAGE survey, is significantly higher than the up to 5% rate reported by subjects in the controlled environment of clinical studies. The USAGE survey reported that 25% of patients currently on statins have muscle-related side effects. Similarly, a study published in the Journal of General Internal Medicine in August 2008 estimated that up to 20% of statin-treated patients in clinical practice complained of muscle pain. Accordingly, we believe that in the presence of a safe and effective non-statin, oral, once-daily, small molecule LDL-C lowering therapy, the statin intolerant market could grow substantially.

#### ***Hypercholesterolemic Patients—Subsequent Market Opportunity for ETC-1002***

In addition to developing ETC-1002 for the treatment of statin intolerant patients, we expect to continue to develop ETC-1002 as an add-on therapy for hypercholesterolemic patients who are unable to reach their recommended LDL-C goals despite the use of statin therapy. The severity of hypercholesterolemia in these patients, their level of cardiovascular disease risk and their therapeutic options all vary widely.



### **Additional Therapies in Development—PCSK9 Inhibitors**

A number of larger biopharmaceutical companies are currently developing a new class of biologic therapies that target proprotein convertase subtilisin/kexin type 9, or PCSK9, an enzyme that binds LDL receptors. These PCSK9 inhibitors, which are still in clinical development, are injectable, fully-human antibodies that are being evaluated as potential therapies to lower LDL-C, including in patients who are statin intolerant or who are statin resistant. In October 2013, Sanofi and Regeneron Pharmaceuticals Inc. announced topline results for the first Phase 3 study of alirocumab, their PCSK9 inhibitor. In December 2013, Amgen Inc. announced topline results for the first two Phase 3 studies of evolocumab, their PCSK9 inhibitor. Also in 2013, Pfizer Inc. announced that it initiated Phase 3 studies of bococizumab. In monotherapy clinical studies to date, PCSK9 inhibitors have demonstrated significant reductions of LDL-C, up to 51%. The PCSK9 inhibitors, if approved, could be an effective therapeutic alternative for statin intolerant patients or as an add on to, statin therapy. Notwithstanding the LDL-C lowering efficacy, we believe the adoption of PCSK9 inhibitor therapy by patients, physicians, and payers will be impacted by the higher cost of biologic therapies, the inconvenient route of administration and the inability to positively impact other important cardiometabolic risk markers.

### ***Clinical Experience***

To date, ETC-1002 has been studied in seven clinical trials across five patient populations: healthy volunteers; 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; and patients with elevated LDL-C levels taking 10 mg of atorvastatin. The first six (6) clinical studies compared ETC-1002 monotherapy to placebo. In ETC-1002-007, the most recent clinical study, ETC-1002 was administered as an add-on to a 10 mg dose of atorvastatin. These clinical trials consisted of four Phase 2a clinical trials and three Phase 1 clinical trials. The individual design and results of each of our completed clinical trials are discussed below.

## Completed Clinical Studies

To date, we have completed the following clinical studies of ETC-1002:

Description	Title	Treatment Duration	Subjects	
			Total	Treated
ETC-1002-007	<b>Phase 2a Clinical Study of Safety and Pharmacokinetic Interaction in Patients with Hypercholesterolemia on a Background of Atorvastatin 10 mg</b> Placebo-controlled, randomized, double-blind, drug interaction study to evaluate the safety, tolerability and effect on atorvastatin pharmacokinetics of ETC-1002 added to atorvastatin 10 mg/day in patients with hypercholesterolemia	8 Weeks	58	42
ETC-1002-006	<b>Phase 2a Proof of Concept Clinical Study in Patients with Hypercholesterolemia and a History of Statin Intolerance</b> Placebo-controlled, randomized, double-blind, multicenter study to evaluate the efficacy and safety of ETC-1002 in patients with hypercholesterolemia and a history of intolerance to statin therapy	8 Weeks	56	37
ETC-1002-005	<b>Phase 2a Proof of Concept Clinical Study in Patients with Hypercholesterolemia and Type 2 Diabetes</b> Placebo-controlled, randomized, double-blind, single site clinical study to evaluate the LDL-C lowering efficacy and safety of ETC-1002 in patients with type 2 diabetes	4 Weeks	60	30
ETC-1002-004	<b>Phase 1b Multiple-Dose Tolerance Greater Than 120 mg Clinical Study</b> Multiple ascending dose clinical study to evaluate safety, tolerability and pharmacokinetics (PK) of ETC-1002 in doses greater than 120 mg once-daily in healthy subjects	2 Weeks	24	18
ETC-1002-003	<b>Phase 2a Proof of Concept Clinical Study in Hypercholesterolemic Patients</b> Placebo-controlled, randomized, double-blind, parallel group, multicenter clinical study to evaluate the LDL-C lowering efficacy and safety of ETC-1002 in patients with hypercholesterolemia and either normal or elevated triglycerides	12 Weeks	177	133
ETC-1002-002	<b>Phase 1b Multiple-Dose Tolerance Clinical Trial</b> Multiple ascending dose clinical trial to evaluate safety, tolerability, PK and pharmacodynamics (PD) of ETC-1002 in doses of up to 120 mg once-daily in healthy subjects	2 Weeks / 4 Weeks	53	39
ETC-1002-001	<b>Phase 1a Single-Dose Tolerance Clinical Trial</b> First-in-human single-dose clinical trial to evaluate safety, tolerability and PK of ETC-1002 in healthy subjects	Single Dose	18	18

Overall, ETC-1002 has been well-tolerated and associated with no dose limiting adverse events. A single patient dosed with ETC-1002 has experienced a serious adverse event, or SAE, which was assessed by the principal investigator at that clinical site as unrelated to ETC-1002. Two patients receiving placebo have also experienced SAEs.

### *Phase 2a Clinical Studies*

#### **ETC-1002-007—Phase 2a Clinical Study of Safety and Atorvastatin Pharmacokinetic Interaction in Patients with Hypercholesterolemia on a Background of Atorvastatin 10 mg**

ETC-1002-007 was an eight-week Phase 2a clinical study in 58 patients, of whom 42 were dosed with ETC-1002, across six participating clinical recruitment sites in the United States. Although the trial was not designed to assess LDL-C lowering with ETC-1002, this was measured as a secondary endpoint to determine whether incremental LDL-C lowering would occur with ETC-1002 added on a background of statin therapy. The results of this clinical study are summarized as follows:

- ETC-1002 dosed as an add-on to 10 mg of atorvastatin was well tolerated and did not result in any serious adverse events
- In patients on a background of atorvastatin, ETC-1002 reduced LDL-C levels, a secondary endpoint, by an average of 22% versus 0% change with placebo ( $p < 0.0001$ ).
- Mean LDL-C level in patients on a background of atorvastatin 10 mg prior to treatment with ETC-1002 or placebo in 1002-007 was 106 mg/dL; this baseline LDL-C level is relatively low.
- No significant changes in HDL-C or triglyceride levels were observed.
- ETC-1002 demonstrated a weak pharmacokinetic interaction with atorvastatin.

#### **ETC-1002-006—Phase 2a Proof of Concept Clinical Study in Patients with Hypercholesterolemia and a History of Statin Intolerance**

ETC-1002-006 was an eight-week Phase 2a proof-of-concept clinical study in 56 patients, of whom 37 were dosed with ETC-1002, across five participating clinical recruitment sites in the United States. This clinical study was designed to evaluate the LDL-C lowering efficacy, tolerability and safety of ETC-1002 versus placebo in patients with hypercholesterolemia and a history of intolerance to two or more statins due to muscle pain or weakness. After completing a lipid-lowering therapy wash-out and two weeks of dosing with placebo, eligible patients were randomized to receive ETC-1002 or placebo in a 2:1 ratio for eight weeks. Patients were given increasing doses of ETC-1002 of 60 mg, 120 mg, 180 mg and 240 mg for two weeks each (or placebo only for the full 8 weeks). The primary endpoint of this clinical study was LDL-C lowering from baseline to end of study. The results of this clinical study are summarized as follows:

- LDL-C levels after eight weeks of treatment of ETC-1002, which was the primary endpoint, were reduced by an average of 32% for patients dosed with ETC-1002, compared to an average of 3% for patients dosed with placebo ( $p < 0.0001$ ).
- Drop-out rates and muscle related adverse events were comparable to placebo and no patients treated with ETC-1002 discontinued the trial because of muscle related adverse events.
- hsCRP, a marker of inflammation, was reduced by 42% after eight weeks of ETC-1002 therapy versus 0% on placebo ( $p = 0.0022$ ).
- No significant changes in HDL-C or triglyceride levels were observed.

#### **ETC-1002-005—Phase 2a Proof of Concept Clinical Study in Patients with Type 2 Diabetes**

ETC-1002-005 was a four-week Phase 2a proof-of-concept clinical study at a single site. This clinical study was designed to evaluate the LDL-C lowering efficacy and safety of ETC-1002 in patients with type 2 diabetes. One treatment arm was placebo and the other was 80 mg of ETC-1002, once-daily

for two weeks, followed by 120 mg of ETC-1002, once-daily for two additional weeks. The key results of this clinical study are summarized as follows:

- LDL-C levels after four weeks of treatment of ETC-1002, which is the primary endpoint, were reduced by an average of 43% for patients on the 120 mg dose of ETC-1002 compared to an average of 4% for patients dosed with placebo ( $p < 0.0001$ ).
- Approximately 80% of the patients were not at their NCEP ATP III LDL-C goal of less than 100 mg/dL at the beginning of the study. Of these, 88% of the patients dosed with ETC-1002 achieved their goal by study end as compared to 4% of patients dosed with placebo ( $p < 0.0001$ ).
- hsCRP was reduced by 41% on the 120 mg dose of ETC-1002 versus 11% on placebo ( $p = 0.001$ ).
- HDL-C and triglyceride levels were unchanged in both treatment arms.
- Intensive assessment of glycemic parameters using blood sampling and 24 hour continuous glucose monitoring showed no worsening of blood glucose with ETC-1002 treatment. Treatment with ETC-1002 resulted in modest trends toward improved glycemic control and insulin resistance.
- Non-HDL-C decreased by 32% for patients dosed with ETC-1002 as compared to an increase of 1% for patients dosed with placebo ( $p < 0.0001$ ).
- No SAEs were observed in patients dosed with ETC 1002. ETC 1002 was safe, well tolerated and associated with no dose limiting side effects.

#### **ETC-1002-003—Phase 2a Proof of Concept Clinical Study in Hypercholesterolemic Patients**

ETC-1002-003 was a 12-week Phase 2a proof-of-concept study in 177 patients, of whom 133 were dosed with ETC-1002, across 11 participating clinical recruitment sites in the United States. This clinical study was designed to evaluate the LDL-C lowering efficacy and safety of ETC-1002 versus placebo in patients with hypercholesterolemia (LDL-C of 130 to 220 mg/dL) and either normal (less than 150 mg/dL) or elevated triglycerides (150 to 400 mg/dL). The four arms were placebo and 40 mg, 80 mg and 120 mg doses of ETC-1002 once-daily. The key results of this clinical study are summarized as follows:

- LDL-C levels were reduced by an average of 18%, 25% and 27% for patients dosed with ETC-1002 40, 80 and 120 mg of ETC-1002, respectively, compared to an average of 2% for patients dosed with placebo ( $p < 0.0001$ ). ETC-1002's lowering of LDL-C levels was maintained across a range of baseline triglycerides levels.
- ETC-1002 also lowered corresponding levels of the atherogenic biomarkers, apolipoprotein (apo) B, non-HDL-C and LDL particle number ( $p < 0.0001$ ) in a dose-dependent manner.
- Patients dosed with ETC-1002 demonstrated a trend in hsCRP reduction of 20% to 26% compared to 2% in patients dosed with placebo. In a subgroup of patients with elevated hsCRP, patients dosed with ETC-1002 demonstrated a trend in hsCRP reduction of 43% to 64% compared to a decrease of 7% for patients dosed with placebo.
- HDL-C and triglyceride levels were unchanged across all treatment arms.
- There were no SAEs observed in patients dosed with ETC-1002. ETC-1002 was safe, well-tolerated and associated with no dose-limiting side-effects.



### ***Phase 1 Clinical Trials***

Our completed Phase 1 clinical trials of ETC-1002 exposed subjects in one single dose tolerance test and two multiple dose tolerance tests. Our single dose tolerance test dosed subjects with up to 250 mg of ETC-1002. Our multiple dose tolerance tests dosed subjects with up to 120 mg and 220 mg of ETC-1002, respectively. We did not identify any dose-limiting side effects in either the single dose tolerance test or the multiple dose tolerance tests, and ETC-1002 was safe and well-tolerated in each clinical trial. In addition, LDL-C was lowered rapidly in the multiple dose tolerance tests, including in as early as five days, and we observed an average reduction in LDL-C levels of up to 36%.

#### **ETC-1002-004—Phase 1b Multiple Dose Tolerance Greater Than 120 mg Clinical Trial**

ETC-1002-004 was a two-week, Phase 1b, multiple dose tolerance clinical trial in 24 subjects, of whom 18 were dosed with ETC-1002. This clinical trial was designed to evaluate the safety and tolerability of escalating, multiple oral doses of ETC-1002 above 120 mg/day. Subjects in this clinical trial received 140, 180, or 220 mg of ETC-1002 or placebo once-daily for 14 days. The key pharmacodynamic results of this clinical trial are as follows:

- LDL-C levels were reduced by an average of 36% for subjects dosed with 220 mg/day of ETC-1002 as compared to a 4% increase for subjects dosed with placebo ( $p < 0.0001$ ). ETC-1002's effect on LDL-C lowering was robust notwithstanding non-elevated baseline LDL-C levels.
- The pharmacokinetics of ETC-1002 were well-characterized and supported once-daily dosing.
- No SAEs were observed in the subjects dosed with ETC-1002. ETC-1002 was safe, well-tolerated and associated with no dose-limiting side-effects.

#### **ETC-1002-002—Phase 1b Multiple-Dose Tolerance Clinical Trial**

ETC-1002-002 was a staged two-week and four-week Phase 1b multiple dose tolerance clinical trial in 53 subjects with 39 receiving ETC-1002 and 23 receiving placebo. The subjects were divided into four different cohorts of six subjects with each receiving 20, 60, 100 or 120 mg of ETC-1002 or placebo once-daily for 14 days. This was followed by a larger cohort that was treated for 28 days during which subjects lived outside of the clinical site for the duration of their treatment. This clinical trial demonstrated that the pharmacokinetics of ETC-1002 were well characterized and supported once-daily dosing.

- The pharmacokinetics of ETC-1002 were well-characterized and supported once-daily dosing.
- No SAEs were observed in the subjects dosed with ETC-1002. ETC-1002 was safe, well-tolerated and associated with no dose-limiting side-effects.

#### **Overall Safety Observations**

To date, 317 subjects have been treated with ETC-1002 for periods of up to 12 weeks at maximum repeated doses of 240 mg per day. ETC-1002 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 although very modest shifts in group mean levels of hemoglobin, uric acid, alkaline phosphatase and homocysteine were identified in some of our completed clinical studies. The

clinical relevance of these shifts is not readily apparent and will be monitored in our future clinical studies.

<b>Trial</b>	<b>Phase</b>	<b>Patient Population</b>	<b>Trial Design</b>	<b>Duration</b>	<b>Patients (Treated)</b>	<b>Doses</b>	<b>LDL Lowering Efficacy</b>
ETC-1002-001	Phase 1a	Healthy subjects	Single dose, PK	Single dose	18 (18)		
ETC-1002-002	Phase 1b	Healthy subjects	Multiple ascending dose, PK/PD	2/4 weeks	53 (39)	20, 60, 100, 120 mg	<b>Up to 17%</b>
ETC-1002-003	Phase 2a	Elevated LDL	Placebo controlled	12 weeks	177 (133)	40, 80, 120 mg	<b>Up to 27%</b>
ETC-1002-004	Phase 1b	Healthy subjects	Multiple ascending dose, PK	2 weeks	24 (18)	40, 180, 220 mg	<b>Up to 36%</b>
ETC-1002-005	Phase 2a	Elevated LDL; T2DM	Placebo controlled	4 weeks	56 (37)	80, 120 mg	<b>Up to 43%</b>
ETC-1002-006	Phase 2a	Elevated LDL; statin intolerant	Placebo controlled	8 weeks	60 (30)	60, 120, 180, 240 mg	<b>Up to 33%</b>
ETC-1002-007 . . .	Phase 2a	Elevated LDL; statin add-on	Placebo controlled, 10 mg atorvastatin	8 weeks	58 (2)	60, 120, 180, 240 mg	<b>Up to 22%</b>

### **Ongoing and Planned Clinical Studies**

#### ***Statin Intolerant Population (ETC-1002-008)***

##### ***ETC-1002-008***

ETC-1002-008 is a 12-week study of the treatment of elevated LDL-C levels in approximately 322 patients either with or without statin intolerance (50% of patients in the trial will meet the definition of statin intolerance) across 70 participating clinical sites in the US. The purpose of this clinical trial is to inform dosing for our Phase 3 program, directly compare the LDL-C lowering efficacy of ETC-1002 versus ezetimibe, and assess safety and tolerability, including muscle-related adverse events, in patients with or without statin intolerance. ETC-1002-008 utilizes two doses of ETC-1002 in a parallel group design of 12 weeks duration, compared with ezetimibe, a common treatment for statin intolerance. The LDL-C lowering efficacy of ETC-1002 in combination with ezetimibe will also be assessed. The goal is to demonstrate comparable tolerability of ETC-1002 monotherapy with superior efficacy to ezetimibe for the treatment of patients with elevated LDL-C levels either with or without intolerance to two or more statins due to muscle-related adverse events. We initiated ETC-1002-008 in October 2013.

#### ***Add-on to Statin Population (ETC-1002-009)***

The objective of this add-on to statin therapy clinical study is to support Phase 3 dosing of ETC-1002 as an add-on to low and moderate doses of statins in patients with elevated levels of LDL-C.

##### ***ETC-1002-009***

ETC-1002-009 will be a 12-week study for the treatment of approximately 132 patients with ETC-1002 as an add-on to statin therapy across 28 participating clinical sites in the U.S. Many hypercholesterolemic patients on statin therapy do not achieve adequate lowering of their LDL-C levels. The goal will be to demonstrate that ETC-1002, when added onto statin therapy, will lead to greater LDL-C lowering and fewer side effects. We expect to initiate ETC-1002-009 in March 2014.

## *Additional Regulatory Studies*

### *Phase 3 Clinical Studies*

We plan to use the results of our Phase 2 clinical studies to inform dosing for our Phase 3 clinical studies. We will conduct these Phase 3 clinical studies in larger patient populations to further evaluate clinical doses, and the efficacy and safety of ETC-1002 in an expanded patient population at geographically dispersed clinical study sites. Any such Phase 3 clinical studies and any additionally required long-term safety study, would be intended to establish the overall risk/benefit ratio of ETC-1002 and to provide an adequate basis for regulatory approval of ETC-1002.

The current Phase 3 clinical program is planned to include two pivotal efficacy studies in patients with statin intolerance and one long term safety study. The doses of ETC-1002 utilized in Phase 3 will be informed by the results of our Phase 2 studies. The overall program will be based upon agreed upon study designs/ duration and size based on an end of Phase 2b meeting with FDA.

### *Studies in Response to Partial Clinical Holds*

In 2009, the FDA determined that ETC-1002 was a potential peroxisome proliferator activated receptor, or PPAR, agonist and as a result was subject to a partial clinical hold. The FDA has issued such notices to all sponsors of PPARs or agents deemed to have PPAR-like properties. The partial clinical hold permits clinical studies of up to six months' duration for ETC-1002 and also requires us to conduct two year rat and mouse carcinogenicity studies before initiating clinical studies of longer than six months. The in-life phase of our two year rat and mouse carcinogenicity studies are scheduled for completion by April and May 2014 and draft reports will be issued by the end of 2014.

The clinical data to date appear to demonstrate the absence of PPAR mediated pharmacology (triglyceride decreases, adiponectin increases, mild ALT increases) or toxicity (weight gain, edema, creatinine kinase/creatinine increases) in humans. This is supportive of the conclusion that the weak PPAR alpha/gamma activities observed at high doses of ETC-1002 in vitro and in animal models preclinically are not observed with therapeutic doses of ETC-1002 in humans. These effects will continue to be monitored in our future clinical program. Most importantly, our clinical studies have demonstrated rapid and significant LDL-C lowering consistent with the dual mechanisms of action inhibiting ATP-citrate lyase and activating hepatic AMPK.

In addition, based upon early preclinical toxicology results, the FDA has limited our ability to dose ETC-1002 above 240 mg in our clinical studies. We do not plan to dose ETC-1002 above 240 mg. We recently completed the in-life phase of our long term, chronic toxicology studies in monkeys (12 months) and rats (6 months) and final reports from these studies will be issued and filed with FDA in the second quarter of 2014.

If we are unable to address FDA's concerns related to the partial clinical holds, we could be delayed in, or prevented from, obtaining marketing approval of ETC-1002. Additionally, FDA could raise these concerns as part of the NDA review process for ETC-1002, which could result in adverse limitations in any approved labeling or on distribution and use of ETC-1002, if approved.

### *Pharmacology and Toxicology Studies*

Our pre-clinical studies of ETC-1002 have demonstrated favorable effects on plasma LDL-C and triglycerides, blood pressure, blood glucose and insulin levels, inflammation and weight gain in diet-induced and genetic pre-clinical models of dyslipidemia, diabetes, and obesity. In a progression model of atherosclerosis using a LDL-receptor deficient mouse model, ETC-1002 demonstrated reductions in atherosclerotic plaque content and size with beneficial changes in inflammatory markers.

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

ETC-1002 has dual mechanisms of action targeting both ATP citrate lyase and AMPK. ETC-1002 works in the liver and, once in the liver inhibits ACL and activates AMPK. Pre-clinical studies have shown that ETC-1002 is activated to a coenzyme A derivative, or ETC-1002-CoA, which directly inhibits ACL, a key enzyme that supplies substrate for cholesterol and fatty acid synthesis. Studies in liver cells show that inhibition of ACL by ETC-1002 increases LDL receptor activity in a manner similar to statins, which are known to reduce LDL-C largely through this mechanism. Activation of AMPK by ETC-1002 complements the effects of ACL inhibition in the liver and is believed to contribute to the beneficial effects on other cardiometabolic risk markers including hsCRP, insulin sensitization, blood pressure and weight. While the relative contributions of ACL inhibition and AMPK activation are currently under investigation, these mechanisms are supported by preclinical and clinical observations that have been published in peer reviewed publications and presented at scientific conferences. We are not aware of any alternative explanations regarding ETC 1002's dual mechanisms of action or the preliminarily accepted conclusion in the scientific community that inhibiting ACL and activating AMPK have the potential to regulate metabolic imbalances in both the lipid and carbohydrate metabolic pathways, which do not function normally in specific patient populations with specific cardiometabolic risk markers.

### **Early-Stage Product Candidates**

#### ***ESP41091***

We acquired the exclusive worldwide rights to ESP40191 from Pfizer in April 2008. ESP41091, our second product candidate, is a pre-IND compound. In pre-clinical pharmacology studies, treatment with ESP41091 also resulted in beneficial effects on lipid metabolism and body weight in obese Zucker rats. Oral intervention with ESP41091 resolved hyperglycemia and reduced body weight following a four week treatment in a diet-induced obese mouse model of insulin resistance.

#### ***4WF***

Our management team has prior success in the identification and clinical development of synthetic apoA-I therapies. ApoA-I is the primary protein in HDL. At the original Esperion, we licensed apoA-I Milano, a synthetic apoA-I therapy, and successfully completed a Phase 2a clinical study showing regression of atherosclerosis in high-risk acute coronary syndrome patients after four weeks of therapy. In June 2011, we acquired the exclusive worldwide rights to 4WF from the Cleveland Clinic Foundation. 4WF is a next generation synthetic apoA-I therapy designed to preserve the function of HDL and apoA-I, and to deliver oxidation-resistant synthetic apoA-I therapy via an injection as opposed to intravenous infusion. Moreover, recent research demonstrates that HDL becomes dysfunctional and loses its cholesterol acceptor and anti-inflammatory activity through myeloperoxidase mediated enzymatic oxidation. We believe the preferred means to improve HDL function is to increase the number and activity of HDL particles in the body through synthetic apoA-I therapy. We believe our initial in vitro protein screening and characterization suggest the benefits of 4WF as an optimized myeloperoxidase oxidation-resistant synthetic apoA-I therapy.

### **Research and Development Expenses**

Research and development expenses for the year ended December 31, 2013 were \$16.0 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 ETC-1002 in the United States, if approved, as a treatment for elevated levels of LDL-C in statin intolerant patients, we would need to invest significant financial and managerial resources. We may engage in partnering discussions with third parties from time to time. If we elect to seek approval and launch commercial sales of ETC-1002 outside of the United States or for broader patient populations in the United States, including statin resistant 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**

ETC-1002 is a small molecule drug that is synthesized with 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 ETC-1002, if approved.

### **Licenses**

In April 2008, we entered into an agreement with Pfizer pursuant to which we acquired a worldwide, exclusive, fully paid-up license from Pfizer to certain patent rights owned or controlled by Pfizer relating to ETC-1002, 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 ETC-1002. The license to us covers the development, manufacture and commercialization of ETC-1002. We may grant sublicenses under the license. Under the license agreement, Pfizer is restricted from making, using, developing or testing any of the compounds claimed under the same patents that claim or cover the composition of matter of ETC-1002. Neither party is entitled to any royalties, milestones or any similar development or commercialization payments under the license agreement, 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 ETC-1002 and our other development programs.

As of December 31, 2013, our patent estate, including patents we own or license from third parties, on a worldwide basis, included approximately 16 issued United States patents and 7 pending United States patent applications and 6 issued patents and 25 pending patent applications in other foreign jurisdictions. Of our worldwide patents and pending applications, only a subset relates to our small molecule program which includes our lead product candidate, ETC-1002. ETC-1002 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 5 years. U.S. Patent No. 8,497,301 claims a method of treatment using ETC-1002. There are currently three issued patents and four pending applications in countries outside the United States that relate to ETC-1002.

A second subset of this portfolio relates to our early-stage product candidate ESP41091. ESP41091 is claimed in U.S. Patent Nos. 7,119,221 and 7,405,226. Various methods of treatment using ESP41091 are claimed in U.S. Patent Nos. 8,153,690 and 8,309,604 and in two pending application in the United States. There are currently two issued patents and four pending applications in countries outside the United States that relate to ESP41091.

We hold an exclusive, worldwide, fully paid-up license from Pfizer to some of these patents and patent applications. This license is described above.

A subset of our worldwide patents and pending patent applications relates to our third drug candidate Apolipoprotein A1-4WF. Apolipoprotein A1-4WF is claimed in United States Patent No. 8,143,224. United States Patent No. 8,143,224 is scheduled to remain in force until its expiration on July 12, 2030. In addition, various methods of treatment using Apolipoprotein A1-4WF are claimed in United States Patent Application Publication No. 2012/0264677. We have rights to 20 issued patents and pending patent applications in the United States and other countries outside the United States that relate to Apolipoprotein A1-4WF and its use in various methods of treatment.

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 ETC-1002 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 ETC-1002—Our market is subject to intense competition. If we are unable to compete effectively, our opportunity to generate revenue from the sale of ETC-1002, if approved, will be materially adversely affected," and elsewhere in this prospectus for more information regarding competitors and competitive products.

## **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 ETC-1002, must be approved by the FDA through the new drug application, or 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 non-clinical 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 non-clinical, also referred to as pre-clinical, testing stage. Non-clinical 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 non-clinical 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 trial, the parameters to be used in monitoring safety and the effectiveness criteria to be evaluated if the initial clinical trial lends itself to an efficacy evaluation. Some non-clinical 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 trial 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 trial 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 trial before it commences at any institution. An IRB considers, among other things, whether the risks to individuals participating in the clinical trial are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the information regarding the clinical trial and the consent form that must be provided to each clinical trial subject or his or her legal representative and must monitor the clinical trial 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 trial, 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 1.* 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 trial 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 trial 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 trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the drug has been associated with unexpected serious harm to patients.

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, non-clinical 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, 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 diseases 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 Phase 4 testing 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 non-clinical 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 trial in accordance with an FDA-issued "Written Request" for such a clinical trial.

#### *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, 2013, we had 16 full-time employees and one part-time employee. Two of our employees have Ph.D. degrees. Nine of our employees are engaged in research and development activities. None of our employees is represented by labor unions or covered by collective bargaining agreements. We consider our relationship with our employees to be good.

### **Facilities**

We lease our facility, which is located at 46701 Commerce Center Drive, Plymouth, Michigan and consists of approximately 2,083 square feet of office and 4,867 square feet of laboratory space. In August 2013, we entered into the second amendment to the operating lease agreement which extended the expiration date of the initial term from October 2, 2013 to April 30, 2014. In February 2014, we entered into a lease for our offices to be located in the Valley Ranch Business Park at 3891 Ranchero Drive, Suite 150, Ann Arbor, Michigan consisting of approximately 7,941 rentable square feet of office space. The term of the lease ends 63 months after the commencement date which we expect to begin in April 2014. We believe our current and future facilities are sufficient to meet our needs until each respective lease expiration.

### **Legal Proceedings**

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



## **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 ETC-1002**

***We depend almost entirely on the success of one product candidate, ETC-1002, which is still in Phase 2 clinical development. We cannot be certain that we will be able to obtain regulatory approval for, or successfully commercialize, ETC-1002.***

We currently have only one product candidate, ETC-1002, in clinical development, and our business depends almost entirely on its 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. ETC-1002, which is currently in Phase 2 clinical studies, will require substantial additional clinical development, testing, and regulatory approvals before we are permitted to commence its commercialization. Our other product candidates are still in pre-clinical development stages. None of our product candidates have advanced into a pivotal study, and it may be years before such studies are initiated, if ever. 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 United States 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 pre-clinical 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 may include post-marketing studies and surveillance, including a Risk Evaluation and Mitigation Strategy, or REMS program, which will require the expenditure of substantial resources beyond the proceeds we raised in our initial public offering. Of the large number of drugs in development in the United States, only a small percentage successfully complete the FDA or any other foreign regulatory approval process 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 ETC-1002 or any other of our product candidates will be successfully developed or commercialized.

We are not permitted to market ETC-1002 in the United States until we receive approval of a New Drug Application, or NDA, from the FDA, or in any foreign countries until we receive the requisite approval from such countries. As a condition to submitting an NDA to the FDA for ETC-1002 to treat patients with hypercholesterolemia, we currently expect to complete two Phase 2b clinical studies, two pivotal Phase 3 clinical studies and one long-term safety study. We commenced our

first Phase 2b clinical study in October 2013 and we expect to initiate our second Phase 2b clinical study in March 2014. We have not commenced any of the Phase 3 clinical studies. Obtaining approval of an NDA is a complex, lengthy, expensive and uncertain process, and the FDA may delay, limit or deny approval of ETC-1002 for many reasons, including, among others:

- we may not be able to demonstrate that ETC-1002 is safe and effective in treating hypercholesterolemia to the satisfaction of the FDA;
- the results of our clinical studies may not meet the level of statistical or clinical significance required by the FDA for marketing approval;
- the FDA may disagree with the number, design, size, conduct or implementation of our clinical studies;
- the FDA may require that we conduct additional clinical studies, such as a cardiovascular outcomes trial;
- the FDA may not release its partial clinical hold on ETC-1002 to permit us to conduct a clinical study for more than six months;
- the FDA or an applicable foreign regulatory agency may not approve the formulation, specifications or labeling of ETC-1002;
- 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 may find the data from pre-clinical studies and clinical studies insufficient to demonstrate that ETC-1002's clinical and other benefits outweigh its safety risks;
- the FDA may disagree with our interpretation of data from our pre-clinical studies and clinical studies;
- the FDA may not accept data generated at our clinical study sites;
- if our NDA, if and when submitted, is 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 application or may recommend that the FDA require, as a condition of approval, additional pre-clinical studies or clinical studies, limitations in approved labeling or distribution and use restrictions;
- the FDA may require the development of a REMS as a condition of approval or post-approval;
- the FDA or the applicable foreign regulatory agency may not approve the manufacturing processes or facilities of third-party manufacturers with which we contract; or
- the FDA may change its approval policies or adopt new regulations.

Any of these factors, many of which are beyond our control, could jeopardize our ability to obtain regulatory approval for and successfully market ETC-1002. Moreover, because our business is almost entirely dependent upon this one product candidate, any setback in our pursuit of its regulatory approval would have a material adverse effect on our business and prospects.

***Failures or delays in the commencement or completion of our Phase 2b or pivotal Phase 3 clinical studies of ETC-1002 could result in increased costs to us and could delay, prevent or limit our ability to generate revenue and continue our business.***

We initiated our first Phase 2b clinical study in October 2013 and we expect to initiate our second Phase 2b clinical study in March 2014. We have not commenced our pivotal Phase 3 clinical studies. Successful completion of such clinical studies is a prerequisite to submitting an NDA to the FDA and,

consequently, the ultimate approval and commercial marketing of ETC-1002. We do not know whether our Phase 2b clinical studies will be completed on schedule, if at all, or whether our pivotal Phase 3 clinical studies will begin or be completed on schedule, if at all, as the commencement and completion of clinical studies can be delayed or prevented for a number of reasons, including, among others:

- the FDA may deny permission to proceed with Phase 3 clinical trials, including not releasing its partial clinical hold on ETC-1002 to permit us to conduct a clinical study for more than six months, or 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 a cardiovascular outcomes study, if one were to be required, 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 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 previously identified in our completed clinical studies;
- reports from pre-clinical 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 IRBs at the sites where the IRBs are overseeing a clinical study, a data safety monitoring board, or DSMB, overseeing the clinical study at issue or 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 or 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, including any that could be identified in our ongoing pre-clinical carcinogenicity studies, adverse side effects or lack of effectiveness;
- 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 Phase 1 and Phase 2a clinical studies of ETC-1002 are not necessarily predictive of the results of our Phase 2b and planned Phase 3 clinical studies of ETC-1002. If we cannot replicate the positive results from our Phase 1 and Phase 2a clinical studies of ETC-1002 in our Phase 2b and Phase 3 clinical studies, we may be unable to successfully develop, obtain regulatory approval for and commercialize ETC-1002.***

Even if we are able to complete our Phase 2b and planned pivotal Phase 3 clinical studies of ETC-1002 according to our current development timeline, the positive results from our Phase 1 and Phase 2a clinical studies of ETC-1002 may not be replicated in our Phase 2b or pivotal Phase 3 clinical study results. 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, pre-clinical findings made while clinical studies were underway or safety or efficacy observations made in clinical studies, including previously unreported adverse events. Our Phase 2b clinical studies are evaluating the safety and efficacy of ETC-1002 in statin-intolerant patients and as an add-on to existing statin treatments. We expect that our Phase 3 clinical studies will evaluate the safety and efficacy of ETC-1002 in these same patient populations. Nevertheless, the results from our Phase 2a clinical studies for ETC-1002, including ETC-1002-006 and ETC-1002-007, may not be predictive of the results we may obtain in our Phase 2b or Phase 3 clinical studies of ETC-1002. Moreover, pre-clinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that believed their product candidates performed satisfactorily in pre-clinical studies and clinical studies nonetheless failed to obtain FDA approval. If we fail to obtain positive results in our Phase 2b and Phase 3 clinical studies of ETC-1002, 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 may need substantial additional capital in the future. If additional capital is not available, we will have to delay, reduce or cease operations.***

Although we believe that the net proceeds from our initial public offering will be sufficient to fund our operations through at least the end of 2015, we will likely need to raise additional capital thereafter to continue to fund the further development of ETC-1002 and our operations. We expect to announce top-line results from our latest currently anticipated Phase 2b clinical studies in the fourth quarter of 2014 and to have our end of Phase 2 meeting with the FDA in the first half of 2015. Our future capital requirements may be substantial and will depend on many factors including:

- the scope, size, rate of progress, results and costs of initiating and completing our Phase 2b clinical studies of ETC-1002 and our operating costs incurred as we conduct these studies and through our planned end of Phase 2 meeting with the FDA, for which we currently estimate that we will use substantially all of the net proceeds from our initial public offering;
- the scope, size, rate of progress, results and costs of initiating and completing our Phase 3 clinical program of ETC-1002, which currently includes two pivotal Phase 3 clinical studies and one long-term safety study;
- the cost, timing and outcome of our efforts to obtain marketing approval for ETC-1002 in the United States, including to fund the preparation and filing of an NDA with the FDA for ETC-1002 and to satisfy related FDA requirements;
- the number and characteristics of any additional product candidates we develop or acquire;
- the costs associated with commercializing ETC-1002 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 ETC-1002 or any future product candidates;

- the cost of manufacturing ETC-1002 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 ETC-1002 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 ETC-1002 or any future product candidate, or to commercialize ETC-1002 or any future product candidate, if approved, unless we find a partner.

***We are a development stage biopharmaceutical company with a limited operating history 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 are a development stage company with a limited operating history on which to base your investment decision. Biopharmaceutical 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 ETC-1002. 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 ETC-1002, which is currently in Phase 2 clinical development. We have funded our operations to date primarily through proceeds from sales of preferred stock, our initial public offering of common stock, which we closed in July 2013, convertible promissory notes and warrants and we have incurred losses in each year since our inception. Our net losses were \$26.1 million, \$11.7 million and \$10.8 million for the years ended December 31, 2013, 2012 and 2011, respectively. As of December 31, 2013, we had an accumulated deficit of \$68.1million. 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 our research and development expenses to significantly increase in connection with our additional clinical studies of ETC-1002 and development of any other product candidates we may choose to pursue. In addition, if we obtain marketing approval for ETC-1002, we will also incur significant sales, marketing and outsourced manufacturing expenses. As a newly public company, we have started to incur and will continue to incur additional costs associated with operating as a public 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 guidance or unanticipated events during our Phase 2b or Phase 3 clinical studies of ETC-1002 may occur, which may result in changes to clinical study protocols or additional clinical study requirements, such as the initiation or completion of a cardiovascular outcomes trial, which could result in increased costs to us and could delay our development timeline.***

Changes in regulatory requirements, FDA guidance or unanticipated events during our clinical studies may force us to amend clinical study protocols or the FDA may impose additional clinical study requirements. Amendments to our clinical study protocols would require resubmission to the FDA and IRBs for review and approval, which may adversely impact the cost, timing and/or successful completion of a clinical study. If we experience delays completing—or if we terminate—any of our Phase 2b or Phase 3 clinical studies, or if we are required to conduct additional clinical studies, such as a cardiovascular outcomes trial, the commercial prospects for ETC-1002 may be harmed and our ability to generate product revenue will be delayed. If the FDA requires us to conduct a cardiovascular outcomes trial, we may not be able to identify and enroll the requisite number of patients in that study. Even if we are successful in enrolling patients in a cardiovascular outcomes study, we may not ultimately be able to demonstrate that lowering LDL-C levels using ETC-1002 provides patients with an incremental lowering of cardiovascular disease risks and our failure to do so may delay or hinder our ability to obtain FDA approval for ETC-1002. Our current development timeline for ETC-1002 does not contemplate the completion of a cardiovascular outcomes trial prior to FDA approval. Any such study, if required, would be costly and time-consuming and, regardless of the outcome, would adversely affect our development timeline and financial condition.

***Even if we receive marketing approval for ETC-1002, we may still face future development and regulatory difficulties.***

Even if we receive marketing approval for ETC-1002, regulatory authorities may still impose significant restrictions on ETC-1002's indicated uses or marketing or impose ongoing requirements for potentially costly post-approval studies, such as a cardiovascular outcomes trial. ETC-1002 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.

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 ETC-1002, such as adverse events of unanticipated severity or frequency, or problems with the facility where ETC-1002 is manufactured, a regulatory agency may impose restrictions on ETC-1002, the manufacturer or us, including requiring withdrawal of ETC-1002 from the market or suspension of manufacturing. If we, ETC-1002 or the manufacturing facilities for ETC-1002 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 ETC-1002 in the United States, we may never receive regulatory approval to market ETC-1002 outside of the United States.***

We have not yet selected any markets outside of the United States where we intend to seek regulatory approval to market ETC-1002. In order to market any product outside of the United States, however, we must establish and comply with the numerous and varying efficacy, safety and other regulatory requirements of other countries. 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 United States as well as other risks. In particular, in many countries outside of the United States, 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 ETC-1002 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 ETC-1002, it may not achieve broad market acceptance, which would limit the revenue that we generate from its sales.***

The commercial success of ETC-1002, if approved by the FDA or other regulatory authorities, will depend upon the awareness and acceptance of ETC-1002 among the medical community, including physicians, patients and healthcare payors. Market acceptance of ETC-1002, if approved, will depend on a number of factors, including, among others:

- ETC-1002's demonstrated ability to treat statin intolerant patients with hypercholesterolemia and, if required by any applicable regulatory authority in connection with the approval for this or any other indication, to provide patients with incremental cardiovascular disease benefits, as compared with other available therapies;
- the relative convenience and ease of administration of ETC-1002, including as compared with other treatments for patients with hypercholesterolemia;
- the prevalence and severity of any adverse side effects such as muscle pain or weakness;
- limitations or warnings contained in the labeling approved for ETC-1002 by the FDA;
- availability of alternative treatments, including a number of competitive LDL-C lowering therapies already approved 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 ETC-1002 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 ETC-1002 is approved but does not achieve an adequate level of acceptance by patients, physicians and payors, we may not generate sufficient revenue from ETC-1002 to become or remain profitable. Before granting reimbursement approval, healthcare payors may require us to demonstrate that, in addition to lowering elevated LDL-C levels, ETC-1002 also provides incremental cardiovascular disease benefits to patients. Our efforts to educate the medical community and third-party payors about the benefits of ETC-1002 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 ETC-1002, 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 ETC-1002, 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 ETC-1002, 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 ETC-1002, if approved, our operating results and financial condition would be materially adversely affected.

***Guidelines and recommendations published by various organizations may adversely affect the use or commercial viability of ETC-1002, if approved.***

Government agencies issue regulations and guidelines directly applicable to us and to ETC-1002, 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 American Heart Association have made recommendations about therapies in the cardiovascular therapeutics market. Changes to these existing recommendations or other guidelines advocating alternative therapies could result in decreased use of ETC-1002, if approved, which would adversely affect our results of operations.

***Even if approved, reimbursement policies could limit our ability to sell ETC-1002.***

Market acceptance and sales of ETC-1002 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 ETC-1002 and, if reimbursement is available, the level of such reimbursement. Reimbursement may impact the demand for, or the price of, ETC-1002. If reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize ETC-1002.

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 ETC-1002 with other available therapies. If reimbursement for ETC-1002 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 product development programs for candidates other than ETC-1002 may require substantial financial resources and may ultimately be unsuccessful.***

In addition to the development of ETC-1002, we may pursue the development of our other two early-stage development programs. Neither of our other potential product candidates has 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 our other two early-stage development programs may adversely affect our ability to continue development and commercialization of ETC-1002, 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 ETC-1002 than some other pharmaceutical products because a significant portion of the target patient population for ETC-1002 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 ETC-1002, 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 ETC-1002 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 ETC-1002, 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 ETC-1002, 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 ETC-1002 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 ETC-1002 as a therapy for lowering LDL-C levels in statin intolerant patients with hypercholesterolemia, the first indication we intend to pursue, physicians may nevertheless prescribe ETC-1002 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 ETC-1002, 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 ETC-1002, if approved, will be materially adversely affected.***

The LDL-C lowering therapies market is highly competitive and dynamic and dominated by the sale of statin treatments, including the cheaper generic versions of statins. We estimate that the total statin monotherapy and fixed combination market, including generic drugs, accounted for 69% of U.S. sales in the LDL-C lowering market in 2012. Our success will depend, in part, on our ability to obtain a share of the market, initially, for patients who are 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 statin intolerant patients that compete with ETC-1002, 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.

Low-density lipoprotein cholesterol (LDL-C) lowering therapies currently on the market that would compete with ETC-1002 include the following:

- Statins, such as Crestor® (rosuvastatin) and Lipitor® (atorvastatin), including their cheaper generic versions;
- Cholesterol absorption inhibitors, such as Zetia® (ezetimibe), a monotherapy marketed by Merck & Co.,
- Bile acid sequestrants such as Welchol® (colesevelam), marketed by Daiichi Sankyo Inc.;
- MTP inhibitors, such as JUXTAPID® (lomitapide), marketed by Aegerion Pharmaceuticals, Inc.;
- Apo B Anti-Sense therapy, such as KYNAMRO® (mipomersen), marketed by Genzyme Corp. a Sanofi company;
- Combination therapies, such as Vytorin® (ezetimibe and simvastatin) and Liptruzet® (ezetimibe and atorvastatin), marketed by Merck & Co., Inc.; and
- Other lipid-lowering monotherapies (including cheaper generic versions), such as Tricor® (fenofibrate) and Niaspan® (niacin extended release), and combination therapies, such as Advicor® (niacin extended release and lovastatin) and Simcor® (niacin and simvastatin), 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 United States or outside of the United States. Based on publicly available information, we believe the current therapies in development that would compete with ETC-1002 include:

- PCSK9 inhibitors, such as alirocumab, a therapy in Phase 3 clinical testing being developed by Sanofi and Regeneron Pharmaceuticals, Inc., evolocumab, a separate therapy in Phase 3 clinical testing being developed by Amgen Inc. and bococizumab, a separate therapy in Phase 3 clinical testing being developed by Pfizer Inc., and four additional PCSK9 inhibitors in earlier phases of development from Lilly, Novartis, Roche and The Medicines Company/Alnylam ; and

- CETP inhibitors, such as anacetrapib, a therapy in Phase 3 clinical testing being developed by Merck, and evacetrapib, a therapy in Phase 3 clinical testing being developed by Eli Lilly & Company.

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 ETC-1002, if approved, and may render ETC-1002 obsolete or non-competitive before we can recover the expenses of developing and commercializing it. If approved, ETC-1002 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 ETC-1002 in clinical studies and the sale of ETC-1002, 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 ETC-1002. 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 ETC-1002 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 ETC-1002 or any future product candidates, if approved.

We maintain product liability insurance coverage for our clinical studies with a \$5 million annual aggregate coverage limit. 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

ETC-1002, 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 ETC-1002, 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 ETC-1002, 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 ETC-1002 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 ETC-1002 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 ETC-1002 could be delayed.

### **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 ETC-1002, 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, 2013, Esperion's patent estate, including patents we own or license from third parties, on a worldwide basis, included approximately 16 issued United States patents and 7 pending United States patent applications, 1 international patent application and 6 issued patents and 25 pending patent applications in other foreign jurisdictions. Of our worldwide patents and pending applications, only a subset relates to our small molecule program which includes our lead product candidate, ETC-1002. ETC-1002 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 5 years. U.S. Patent No. 8,497,301 claims a method of treatment using ETC-1002. We also have a pending U.S. patent application claiming methods of treatment using

ETC-1002. There are currently three issued patents and four pending applications in countries outside the United States that relate to ETC-1002.

A second subset of this portfolio relates to our early-stage product candidate ESP41091. ESP41091 is claimed in U.S. Patent Nos. 7,119,221 and 7,405,226. Various methods of treatment using ESP41091 are claimed in U.S. Patent Nos. 8,153,690 and 8,309,604 and in two pending applications in the United States. There are currently two issued patents and four pending applications in countries outside the United States that relate to ESP41091.

Our 4WF patent portfolio currently consists of 20 issued patents and pending patent applications in the United States and other foreign jurisdictions regarding apolipoprotein mixtures, dimeric oxidation-resistant apolipoprotein variants and oxidant resistant apolipoprotein A1 variants and mimetic peptides thereof.

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 ETC-1002 or our 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 ETC-1002.

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 ETC-1002, 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 ETC-1002, 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 ETC-1002;
- any of our pending patent applications will result in issued patents;
- we will be able to successfully commercialize ETC-1002, 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 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 to our lead product candidate 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 ETC-1002, 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 ETC-1002 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 ETC-1002.

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 ETC-1002;
- 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, ETC-1002 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 recently 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 United States Patent and Trademark Office, or 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 ETC-1002 or our 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 biopharmaceuticals, 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 ETC-1002, 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 will rely on CROs to conduct our Phase 2b and Phase 3 clinical studies for ETC-1002. 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 ETC-1002 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 ETC-1002 and preclude our ability to commercialize ETC-1002, 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 ETC-1002, and we intend to rely on third parties to produce commercial supplies of ETC-1002 and pre-clinical, 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 ETC-1002, or any future product candidates, for use in the conduct of our pre-clinical studies and clinical studies, and we lack the internal resources and the capability to manufacture any product candidates on a clinical or commercial scale. The facilities used by our contract manufacturers to manufacture the active pharmaceutical ingredient and final drug for ETC-1002, 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 ETC-1002.***

Our drug development programs and commercialization plans for ETC-1002 will require substantial additional cash to fund expenses. We may develop and initially commercialize ETC-1002 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 ETC-1002 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 ETC-1002 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 ETC-1002 could be delayed or terminated.***

We are not currently party to any collaborative arrangements for the commercialization of ETC-1002 or similar arrangements, although we may pursue such arrangements before any commercialization of ETC-1002 outside of the United States or to further commercialize ETC-1002 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 ETC-1002 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 ETC-1002 could be delayed, curtailed or terminated because we may not have sufficient financial resources or capabilities to continue commercialization of ETC-1002 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 ETC-1002 and, as a result, could delay or otherwise negatively affect the commercialization of ETC-1002 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 ETC-1002 for any of these reasons, our sales of ETC-1002, 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.*

In connection with being a relatively new public company, we expect that we will continue to increase our number of employees 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 ETC-1002. 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 ETC-1002, 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 both our founder, Executive Chairman and Chief Scientific Officer and our President and Chief Executive Officer, and to attract, retain and motivate qualified personnel.*

We are highly dependent on Dr. Roger S. Newton, our founder, Executive Chairman and Chief Scientific Officer, and Tim M. Mayleben, our President and Chief Executive Officer. We have entered into employment agreements with Dr. Newton and Mr. Mayleben, 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 either Dr. Newton or Mr. Mayleben in the foreseeable future, the loss of the services of either individual 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 public 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 adopted in connection with our initial public offering. 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 ETC-1002 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 our lead product candidate, ETC-1002, 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, ETC-1002. Our ability to generate revenue depends on a number of factors, including, but not limited to, our ability to:

- initiate and successfully complete our Phase 2b clinical studies that meet their clinical endpoints;
- initiate and successfully complete our Phase 3 clinical program;
- initiate and successfully complete all safety studies required to obtain U.S. and foreign marketing approval for ETC-1002 as a treatment for patients with hypercholesterolemia;
- commercialize ETC-1002, if approved, by developing a sales force or entering into collaborations with third parties; and
- achieve market acceptance of ETC-1002 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 ETC-1002. Even if we initiate and successfully complete our Phase 3 clinical program of ETC-1002, which includes two pivotal Phase 3 clinical studies and one long-term safety study, which each meet their clinical endpoints and ETC-1002 is approved for commercial sale, and despite expending these costs, ETC-1002 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, 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 and 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 ETC-1002, 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 carry forwards may be subject to limitation.***

Under Section 382 of the Internal Revenue Code of 1986, as amended, or the Code, changes in our ownership may limit the amount of our net operating loss carryforwards that could be utilized annually to offset our future taxable income, if any. This limitation would generally apply in the event of a cumulative change in ownership of our company of more than 50% within a three-year period. Any such limitation may significantly reduce our ability to utilize our net operating loss carryforwards before they expire. The closing of our initial public offering, together with private placements and other transactions that have occurred since our inception, may trigger such an ownership change pursuant to Section 382. Any such limitation, whether as the result of our initial public offering, prior private placements, sales of our common stock by our existing stockholders or additional sales of our common stock by us after our initial public offering, could have a material adverse effect on our results of operations in future years. We have not completed a study to assess whether an ownership change for purposes of Section 382 has occurred, or whether there have been multiple ownership changes since our inception, due to the significant costs and complexities associated with such study.

*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 initial and 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. Beginning with the second annual report that we will be required to file with the SEC, Section 404 requires an annual management assessment of the effectiveness of our internal control over financial reporting. However, for so long as we remain an “emerging growth company” as defined in the JOBS Act, we intend to take advantage of certain exemptions from various reporting requirements that are applicable to public companies that are not emerging growth companies, including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404. Once we are no longer an “emerging growth company” or, if before such date, we opt to no longer take advantage of the applicable exemption, we will be required to include an opinion from our independent registered public accounting firm on the effectiveness of our internal control over financial reporting.

We are in the early stages of 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**

*Market volatility may affect our stock price and the value of your investment.*

The market price of our common stock may fluctuate significantly in response to a number of factors, most of which we cannot control, including, among others:

- plans for, progress of or results from clinical efficacy or safety studies of ETC-1002;



- the failure of the FDA to approve ETC-1002;
- announcements of new products, technologies, commercial relationships, acquisitions or other events by us or our competitors;
- the success or failure of other LDL-C lowering therapies;
- regulatory or legal developments in the United States and other countries;
- failure of ETC-1002, if approved, to achieve commercial success;
- fluctuations in stock market prices and trading volumes of similar companies;
- general market conditions and overall fluctuations in U.S. equity markets;
- variations in our quarterly operating results;
- changes in our financial guidance or securities analysts' estimates of our financial performance;
- changes in accounting principles;
- our ability to raise additional capital and the terms on which we can raise it;
- sales of large blocks of our common stock, including sales by our executive officers, directors and significant stockholders;
- additions or departures of key personnel;
- discussion of us or our stock price by the press and by online investor communities; and
- other risks and uncertainties described in these risk factors.

As a result, you may not be able to sell your shares of common stock at or above the price at which you purchase them.

***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. If we were to be sued, it 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.

***We are an "emerging growth company," and as a result of the reduced disclosure and governance requirements applicable to emerging growth companies, our common stock may be less attractive to investors.***

We are an "emerging growth company," as defined in the JOBS Act, and we intend to take advantage of certain exemptions from various reporting requirements that are applicable to other

public companies that are not emerging growth companies including, but not limited to, not being required to comply with the auditor attestation requirements of Section 404, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements, and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We cannot predict if investors will find our common stock less attractive because we will rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile. We may take advantage of these reporting exemptions until we are no longer an “emerging growth company.” We will remain an “emerging growth company” until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of our initial public offering, (b) in which we have total annual gross revenue of at least \$1.0 billion, or (c) in which we are deemed to be a large accelerated filer, which means the market value of our common stock that is held by non-affiliates exceeds \$700 million as of the prior June 30th, and (2) the date on which we have issued more than \$1.0 billion in non-convertible debt during the prior three-year period.

***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.

***Future sales of our common stock may cause our stock price to decline.***

Sales of a substantial number of shares of our common stock in the public market or the perception that these sales might occur could significantly reduce the market price of our common stock and impair our ability to raise adequate capital through the sale of additional equity securities.

**Item 1B. Unresolved Staff Comments**

None.

**Item 2. Properties**

Our corporate headquarters and clinical development operations are located in Plymouth, Michigan where we lease and occupy approximately 2,083 square feet of office and 4,867 square feet of laboratory space. In August 2013, we entered into the second amendment to the operating lease agreement which extended the expiration date of the initial term from October 2, 2013 to April 30, 2014. On February 4, 2014, we entered into a lease for 7,941 square feet of office space at 3891 Rancho Drive, Suite 150, Ann Arbor, Michigan 48108. We anticipate moving into this space in April 2014, at which point our principal office space will be relocated there. This lease expires 63 months after its commencement date. We believe our current and new facility will be sufficient to meet our needs until expiration.

**Item 3. Legal Proceedings**

We are not a party to any legal proceedings and we are not aware of any claims or actions pending or threatened against us. In the future, we might from time to time become involved in litigation relating to claims arising from our ordinary course of business.

**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, 2013, the closing price for our common stock as reported on the NASDAQ Global Market was \$13.74. 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, 2013</u>	<u>High</u>	<u>Low</u>
Second Quarter (from June 26, 2013) . . . . .	\$17.40	\$13.65
Third Quarter . . . . .	\$20.10	\$13.55
Fourth Quarter . . . . .	\$19.30	\$10.90

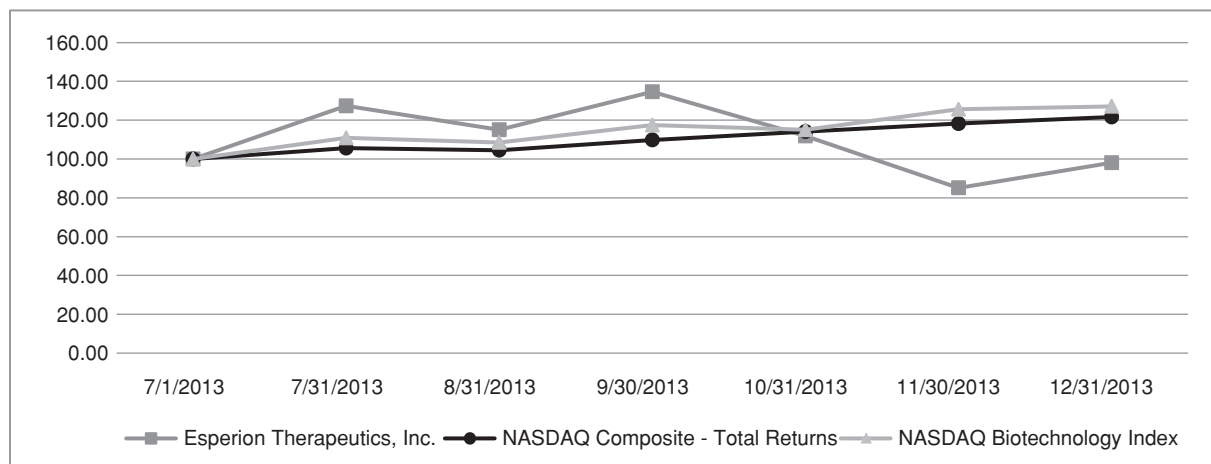
#### Stockholders

As of March 1, 2014, there were 20 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 July 1, 2013, which is the date our initial public offering, to two indices: the NASDAQ Composite Index and the NASDAQ Biotechnology Index. The graph assumes an initial investment of \$100 on July 1, 2013, 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 6 Month Cumulative Total Return\***  
**Among Esperion Therapeutics, Inc., the NASDAQ Composite Index and**  
**the NASDAQ Biotechnology Index**



\* \$100 invested on July 1, 2013 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.

## 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.



## **Recent Sales of Unregistered Securities**

Set forth below is information regarding securities sold by us during the year ended December 31, 2013 that were not registered under the Securities Act. Also included is the consideration, if any, received by us for the securities and information relating to the section of the Securities Act, or rule of the Securities and Exchange Commission, under which exemption from registration was claimed.

### *Issuances of securities*

On April 19, 2013, in connection with a preferred stock financing, we issued 17,000,000 shares of our Series A preferred stock to ten accredited investors at a per share purchase price of \$1.00 for aggregate gross consideration of \$17.0 million. Upon the completion of our initial public offering, the Series A preferred stock was converted into shares of common stock.

On April 28, 2008, we issued a convertible promissory note to an accredited investor in the original principal amount of \$5.0 million. The convertible promissory note accrued interest at a rate of 8.931% per year and had a maturity date of April 28, 2018. Accrued interest under the note was capitalized on June 30th and December 31st of each year. On May 29, 2013, we entered into a stock purchase agreement pursuant to which we issued 6,750,000 shares of our Series A-1 preferred stock to the noteholder at a price of \$1.1560 per share, which purchase price was paid through the cancellation of all outstanding indebtedness, including accrued interest, under the convertible promissory note. Upon the completion of our initial public offering, the Series A-1 preferred stock was converted into shares of common stock.

No underwriters were involved in the foregoing sales of securities. The securities described above were issued and sold in reliance on the exemptions from registration provided by Section 4(2) of the Securities Act and/or Rule 506 of Regulation D promulgated under the Securities Act. Each of the purchasers in these transactions represented to us in connection with its purchase that it was acquiring the securities for investment and not for distribution and that it could bear the risks of the investment. Each purchaser received written disclosures that the securities had not been registered under the Securities Act and that any resale must be made pursuant to a registration statement or an available exemption from registration. All of the foregoing securities are deemed restricted securities for the purposes of the Securities Act.

### *Stock option and other equity awards*

During the year ended December 31, 2013, we granted stock options to purchase an aggregate of 1,251,749 shares of common stock with a weighted exercise price of \$10.62 per share pursuant to our 2008 Stock Option and Incentive Plan and our 2013 Stock Option and Incentive Plan to our employees, consultants and non-employee directors.

The issuances of such options were exempt either pursuant to Rule 701 under the Securities Act, as a transaction pursuant to a compensatory benefit plan, or pursuant to Section 4(2) of the Securities Act, as a transaction by an issuer not involving a public offering.

## **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.

## **Use of Proceeds from Registered Securities**

On July 1, 2013, we closed the sale of 5,000,000 shares of common stock to the public, or the IPO, at an initial public offering price of \$14.00 per share. On July 11, 2013, the underwriters exercised their over-allotment option in full, pursuant to which we sold an additional 750,000 shares of common stock

at a price of \$14.00 per share. The offer and sale of the shares in the IPO was registered under the Securities Act pursuant to registration statements on Form S-1 (File No. 333-188595), which was filed with the SEC on May 14, 2013 and amended subsequently and declared effective on June 25, 2013, and Form S-1MEF (File No. 333-189590), which was filed with the SEC on June 25, 2013 and declared effective on June 25, 2013. Following the sale of the shares in connection with the closing of our IPO, the offering terminated. The offering did not terminate before all the securities registered in the registration statements were sold. Credit Suisse Securities (USA) LLC and Citigroup Global Markets Inc. acted as joint book-running managers for the offering and as representatives of the underwriters. JPM Securities LLC and Stifel, Nicolaus & Company, Incorporated acted as co-managers for the offering.

We raised approximately \$72.2 million in net proceeds after deducting underwriting discounts and commissions of approximately \$5.6 million and other offering expenses of approximately \$2.7 million. No offering expenses were paid directly or indirectly to any of our directors or officers (or their associates) or persons owning ten percent or more of any class of our equity securities or to any other affiliates.

To date, we have not yet used the net proceeds from our IPO. We invested the funds received in cash equivalents and other short-term and long-term investments in accordance with our investment policy. As described in our final prospectus filed with the SEC on June 26, 2013 pursuant to Rule 424(b) under the Securities Act, we expect to use the net proceeds from our IPO to fund the clinical development of ETC-1002 through the completion of our Phase 2b clinical studies and end of Phase 2 meeting with the FDA, as well as for working capital and general corporate purposes, including funding the costs of operating as a public company. We currently expect to have our end of Phase 2 meeting with the FDA in the first half of 2015.

**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 consolidated financial statements and may not be indicative of future operating results. The following selected consolidated financial data should be read in conjunction with Item 7, “Management’s Discussion and Analysis of Financial Condition and Results of Operations” and the consolidated financial statements and the notes thereto included elsewhere in this report. The selected financial data in this section are not intended to replace our consolidated 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,			Period From January 22, 2008 (Inception) through December 31, 2013
	2013	2012	2011	2013	2012	2011	
	(in thousands, except share and per share data)						
Grant income . . . . .	\$ —	\$ —	\$ —	\$ —	\$ —	\$ —	\$ 244
Operating expenses:							
Research and development . . . . .	7,339	1,654	1,898	16,014	7,998	7,807	43,428
General and administrative . . . . .	2,397	506	788	6,745	2,206	2,357	18,194
Acquired in-process research and development . . . . .	—	—	—	—	—	—	86
Total operating expenses . . . . .	9,736	2,160	2,686	22,759	10,204	10,164	61,708
Loss from operations . . . . .	\$ (9,736)	\$ (2,160)	\$ (2,686)	\$ (22,759)	\$ (10,204)	\$ (10,164)	\$ (61,464)
Total other income (expense) . . . . .	47	(615)	(158)	(3,329)	(1,538)	(653)	(6,599)
Net loss . . . . .	\$ (9,689)	\$ (2,775)	\$ (2,844)	\$ (26,088)	\$ (11,742)	\$ (10,817)	\$ (68,063)
Net loss per common share (basic and diluted) . . . . .	\$ (0.63)	\$ (8.12)	\$ (9.30)	\$ (3.31)	\$ (36.31)	\$ (36.22)	
Weighted average shares outstanding (basic and diluted) . . . . .	15,340,713	341,935	305,658	7,885,921	323,382	298,689	

The table below presents a summary of our balance sheet data as of December 31, 2013 and 2012:

	As of December 31,	
	2013	2012
	(in thousands)	
<b>Balance Sheet Data:</b>		
Cash and cash equivalents . . . . .	\$ 56,537	\$ 6,512
Working capital (deficit) . . . . .	56,417	(10,035)
Investments . . . . .	21,063	—
Total assets . . . . .	78,294	7,312
Total convertible short-term debt . . . . .	—	15,241
Total convertible long-term debt . . . . .	—	7,529
Convertible preferred stock warrant liability . . . . .	—	265
Convertible preferred stock . . . . .	—	23,975
Common stock . . . . .	15	—
Deficit accumulated during the development stage . . . . .	(68,063)	(41,975)
Total stockholders’ equity (deficit) . . . . .	74,091	(41,365)

## **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 consolidated 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 a clinical stage biopharmaceutical company focused on developing and commercializing first-in-class, oral, low-density lipoprotein cholesterol (LDL-C) lowering therapies for the treatment of patients with hypercholesterolemia and other cardiometabolic risk markers. ETC-1002, our lead product candidate, is a unique, first in class, orally available, once-daily small molecule designed to lower LDL-C levels and avoid the side effects associated with LDL-C lowering therapies currently available. ETC-1002 is being developed primarily for patients intolerant of statins with elevated levels of LDL-C. Phase 2b clinical trials for ETC-1002 are currently underway and build upon a successful and comprehensive Phase 1 and Phase 2 program. We own the exclusive worldwide rights to ETC-1002 and our other product candidates.

We were incorporated in Delaware in January 2008 and commenced our operations in April 2008. Since our inception, we have devoted substantially all of our resources to developing ETC-1002 and our other product candidates, business planning, raising capital and providing general and administrative support for these operations. We have funded our operations primarily through the issuance of preferred stock, our initial public offering of common stock, which we closed in July 2013, convertible promissory notes and warrants to purchase shares of preferred stock.

On July 1, 2013, we completed the initial public offering, or IPO, of our common stock pursuant to a registration statement on Form S-1. In the IPO, we sold an aggregate of 5,000,000 shares of common stock under the registration statement at a public offering price of \$14.00 per share. Net proceeds from the IPO were approximately \$62.7 million, after deducting underwriting discounts and commissions and offering expenses. Upon the closing of the IPO, all outstanding shares of our preferred stock were converted into 9,210,999 shares of common stock. Additionally, as part of the IPO, we granted the underwriters a 30-day option to purchase up to 750,000 additional shares of common stock at the IPO price to cover over-allotments, if any. On July 11, 2013, the underwriters exercised this option in full. As a result of this exercise, we received an additional \$9.5 million in proceeds, net of underwriting discounts and commissions and offering expenses.

We are a development stage company and do not have any products approved for sale. To date, we have not generated any revenue. We have never been profitable and, from inception to December 31, 2013, our losses from operations have been \$61.5 million. Our net losses were \$26.1 million, \$11.7 million and \$10.8 million for the years ended December 31, 2013, 2012 and 2011, 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 expenses and increasing operating losses for the foreseeable future. We expect our expenses to increase in connection with our ongoing activities, including, among others:

- conducting additional clinical studies of ETC-1002 to complete its development;
- seeking regulatory approval for ETC-1002;

- commercializing ETC-1002; 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***

ETC-1002, our lead product candidate, is a novel, first in class, orally available, once-daily LDL-C lowering small molecule therapy designed to target known lipid and carbohydrate metabolic pathways to lower levels of LDL-C and to avoid side effects associated with existing LDL-C lowering therapies. We acquired the rights to ETC-1002 from Pfizer in 2008. We own the exclusive worldwide rights to ETC-1002 and we are not obligated to make any royalty or milestone payments to Pfizer.

In 2011, we incurred \$4.6 million in expenses related to our Phase 1b multiple dose tolerance clinical trial (ETC-1002-004), our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia (ETC-1002-003) which reported top-line results in March 2012, and our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia and Type 2 diabetes (ETC-1002-005) which reported top-line results in January 2013.

In 2012, we incurred \$5.8 million in expenses related to our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia and Type 2 diabetes (ETC-1002-005) and our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia and a history of statin intolerance (ETC-1002-006) which reported top-line results in June 2013, and our phase 2a clinical study in patients with hypercholesterolemia taking 10 mg of atorvastatin (ETC-1002-007) which reported top-line results in September 2013.

During the year ended December 31, 2013, we incurred \$13.7 million in expenses related to our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia and Type 2 diabetes (ETC-1002-005), our Phase 2a proof-of-concept clinical study in patients with hypercholesterolemia and a history of statin intolerance (ETC-1002-006), our Phase 2a clinical study in patients with hypercholesterolemia taking 10 mg of atorvastatin (ETC-1002-007) and our Phase 2b clinical study in patients with hypercholesterolemia and either with or without statin intolerance (ETC-1002-008).

We also have two other early-stage programs in pre-clinical development. We licensed one of these candidates from The Cleveland Clinic Foundation, or CCF, and are obligated to make certain royalty and milestone payments (consisting of cash and common stock) to CCF, including a minimum annual cash payment of \$50,000 during years when a milestone payment is not met. No milestone or royalty payments will be due to any third-party in connection with the development and commercialization of our other pre-clinical product candidate, ESP41091.

### **Financial Operations Overview**

#### ***Revenue***

To date, we have not generated any revenue, other than grant income. In the future, we may never generate revenue from the sale of ETC-1002 or our other product candidates. If we fail to complete the development of ETC-1002 or our 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 preclinical and clinical studies. Our research and development expenses consist primarily of costs incurred in connection with the development of ETC-1002, which include:

- expenses incurred under agreements with consultants, contract research organizations, or CROs, and investigative sites that conduct our pre-clinical and clinical studies;
- the cost of acquiring, developing and manufacturing clinical study materials;
- 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 ETC-1002. 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.

Our research and development expenses are expected to increase in the foreseeable future. Costs associated with ETC-1002 will increase as we continue to conduct our Phase 2b clinical studies and initiate our Phase 3 clinical studies. We cannot determine with certainty the duration and completion costs associated with the ongoing or future clinical studies of ETC-1002. Also, we cannot conclude with certainty if, or when, we will generate revenue from the commercialization and sale of ETC-1002 or our other product candidates for which we obtain regulatory approval, if ever. We may never succeed in obtaining regulatory approval for any of our product candidates, including ETC-1002. The duration, costs and timing associated with the development and commercialization of ETC-1002 and our other product candidates 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 ETC-1002, 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 ETC-1002.

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

General and administrative expenses primarily consist of salaries and related costs for personnel, including stock-based compensation and travel expenses, 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 ETC-1002, increases in our headcount, expansion of our information technology infrastructure, increased legal, compliance, accounting and investor and public relations expenses associated with being a public company.

### ***Interest Expense***

Interest expense consists primarily of non-cash interest costs associated with our convertible promissory notes.

### **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, or U.S. GAAP. 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 trials 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 trials on our behalf. We generally accrue expenses related to clinical trials 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 trial 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, net of estimated forfeitures. Compensation expense is recognized for the portion that is ultimately expected to vest over the period during which the recipient renders the required services to

us using the straight-line method. In accordance with authoritative guidance, the fair value of non-employee stock-based awards is re-measured 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 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.

We are also required to estimate forfeitures at the time of grant, and revise those estimates in subsequent periods if actual forfeitures differ from our estimates. We use historical data to estimate pre-vesting option forfeitures and record stock-based compensation expense only for those awards that are expected to vest. To the extent that actual forfeitures differ from our estimates, the difference is recorded as a cumulative adjustment in the period the estimates were revised.

#### *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.

Prior to our initial public, on each grant date, we developed an estimate of the fair value of our common stock in order to determine an exercise price for the option grants based in part on input from an independent third-party valuation as there was no active public market for our common stock. Our determinations of the fair value of our common stock was done using methodologies, approaches and assumptions consistent with the American Institute of Certified Public Accountants, or AICPA, Audit and Accounting Practice Aid Series: *Valuation of Privately-Held-Company Equity Securities Issued as Compensation*, or the AICPA Practice Guide. Our board of directors considered various objective and subjective factors, along with input from management and the independent third-party valuation, to determine the fair value of our common stock, including: external market conditions affecting the biopharmaceutical industry, trends within the biopharmaceutical industry, the prices at which we sold shares of preferred stock, the superior rights and preferences of the preferred stock relative to our common stock at the time of each grant, the results of operations, financial position, status of our research and development efforts, our stage of development and business strategy, the lack of an active public market for our common and our preferred stock, and the likelihood of achieving a liquidity

event such as an IPO. Since our initial public offering, the fair value of our common stock is estimated to be the closing price of our common stock on the NASDAQ Global Market on the applicable date.

### Warrant Liability

Our previously outstanding warrants to purchase shares of preferred stock had provisions by which the underlying issuance is contingently redeemable based on events outside of our control and were recorded as a liability in accordance with ASC 480-10. Warrants classified as liabilities are recorded on our balance sheet at fair value on the date of issuance and are marked-to-market on each subsequent reporting period. Non-cash changes in the fair value at each reporting period are recognized in the statement of operations. Upon the closing of our IPO on July 1, 2013, all warrants exercisable for shares of preferred stock became exercisable for shares of common stock and, as a result, the warrants no longer met the criteria to be classified as liabilities and were reclassified to additional paid-in capital at fair value.

### Results of Operations

#### *Comparison of the Years Ended December 31, 2013 and 2012*

The following table summarizes our results of operations for the years ended December 31, 2013 and 2012:

	Year Ended December 31,		Change
	2013	2012	
	(in thousands)		
<b>Operating Expenses:</b>			
Research and development . . . . .	\$ 16,014	\$ 7,998	\$ 8,016
General and administrative . . . . .	6,745	2,206	4,539
<b>Loss from operations . . . . .</b>	<b>(22,759)</b>	<b>(10,204)</b>	<b>(12,555)</b>
Other income (expense):			
Interest expense . . . . .	(936)	(1,486)	550
Change in fair value of warrant liability . . . . .	(2,587)	32	(2,619)
Other income (expense), net . . . . .	194	(84)	278
<b>Net loss . . . . .</b>	<b><u>\$(26,088)</u></b>	<b><u>\$(11,742)</u></b>	<b><u>\$(14,346)</u></b>

#### *Research and development expenses*

Research and development expenses for the year ended December 31, 2013 were \$16.0 million, compared to \$8.0 million for the year ended December 31, 2012, an increase of \$8.0 million. The increase in research and development expenses is primarily related to the further clinical development of ETC-1002 in our Phase 2 clinical program, which includes the completion of two Phase 2a clinical studies and the initiation of our Phase 2b clinical study in patients with or without statin intolerance.

#### *General and administrative expenses*

General and administrative expenses for the year ended December 31, 2013 were \$6.7 million, compared to \$2.2 million for the year ended December 31, 2012, an increase of \$4.5 million. The increase in general and administrative expenses was primarily attributable to costs to support public company operations, increases in our headcount, which includes increased stock-based compensation expense, and other costs to support our growing organization.

### *Interest expense*

Non-cash interest expense for the year ended December 31, 2013 was \$0.9 million, compared to \$1.5 million for the year ended December 31, 2012, a decrease of \$0.6 million. The decrease in interest expense was primarily related to the conversion of our convertible promissory notes issued in January, September and November 2012, into an aggregate of 16,623,092 shares of Series A preferred stock in February 2013 as well as the a decrease in accrued interest on the 8.931% convertible promissory note issued to Pfizer, which was subsequently converted into 6,750,000 shares of Series A-1 preferred stock on May 29, 2013.

### *Change in fair value of warrant liability*

The outstanding warrants to purchase 277,690 shares of our common stock required liability classification and mark-to-market accounting at each reporting period in accordance with ASC 480-10 prior to the completion of our IPO. The fair values of the warrants were determined using the Monte Carlo or the Black Scholes valuation models and resulted in the recognition of a loss of approximately \$2.6 million related to the change in fair values for the year ended December 31, 2013. Subsequent to our IPO, the warrants were reclassified to equity as they no longer met the criteria for classification as liabilities.

### *Other income (expense), net*

Other income (expense), net for the year ended December 31, 2013 was income of approximately \$194,000 compared to expense of approximately \$84,000 for the year ended December 31, 2012, a \$278,000 increase in income. This increase was primarily related to gains on the sale of assets and an increase in interest income earned on our cash and cash equivalents.

### *Comparison of the Years Ended December 31, 2012 and 2011*

The following table summarizes our results of operations for the years ended December 31, 2012 and 2011:

	Year Ended December 31,		Change
	2012	2011	
	(in thousands)		
<b>Operating Expenses:</b>			
Research and development .....	\$ 7,998	\$ 7,807	\$ 191
General and administrative .....	2,206	2,357	(151)
<b>Loss from operations</b> .....	<b>(10,204)</b>	<b>(10,164)</b>	<b>(40)</b>
Other income (expense):			
Interest expense .....	(1,486)	(577)	(909)
Change in fair value of warrant liability .....	32		32
Other income (expense), net .....	(84)	(76)	(8)
<b>Net loss</b> .....	<b><u>\$(11,742)</u></b>	<b><u>\$(10,817)</u></b>	<b><u>\$(925)</u></b>

### *Research and development expenses*

Research and development expenses for the year ended December 31, 2012 were \$8.0 million, compared to \$7.8 million for the year ended December 31, 2011, an increase of \$0.2 million primarily related to the further clinical development of ETC-1002, including the initiation of two Phase 2a clinical trials, which includes the initiation and completion of our Phase 2a Glucose Proof-of-Concept clinical trial and the initiation of our Phase 2a Lipid Proof-of-Concept clinical trial.

### ***General and administrative expenses***

General and administrative expenses for the year ended December 31, 2012 were \$2.2 million, compared to \$2.4 million for the year ended December 31, 2011, a decrease of \$0.2 million. The decrease in general and administrative expenses was primarily attributable to a decrease in professional consulting services provided to us.

### ***Interest expense***

Non-cash interest expense for the year ended December 31, 2012 was \$1.5 million, compared to \$0.6 million for the year ended December 31, 2011, a \$0.9 million increase in interest expense. This increase in interest expense was primarily related to our issuance of convertible promissory notes in January, September and November 2012, which each bear interest at a rate of 10%, as well as the accrued interest on the 8.931% convertible promissory note issued to Pfizer, which had an outstanding balance of \$7,528,845 as of December 31, 2012.

### ***Change in fair value of warrant liability***

The outstanding warrants to purchase 1,940,000 shares of our Series A preferred stock require liability classification and mark-to-market accounting at each reporting period in accordance with ASC 480-10. The fair values of the warrants were determined using the Monte Carlo simulation valuation model and resulted in the recognition of a gain of \$32,000 related to the change in fair values for the year ended December 31, 2012.

### ***Other income (expense), net***

Other expense, net for the year ended December 31, 2012 was approximately \$84,000 compared to \$76,000 for the year ended December 31, 2011, an \$8,000 decrease. This decrease was primarily related to a reduction in interest income earned on our money market funds.

## **Liquidity and Capital Resources**

We have funded our operations since inception through the sale of common stock in our IPO, private placements of preferred stock, convertible promissory notes and warrants to purchase shares of preferred stock. To date, we have not generated any revenue, and we anticipate that we will continue to incur losses for the foreseeable future.

In July 2013, we completed our IPO pursuant to a registration statement on Form S-1. In the IPO, we issued and sold an aggregate of 5,750,000 shares of common stock, including the underwriters' exercise in full of their over-allotment option, under the registration statement at a public offering price of \$14.00 per share. Net proceeds were approximately \$72.2 million, after deducting underwriting discounts and commissions and offering expenses.

As of December 31, 2013, our primary sources of liquidity were our cash and cash equivalents and available-for-sale investments, which totaled \$56.5 million and \$21.1 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:

	<b>Year Ended December 31,</b>	
	<b>2013</b>	<b>2012</b>
	<b>(in thousands)</b>	
Cash (used in) operating activities . . . . .	\$(18,113)	\$(10,809)
Cash provided by (used in) investing activities . . . . .	(21,002)	(2)
Cash provided by financing activities . . . . .	89,141	15,751
<b>Net increase (decrease) in cash and cash equivalents . . . . .</b>	<b>\$ 50,026</b>	<b>\$ 4,940</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 ETC-1002.

Net cash used in operating activities totaled \$18.1 million and \$10.9 million for the year ended December 31, 2013 and 2012, respectively. The primary use of our cash was to fund the development of ETC-1002, adjusted for non-cash expenses, such as depreciation and amortization, interest expense, stock-based compensation expense, mark-to-market of our warrants previously classified as liabilities, and changes in working capital.

***Investing Activities***

Net cash used in investing activities of \$21.0 million for the year ended December 31, 2013 consisted primarily of our purchase of highly liquid, interest bearing investment-grade and government securities. Net cash used in investing activities of approximately \$1,700 in the year ended December 31, 2012 consisted primarily of property and equipment purchases, partially off-set by our sale of certain assets.

***Financing Activities***

Net cash provided by financing activities of \$89.1 million for the year ended December 31, 2013 related primarily to the net proceeds of our IPO in July 2013 and the issuance and sale of 17,000,000 shares of our Series A preferred stock at a price of \$1.00 per share in April 2013. Net cash provided by financing activities of \$15.8 million for the year ended December 31, 2012 consisted primarily of the issuance of convertible promissory notes.

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

ETC-1002 is currently in Phase 2b clinical development, and we expect to continue to incur significant expenses and increasing operating losses for the foreseeable future. We expect that our existing cash and cash equivalents and available-for-sale investments will enable us to fund our operating expenses and capital expenditure requirements through at least the end of 2015 and that we will likely need to raise additional capital thereafter to continue to fund the further development of ETC-1002 and our operations. We expect to announce top-line results from our Phase 2b ETC-1002-008 clinical study and our Phase 2b ETC-1002-009 clinical study by the end of 2014 and to have an end-of-Phase 2 meeting with the FDA in the first half of 2015. 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 ETC-1002, and the extent to which we may enter into collaborations with pharmaceutical partners regarding the development and commercialization of

ETC-1002, we are unable to estimate the amounts of increased capital outlays and operating expenses associated with completing the development and commercialization of ETC-1002. Our future funding requirements will depend on many factors, including, but not limited to:

- our ability to successfully develop and commercialize ETC-1002 and our other product candidates;
- the costs, timing and outcomes of our ongoing and planned clinical studies of ETC-1002;
- the time and cost necessary to obtain regulatory approvals for ETC-1002, if at all;
- our ability to establish a sales, marketing and distribution infrastructure to commercialize ETC-1002 in the United States and abroad 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, we may have to relinquish valuable rights to our technologies, future revenue streams or ETC-1002 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 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 ETC-1002 that we would otherwise prefer to develop and market ourselves.

### Contractual Obligations and Commitments

We lease office and laboratory space in Plymouth, MI under an operating lease agreement that was originally scheduled to expire on October 2, 2013. In August 26, 2013, we entered into an amendment to the lease to extend the expiration date of the initial term from October 2, 2013 to April 30, 2014.

The following table summarizes our future minimum lease obligations as of December 31, 2013:

	<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 . . . . .	\$101	\$101	\$—	\$—	\$—
Total . . . . .	<u>\$101</u>	<u>\$101</u>	<u>\$—</u>	<u>\$—</u>	<u>\$—</u>

On February 4, 2014 we entered into a lease for facilities in Ann Arbor, MI, scheduled to expire 63 months after its commencement. We anticipate moving into this space in April 2014, at which point our principal office space will be relocated there. The lease provides for a fixed monthly rent of approximately \$7,941 per month, with monthly rent increasing every 12 months subsequent to the first

3 months of the lease, and also provides for certain rent adjustments to be paid as determined by the landlord.

We also hold a license agreement in which we are obligated to make future minimum annual payments of \$50,000 in years where there is not a milestone payment required under the terms of the agreement (see Note 15 to our audited financial statements). Further, we are contractually obligated to issue up to an aggregate of 11,451 shares of common stock upon meeting various future milestones set forth in the agreement.

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 \$56.5 million and \$21.1 million at December 31, 2013. 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.

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, 2013.

**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 principal financial officer, to allow timely decisions regarding required disclosure.

As of December 31, 2013, 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 has concluded based upon the evaluation described above that, as of December 31, 2013, our disclosure controls and procedures were effective at the reasonable assurance level.

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

This Annual Report on Form 10-K does not include a report of management's assessment regarding internal control over financial reporting or an attestation report of our independent registered public accounting firm due to a transition period established by rules of the Securities and Exchange Commission for newly public companies.

**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.

**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 2014 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 2014 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 2014 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 2014 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 2014 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 15. Exhibits, Financial Statement Schedules**

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

(1) Financial Statements:

Report of Independent Registered Public Accounting Firm .....	F-2
Consolidated Balance Sheet .....	F-3
Consolidated Statements of Operations and Comprehensive Loss .....	F-4
Consolidated Statements of Changes in Stockholders' Equity (Deficit) .....	F-5
Consolidated Statements of Cash Flows .....	F-6
Notes to Consolidated Financial Statements .....	F-7

(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 immediately following our consolidated financial statements. The Exhibit Index is incorporated herein 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: March 13, 2014

By:           /s/ TIM M. MAYLEBEN            
          Tim M. Mayleben  
          *President and Chief Executive Officer*  
          *(Principal Executive Officer and*  
          *Principal Financial 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 and Principal Financial Officer)	March 13, 2014
<u>          /s/ RICHARD B. BARTRAM          </u> Richard B. Bartram	Controller (Principal Accounting Officer)	March 13, 2014
<u>          /s/ ROGER S. NEWTON, PH.D., FAHA          </u> Roger S. Newton, Ph.D., FAHA	Executive Chairman, Chief Scientific Officer and Director	March 13, 2014
<u>          /s/ PATRICK ENRIGHT          </u> Patrick Enright	Director	March 13, 2014
<u>          /s/ DOV A. GOLDSTEIN, M.D.          </u> Dov A. Goldstein, M.D.	Director	March 13, 2014
<u>          /s/ ANTONIO M. GOTTO, M.D., D. PHIL          </u> Antonio M. Gotto, M.D., D. Phil	Director	March 13, 2014

<u>Signature</u>	<u>Title</u>	<u>Date</u>
<u>/s/ DANIEL JANNEY</u> Daniel Janney	Director	March 13, 2014
<u>/s/ LOUIS G. LANGE, M.D., PH.D.</u> Louis G. Lange, M.D., Ph.D.	Director	March 13, 2014
<u>/s/ MARK E. MCGOVERN, M.D.</u> Mark E. McGovern, M.D.	Director	March 13, 2014
<u>/s/ NICOLE VITULLO</u> Nicole Vitullo	Director	March 13, 2014

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**

**Index to the Financial Statements**

**Years Ended December 31, 2013 and 2012, and Period From  
January 22, 2008 (Inception) to December 31, 2013**

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

The Board of Directors and Shareholders  
Esperion Therapeutics, Inc.

We have audited the accompanying balance sheets of Esperion Therapeutics, Inc. (a development stage company) (the Company) as of December 31, 2013 and 2012, and the related statements of operations and comprehensive loss, convertible preferred stock and stockholders' equity (deficit), and cash flows for each of the three years in the period ended December 31, 2013, and for the period from January 22, 2008 (Inception) to December 31, 2013. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Esperion Therapeutics, Inc. at December 31, 2013 and 2012, and the results of its operations and its cash flows for each of the three years in the period ended December 31, 2013, and for the period from January 22, 2008 (Inception) through December 31, 2013, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Detroit, Michigan  
March 13, 2014

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Balance Sheets**

	<u>December 31,</u> <u>2013</u>	<u>December 31,</u> <u>2012</u>
<b>Assets</b>		
Current assets:		
Cash and cash equivalents . . . . .	\$ 56,537,361	\$ 6,511,521
Short-term Investments . . . . .	3,525,123	—
Prepaid clinical development costs . . . . .	195,652	367,216
Other prepaid and current assets . . . . .	361,513	259,669
Total current assets . . . . .	60,619,649	7,138,406
Property and equipment, net . . . . .	80,808	120,210
Intangible assets . . . . .	55,740	53,825
Long-term investments . . . . .	17,537,663	—
Total assets . . . . .	<u>\$ 78,293,860</u>	<u>\$ 7,312,441</u>
<b>Liabilities, convertible preferred stock and stockholders' equity (deficit)</b>		
Current liabilities:		
Short term borrowings with related parties, net of debt discount . . . . .	\$ —	\$ 15,241,007
Accrued interest . . . . .	—	738,192
Accounts payable . . . . .	2,231,890	476,277
Accrued clinical development costs . . . . .	883,465	242,171
Warrant liabilities . . . . .	—	265,323
Other accrued liabilities . . . . .	1,087,136	210,329
Total current liabilities . . . . .	4,202,491	17,173,299
Long-term debt . . . . .	—	7,528,845
Total liabilities . . . . .	<u>\$ 4,202,491</u>	<u>\$ 24,702,144</u>
Commitments and contingencies (Note 6)		
<b>Convertible preferred stock:</b>		
Series A preferred stock par value \$0.001; no shares authorized, issued or outstanding as of December 31, 2013; 34,785,000 shares authorized and 23,975,000 shares issued and outstanding at December 31, 2012, aggregate liquidation preference of \$23,975,000 at December 31, 2012 . . . . .	—	23,975,000
<b>Stockholders' equity (deficit):</b>		
Preferred stock, \$0.001 par value; 5,000,000 shares authorized and no shares issued or outstanding as of December 31, 2013; no shares authorized, issued or outstanding as of December 31, 2012. . . . .	—	—
Common stock, \$0.001 par value; 120,000,000 shares authorized as of December 31, 2013 and 50,000,000 shares authorized as of December 31, 2012, respectively; 15,357,413 shares issued and 15,340,710 outstanding at December 31, 2013 and 346,478 shares issued and outstanding at December 31, 2012 . . . . .	15,357	346
Additional paid-in capital . . . . .	142,142,204	609,976
Accumulated other comprehensive income . . . . .	(3,034)	—
Deficit accumulated during the development stage . . . . .	(68,063,158)	(41,975,025)
Total stockholders' equity (deficit) . . . . .	74,091,369	(41,364,703)
Total liabilities, convertible preferred stock and stockholders' equity (deficit) . . . . .	<u>\$ 78,293,860</u>	<u>\$ 7,312,441</u>

See accompanying notes to the condensed financial statements.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Statements of Operations and Comprehensive Loss**

	Years Ended December 31,			Period from January 22, 2008 (Inception) to December 31, 2013
	2013	2012	2011	
<b>Grant income</b> . . . . .	\$ —	\$ —	\$ —	\$ 244,479
<b>Operating expenses:</b>				
Research and development . . . . .	16,014,205	7,998,128	7,807,702	43,428,066
General and administrative . . . . .	6,744,493	2,205,632	2,356,669	18,194,189
Acquired in-process research and development . . . . .	—	—	—	85,612
Total operating expenses . . . . .	22,758,698	10,203,760	10,164,371	61,707,867
<b>Loss from operations</b> . . . . .	(22,758,698)	(10,203,760)	(10,164,371)	(61,463,388)
Interest expense . . . . .	(936,580)	(1,486,696)	(577,157)	(4,320,696)
Change in fair value of warrant liability . . . . .	(2,586,865)	32,367	—	(2,554,498)
Other income (expense), net . . . . .	194,010	(83,647)	(75,813)	275,424
<b>Net loss</b> . . . . .	\$(26,088,133)	\$(11,741,736)	\$(10,817,341)	\$(68,063,158)
Net loss per common share (basic and diluted) . . . . .	\$ (3.31)	\$ (36.31)	\$ (36.22)	
Weighted-average shares outstanding (basic and diluted) . .	7,885,921	323,382	298,689	
<b>Other comprehensive loss:</b>				
Unrealized loss on investments . .	\$ (3,034)	\$ —	\$ —	\$ (3,034)
<b>Total comprehensive loss</b> . . . . .	\$(26,091,167)	\$(11,741,736)	\$(10,817,341)	\$(68,066,192)

See accompanying notes to the condensed financial statements.



**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**

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

	Series A Convertible Preferred Stock		Series A 1 Convertible Preferred Stock		Common Stock		Additional Paid-In Capital	Deficit Accumulated During the Development Stage	Accumulated Other Comprehensive Loss	Total Stockholders' Equity (Deficit)
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at January 22, 2008 (Inception)	—	\$ —	—	\$ —	—	\$ —	—	\$ —	\$ —	\$ —
Issuance of Series A preferred stock in exchange for cash	17,025,000	17,025,000	—	—	—	—	—	—	—	—
Issuance of Series A preferred stock in exchange for convertible note	250,000	250,000	—	—	—	—	—	—	—	—
Issuance of common stock at \$0.0001 per share	—	—	—	—	286,286	286	(86)	—	—	200
Exercise of stock options	—	—	—	—	3,578	4	3,746	—	—	3,750
Stock-based compensation	—	—	—	—	—	—	98,796	—	—	98,796
Net loss	—	—	—	—	—	—	—	(19,415,948)	—	(19,415,948)
Balance at December 31, 2010	17,275,000	17,275,000	—	—	289,864	290	102,456	(19,415,948)	—	(19,313,202)
Issuance of Series A preferred stock in exchange for cash	6,700,000	6,700,000	—	—	—	—	—	—	—	—
Exercise of stock options	—	—	—	—	15,016	15	15,871	—	—	15,886
Issuance of common stock in consideration for a license agreement	—	—	—	—	2,862	3	4,397	—	—	4,400
Stock-based compensation	—	—	—	—	—	—	78,451	—	—	78,451
Net loss	—	—	—	—	—	—	—	(10,817,341)	—	(10,817,341)
Balance at December 31, 2011	23,975,000	23,975,000	—	—	307,742	308	201,175	(30,233,289)	—	(30,031,806)
Exercise of stock options	—	—	—	—	38,736	38	40,950	—	—	40,988
Beneficial conversion feature from issuance of convertible notes	—	—	—	—	—	—	287,990	—	—	287,990
Stock-based compensation	—	—	—	—	—	—	79,861	—	—	79,861
Net loss	—	—	—	—	—	—	—	(11,741,736)	—	(11,741,736)
Balance December 31, 2012	23,975,000	23,975,000	—	—	346,478	346	609,976	(41,975,025)	—	(41,364,703)
Issuance of Series A preferred stock in exchange for convertible promissory notes	16,623,092	16,623,092	—	—	—	—	—	—	—	—
Issuance of Series A preferred stock, net of issuance costs (\$119,537)	17,000,000	16,880,463	—	—	—	—	—	—	—	—
Issuance of Series A-1 preferred stock in exchange for convertible promissory notes, net of issuance costs (\$53,469)	—	—	6,750,000	7,749,531	—	—	—	—	—	—
Early exercise of stock options and vesting of restricted stock	—	—	—	—	25,765	26	21,167	—	—	21,193
Preferred shares converted into common stock	(57,598,092)	(57,478,555)	(6,750,000)	(7,749,531)	9,210,999	9,211	65,215,954	—	—	65,225,165
Issuance of common stock from initial public offering, net of issuance costs (\$2,671,169)	—	—	—	—	5,750,000	5,750	72,188,081	—	—	72,193,831
Reclassification of warrants from liabilities to equity	—	—	—	—	—	—	2,852,188	—	—	2,852,188
Exercise of stock options	—	—	—	—	24,171	24	27,850	—	—	27,874
Stock-based compensation	—	—	—	—	—	—	1,226,988	—	—	1,226,988
Other comprehensive loss	—	—	—	—	—	—	—	—	(3,034)	(3,034)
Net loss	—	—	—	—	—	—	—	(26,088,133)	—	(26,088,133)
Balance December 31, 2013	—	\$ —	—	\$ —	15,357,413	\$15,357	\$142,142,204	\$(68,063,158)	\$(3,034)	\$ 74,091,369

See accompanying notes to the financial statements.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Statements of Cash Flows**

	Year Ended December 31,			Period from
	2013	2012	2011	January 22, 2008 (Inception) to December 31, 2013
<b>Operating activities</b>				
Net loss . . . . .	\$(26,088,133)	\$(11,741,736)	\$(10,817,341)	\$(68,063,158)
Adjustments to reconcile net loss to net cash used in operating activities:				
Depreciation expense . . . . .	70,551	139,433	178,471	1,447,722
Amortization of debt discount and beneficial conversion . . . . .	458,993	116,988	—	575,981
Amortization of debt issuance costs . . . . .	18,533	15,378	—	33,911
Amortization of premiums and discounts on investments . . . . .	46,758	—	—	46,758
Revaluation of warrants . . . . .	2,586,865	(32,367)	—	2,554,498
Noncash interest expense on convertible notes . . . . .	459,055	1,369,709	577,065	3,726,092
Write-off of acquired in-process research and development . . . . .	—	—	—	85,612
Stock-based compensation expense . . . . .	1,226,988	79,861	78,451	1,484,096
Common stock issued in license agreement . . . . .	—	—	4,400	4,400
Loss related to assets held for sale . . . . .	27,000	86,887	108,308	322,701
Gain on sale of assets . . . . .	(147,805)	(2,549)	—	(166,264)
Changes in assets and liabilities:				
Prepays and other assets . . . . .	28,489	(429,720)	168,277	(508,281)
Accounts payable . . . . .	1,755,613	(214,903)	619,238	2,231,890
Other accrued liabilities . . . . .	1,443,732	(195,548)	14,406	1,896,229
Net cash used in operating activities . . . . .	(18,113,361)	(10,808,567)	(9,068,725)	(54,327,813)
<b>Investing activities</b>				
Purchases of investments . . . . .	(24,676,822)	—	—	(56,245,988)
Proceeds from sales/maturities of investments . . . . .	3,504,791	—	500,350	35,020,141
Cash obtained in stock acquisition . . . . .	—	—	—	2,500,000
Proceeds from sale of assets . . . . .	201,265	5,100	42,312	952,464
Purchase of property and equipment . . . . .	(31,373)	(6,783)	(33,635)	(298,932)
Other investing . . . . .	—	—	—	50,626
Net cash (used in) provided by investing activities . . . . .	(21,002,139)	(1,683)	509,027	(18,021,689)
<b>Financing activities</b>				
Proceeds from initial public offering, net of issuance costs . . . . .	72,193,831	—	—	72,193,831
Proceeds from issuance of preferred stock, net of issuance costs . . . . .	16,824,235	—	6,700,000	40,799,235
Proceeds from exercise of common stock options . . . . .	123,274	40,987	15,886	184,097
Proceeds from warrant issuance . . . . .	—	297,690	—	297,690
Proceeds from debt issuance with related parties . . . . .	—	15,412,010	—	15,412,010
Net cash provided by financing activities . . . . .	89,141,340	15,750,687	6,715,886	128,886,863
Net increase (decrease) in cash and cash equivalents . . . . .	50,025,840	4,940,437	(1,843,812)	56,537,361
Cash and cash equivalents at beginning of period . . . . .	6,511,521	1,571,084	3,414,896	—
Cash and cash equivalents at end of period . . . . .	\$ 56,537,361	\$ 6,511,521	\$ 1,571,084	\$ 56,537,361
<b>Supplemental disclosure of cash flow information:</b>				
Conversion of convertible promissory notes, including accrued interest of \$923,092 into Series A preferred stock . . . . .	\$ 16,623,092	\$ —	\$ —	\$ 16,623,092
Conversion of convertible long-term Pfizer note, including accrued interest of \$274,155 into Series A-1 preferred stock . . . . .	\$ 7,803,000	\$ —	\$ —	\$ 7,803,000

See accompanying notes to the financial statements.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements**

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

The Company is a clinical stage biopharmaceutical company focused on developing and commercializing first-in-class, oral, low-density lipoprotein cholesterol (LDL-C) lowering therapies for the treatment of patients with hypercholesterolemia and other cardiometabolic risk markers. ETC-1002, the Company's lead product candidate, is a unique, first-in-class, orally available, once-daily small molecule designed to lower LDL-C levels and avoid the side effects associated with LDL-C lowering therapies currently available. ETC-1002 is being developed primarily for patients intolerant of statins with elevated levels of LDL-C. Phase 2b clinical trials for ETC-1002 are currently underway and build upon a successful and comprehensive Phase 1 and Phase 2 program. The Company owns the exclusive worldwide rights to ETC-1002 and our other product candidates.

HDL Therapeutics, Inc. (HDL) was incorporated in the state of Delaware on January 22, 2008. On April 28, 2008, HDL acquired all of the capital stock of Esperion Therapeutics, Inc. (Esperion), a wholly owned subsidiary of Pfizer Inc. On May 5, 2008, Esperion was merged with and into HDL and the Company assumed the name Esperion Therapeutics, Inc. (the Company). Its facilities are located in Plymouth, Michigan.

The Company's primary activities since incorporation have been recruiting personnel, conducting research and development activities, including pre-clinical and clinical testing, performing business and financial planning, and raising capital. Accordingly, the Company is considered to be in development stage.

The Company is subject to the risks associated with a development stage entity, which includes 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 finance operations with a combination of public and private equity issuances, debt arrangements, collaborations and strategic and licensing arrangements. 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.

**Reverse Stock Split**

On June 11, 2013, in connection with its initial public offering (the IPO), the Company effectuated a 1-for-6.986 reverse stock split of its outstanding common stock, which was approved by the Company's board of directors on June 5, 2013. The reverse stock split resulted in an adjustment to the Series A preferred stock and Series A-1 preferred stock conversion prices to reflect a proportional decrease in the number of shares of common stock to be issued upon conversion. The accompanying financial statements and notes to the financial statements give effect to the reverse stock split for all periods presented. The shares of common stock retained a par value of \$0.001 per share. Accordingly, the stockholders' equity reflects the reverse stock split by reclassifying from "common stock" to "Additional paid-in capital" in an amount equal to the par value of the decreased shares resulting from the reverse stock split.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

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

**Initial Public Offering**

On July 1, 2013, the Company completed its IPO whereby the Company sold 5,000,000 shares of common stock at a price of \$14.00 per share. The shares began trading on the Nasdaq Global Market on June 26, 2013. On July 11, 2013, the underwriters exercised their over-allotment option in full and purchased an additional 750,000 shares of common stock at a price of \$14.00 per share. The Company received approximately \$72.2 million in net proceeds from the IPO, including proceeds from the exercise of the underwriters' over-allotment option, net of underwriting discounts and commissions and offering expenses. Upon closing of the IPO, all outstanding shares of preferred stock converted into 9,210,999 shares of common stock; and warrants exercisable for convertible preferred stock were automatically converted into warrants exercisable for 277,690 shares of common stock, resulting in the reclassification of the related convertible preferred stock warrant liability of \$2,852,188 to additional paid-in capital (See Note 5).

The following table summarizes the Company's capitalization upon closing of its initial public offering:

Total common stock issued as of June 30, 2013 . . . . .	396,414
Conversion of Series A preferred stock into common stock upon closing of IPO . . . . .	8,244,781
Conversion of Series A-1 preferred stock into common stock upon closing of IPO . . . . .	966,218
Sales of common stock through IPO . . . . .	<u>5,000,000</u>
Common stock issued as of July 1, 2013 . . . . .	14,607,413
Issuance of common stock to underwriters due to exercise of over-allotment . . . . .	<u>750,000</u>
Total common stock issued as of July 11, 2013 . . . . .	<u>15,357,413</u>

**2. Summary of Significant Accounting Policies**

**Use of Estimates**

The preparation of financial statements in conformity with U.S. generally accepted accounting principles (GAAP) 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.

Prior to the completion of the IPO on July 1, 2013, the Company utilized significant estimates and assumptions in determining the fair value of its Common Stock. The Company utilized valuation methodologies in accordance with the framework of the 2004 American Institute of Certified Public Accountants Technical Practice Aid, Valuation of Privately-Held Company Equity Securities Issued as Compensation, to estimate the fair value of its Common Stock. Each valuation methodology includes estimates and assumptions that require the Company's judgment. These estimates and assumptions include a number of objective and subjective factors, including external market conditions affecting the biotechnology industry sector, the prices at which the Company sold shares of its Preferred Stock, the

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

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

superior rights and preferences of securities senior to its Common Stock at the time and the likelihood of achieving a liquidity event, such as an initial public offering or sale. Significant changes to the key assumptions used in the valuations could result in different fair values of Common Stock at each valuation date.

**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' deficit. 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 (expense), net. Realized gains and losses, if any, are determined using the specific identification method and in other income (expense), net. Investments 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 levels of low-density lipoprotein cholesterol and other cardiometabolic risk markers.

**Fair Value of Financial Instruments**

The Company's financial instruments consist principally of cash and cash equivalents, investments, other current assets, accounts payable and accrued liabilities that approximate their carrying value at December 31, 2013 and 2012.

**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.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

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

**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. Excluding impairment losses recorded on assets held for sale, no other impairment losses have been recorded through December 31, 2013.

**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 pre-clinical studies and trials, non-clinical activities (such as toxicology studies), regulatory activities, manufacturing activities to support clinical activities, research-related overhead expenses, and fees paid to external service providers that conduct certain research and development, clinical, and manufacturing activities on behalf of the Company. Research and development costs are expensed as incurred.

**In-Process Research and Development**

In April 2008, the Company acquired certain tangible research and development assets and intellectual property from Pfizer Inc. (Pfizer). As the acquired in-process research and development had not reached technological feasibility and had no alternative future uses in connection with this asset and intellectual property acquisition and the related purchase price allocation, the Company expensed \$85,612 as in-process research and development costs in 2008.

**Accrued Clinical Development Costs**

Outside research costs are a component of research and development expense. These expenses include fees paid to contract 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 deferred tax assets and as such, it has recorded a full valuation allowance.



**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

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

**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 liabilities are recorded on the Company's balance sheet at their fair value on the date of issuance and are marked-to-market on each subsequent reporting period, with the fair value changes recognized in the statement of operations. 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. The Company adjusts the liability for changes in the fair value of the warrants until the earlier of the exercise of the warrants, the expiration of the warrants, or until such time as the warrants are no longer determined to be liabilities and reclassified into paid-in-capital at fair value. The warrants were reclassified into paid-in-capital upon closing of the IPO. (See Note 5).

**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. Additionally, under the provisions of ASC 718, the Company is 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 are 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.

**Reclassifications**

Certain prior period information has been reclassified to be comparable to the current year presentation. These items had no impact on the amounts of previously reported net loss or total shareholder's equity (deficit).

**Recent Accounting Pronouncements**

In February 2013, the Financial Accounting Standards Board (FASB) issued Accounting Standards Update (ASU) 2013-02 which is an amendment to the accounting guidance for the presentation of comprehensive income. Under the amended guidance, items that are reclassified to net income from accumulated other comprehensive income in the same reporting period require separate disclosure on the face of the financial statements where net income is presented or within the notes to the financial statements. The adoption of this update did not have a material impact on the Company's financial statements.

In July 2013, the FASB issued ASU 2013-11 which is an amendment to the accounting guidance on income taxes. This guidance provides clarification on the financial statement presentation of an unrecognized benefit when a net operating loss carryforward, a similar tax loss, or a tax credit

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

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

carryforward exists. The amendment will be effective for the Company for interim and annual periods beginning after December 15, 2013, with early adoption permitted. The adoption of this standard is not expected to have a material impact on the Company's financial statements.

**3. Stock Acquisition**

On April 28, 2008, HDL acquired all of the capital stock of Esperion from Pfizer in exchange for a non-subordinated convertible promissory note in the original principal amount of \$5,000,000 (see Note 4).

The Company allocated the purchase price of the Esperion acquisition in accordance with the provisions of Statement of Financial Accounting Standards (SFAS) No. 141, *Business Combinations*, related to the purchase of a group of assets. SFAS No. 141 provides that the cost of a group of assets acquired in a transaction other than a business combination shall be allocated to the individual assets acquired based on their relative fair values and shall not give rise to goodwill.

In accordance with the provisions of SFAS No. 141, this transaction did not meet the criteria of a business combination, and all identifiable intangible assets, including in-process research and development, were assigned a portion of the purchase price based on their relative fair values. To this end, an independent valuation of the tangible assets acquired was used to determine the fair value of the identifiable tangible assets. The Company determined the value assigned to in-process research and development and intangible assets. The fair value of assets acquired exceeded the transaction consideration and therefore, under SFAS No. 141, the excess of fair value of assets received over consideration paid was allocated on a relative fair market value basis to in-process research and development, tangible and intangible assets.

The Company allocated total cost of the Esperion acquisition as follows:

Cash . . . . .	\$2,500,000
Property and equipment . . . . .	1,317,005
Intangible assets . . . . .	50,000
In-process research and development . . . . .	85,612
Assets held for sale . . . . .	<u>1,047,383</u>
Total . . . . .	<u>\$5,000,000</u>

The income approach was used to estimate the fair value of the acquired in-process research and development based on projected cash flows through 2014 and a 35 percent discount rate. Material cash inflows were projected to begin in 2014. The replacement cost method was used to estimate the fair value of the tangible assets.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

**4. Debt**

The following is a reconciliation of the Company's various debt instruments:

	December 31,	
	2013	2012
Short term convertible notes issued January 2012 . . . . .	\$ —	\$ 6,000,000
Short term convertible notes issued September 2012 . . . .	—	4,000,000
Short term convertible notes issued November 2012 . . . .	—	5,700,000
Discount on short term convertible notes . . . . .	—	(458,993)
Total short term convertible notes, net of debt discount . . . . .	—	15,241,007
Long term debt . . . . .	—	5,000,000
Accumulated paid-in-kind interest . . . . .	—	2,528,845
Total long-term debt . . . . .	—	7,528,845
Total debt . . . . .	\$ —	\$22,769,852

**Convertible Notes**

In January 2012, the Company issued \$6,000,000 of 10% convertible promissory notes to certain existing investors for cash. In September and November 2012, the Company issued the aggregate of \$9,700,000 of 10% convertible promissory notes that mature on September 4, 2013 for cash to certain existing investors. In connection with the September convertible note financing, the Company and the holders of the January 2012 convertible promissory notes agreed to extend the maturity date of the January 2012 notes to September 4, 2013. In February 2013, these convertible promissory notes, with an outstanding principal of \$15,700,000 and accrued interest of \$923,092, were amended and then converted into 16,623,092 shares of Series A preferred stock, in accordance with their terms and at their conversion price of \$1.00 per share, and following such conversion, the notes were cancelled.

The holders of the September convertible promissory notes received the benefit of a deemed conversion price of the September convertible promissory notes that were below the estimated fair value of the Series A convertible preferred stock at the time of their issuance. The fair value of this beneficial conversion feature was estimated to be \$287,990. The fair value of this beneficial conversion feature was recorded to debt discount and amortized to interest expense using the effective interest method over the term of the convertible promissory notes. As a result of the conversion of the convertible promissory notes into shares of Series A preferred stock in February 2013, the Company recorded the remaining accretion of the beneficial conversion feature of \$229,496 as interest expense during the year ended December 31, 2013.

In connection with the issuance of the September and the November 2012 convertible promissory notes, the Company issued warrants to purchase shares of Series A preferred stock for an aggregate price of \$9,700. The estimated fair value of the warrants at issuance was \$297,690. The proceeds from the sale of the preferred stock and warrants was allocated with \$9,412,010 to the convertible promissory notes and \$297,690 to warrants. This resulted in a discount on the convertible promissory notes which was amortized into interest expense, using the effective interest method, over the life of the convertible promissory notes (see Note 5). The Company recorded \$58,494 of interest expense for the accretion of

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

**4. Debt (Continued)**

this discount during the year ended December 31, 2012. As a result of the conversion of the convertible promissory notes into shares of Series A preferred stock in February 2013, the Company recorded the remaining \$229,496 of interest expense for the accretion of this discount during the year ended December 31, 2013 and \$297,690 during the period from Inception through December 31, 2013.

In April 2008, the Company acquired all of the capital stock of Esperion from Pfizer in exchange for a non-subordinated convertible note in the original principal amount of \$5,000,000. This convertible promissory note had a maturity date of April 28, 2018. The note bore interest at 8.931% annually, payable semiannually on June 30 and December 31 by adding such unpaid interest to the principal of the note, which would thereafter accrue interest. During the years ended December 31, 2013, 2012, 2011, and the period from Inception through December 31, 2013, the Company accrued interest related to the note of \$274,155, \$631,517, \$577,065, and \$2,803,000, respectively.

In May 2013 the Company entered into a stock purchase agreement with Pfizer Inc. and sold 6,750,000 shares of Series A-1 preferred stock at a price of \$1.1560 per share, which was the fair value at the transaction date. The purchase price was paid through the cancellation of all outstanding indebtedness, including accrued interest, under the Pfizer convertible promissory note, which had an outstanding balance, including accrued interest, of \$7,803,000 as of May 29, 2013. The Series A-1 preferred stock issued in connection with this transaction was subsequently converted into 966,218 shares of common stock upon completion of the IPO on July 1, 2013.

**5. Warrants**

In connection with its various financing transactions, the Company issued warrants to purchase shares of preferred stock which had provisions where the underlying issuance was contingently redeemable based on events outside the Company's control and were recorded as a liability in accordance with ASC 480-10. The warrants were classified as liabilities and were recorded on the Company's balance sheet at fair value on the date of issuance and marked-to-market on each subsequent reporting period, with the fair value changes recognized in the statement of operations. Subsequent to the pricing of the IPO, the Company estimated the fair values of the warrants at each reporting period 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 warrants, the risk free interest rate and the fair value of the common stock underlying the warrants. 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 warrants. 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 warrants. The expected remaining life of the warrants is assumed to be equivalent to their remaining contractual term. Prior to the pricing of the IPO, a Monte Carlo valuation model was utilized to estimate the fair value of the warrants based on the probability and timing of future financings.

The assumptions used in calculating the estimated fair market value at each reporting period prior to the closing of the Company's IPO represent the Company's best estimate, however, do involve inherent uncertainties. The estimated fair value of the warrants was determined using the Monte Carlo valuation model which totaled \$297,690 and was comprised of \$141,779 and \$155,911 as of and for the September and November 2012 financing, respectively, and was recorded as a discount on the related convertible promissory notes and amortized as interest expense over the term of the convertible

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

**5. Warrants (Continued)**

promissory notes. Inherent in the Monte Carlo valuation model are assumptions related to expected stock-price volatility, expected life and risk-free interest rate. 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 warrants. The risk-free interest rate is based on the U.S. Treasury zero-coupon bond on the grant date for a maturity similar to the expected remaining life of the warrants. The expected life of the warrants is assumed to be equivalent to their remaining contractual term. The dividend rate is based on the historical rate, which the Company anticipates to remain at zero. The Monte Carlo model was used prior to the closing of the Company's IPO to appropriately value the potential future exercise price based on various exit scenarios. This requires Level 3 inputs which are based on the Company's estimates of the probability and timing of potential future financings.

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, 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. The 277,690 warrants outstanding as of December 31, 2013 expire in February 2018. During the years ended December 31, 2013, 2012, 2011, and for the period from Inception through December 31, 2013, the Company recognized a (loss)/gain of \$(2,586,865), \$32,367, \$0, and \$(2,554,498), respectively, relating to the change in the fair value of the warrant liability.

**6. Commitments and contingencies**

In August 2013, the Company entered into the second amendment to the operating lease agreement for its current office and laboratory facility in Plymouth, MI which extended the expiration date of the initial term from October 2, 2013 to April 30, 2014. The Company's facility lease provides for a fixed monthly rent for the term of the lease and also provides for certain rent adjustments to be paid as determined by the landlord.

The total rent expense for the years ended December 31, 2013, 2012, 2011, and for the period from Inception to December 31, 2013, was approximately \$344,604, \$335,000, \$323,000 and \$1,631,004, respectively. Future minimum payments as of December 31, 2013, under the facility lease are presented in the table below:

	<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>
Operating lease . . . . .	\$100,868	\$100,868	\$—	\$—	\$—
Total . . . . .	<u>\$100,868</u>	<u>\$100,868</u>	<u>\$—</u>	<u>\$—</u>	<u>\$—</u>

The Company also holds a license agreement in which it is obligated to make future minimum annual payments of \$50,000 in years where there is not a milestone payment required under the terms of the agreement (see Note 15). Further, the Company is contractually obligated to issue up to an aggregate of 11,451 shares of common stock upon meeting various future milestones set forth in the agreement.

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

**7. Property and Equipment**

Property and equipment consist of the following:

	December 31,	
	2013	2012
Lab equipment . . . . .	\$511,403	\$1,057,276
Computer equipment . . . . .	99,412	99,412
Software . . . . .	118,572	96,668
Furniture and fixtures . . . . .	11,309	11,309
Leasehold improvements . . . . .	21,381	21,381
Assets in Progress . . . . .	6,530	—
Subtotal . . . . .	768,607	1,286,046
Less accumulated depreciation and amortization . . . . .	687,799	1,165,836
Property and equipment, net . . . . .	<u>\$ 80,808</u>	<u>\$ 120,210</u>

Depreciation expense was \$70,551, \$139,433, \$178,471 and \$1,447,722 for the years ended December 31, 2013, 2012, 2011 and the period from Inception through December 31, 2013, respectively.

**8. Other Accrued Liabilities**

Other accrued liabilities consist of the following:

	December 31,	
	2013	2012
Stock based compensation liability . . . . .	\$ 74,208	\$ —
Accrued professional fees . . . . .	209,961	\$136,907
Accrued franchise and property taxes . . . . .	94,600	12,697
Accrued compensation . . . . .	667,164	60,725
Accrued other . . . . .	41,203	—
Total other accrued liabilities . . . . .	<u>\$1,087,136</u>	<u>\$210,329</u>



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

**9. Investments**

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

	December 31, 2013			Estimated Fair Value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
<b>Cash equivalents:</b>				
Money market funds . . . . .	\$ 5,356,453	\$ —	\$ —	\$ 5,356,453
<b>Short-term investments:</b>				
U.S. treasury notes . . . . .	2,070,774	—	(234)	2,070,540
U.S. government agency securities . . . . .	1,454,426	157	—	1,454,583
<b>Long-term investments:</b>				
Certificates of deposit . . . . .	237,525	—	—	237,525
U.S. treasury notes . . . . .	9,116,136	3,595	(2,497)	9,117,234
U.S. government agency securities . . . . .	8,186,958	640	(4,695)	8,182,903
Total . . . . .	<u>\$26,422,272</u>	<u>\$4,392</u>	<u>\$(7,426)</u>	<u>\$26,419,238</u>

	December 31, 2012			Estimated Fair Value
	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	
<b>Cash equivalents:</b>				
Money market funds . . . . .	\$6,357,542	—	—	\$6,357,542
Total . . . . .	<u>\$6,357,542</u>	<u>\$—</u>	<u>\$—</u>	<u>\$6,357,542</u>

At December 31, 2013, 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.

There were no unrealized gains or losses on investments reclassified from accumulated other comprehensive income to other income (expense) in the Statement of Operations during the year ended December 31, 2013.

**10. 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

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

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

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.

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>
<b>December 31, 2013</b>				
Assets:				
Money market funds . . . . .	\$ 5,356,453	\$ 5,356,453	\$ —	\$ —
Available for sale securities:				
Certificates of deposit . . . . .	237,525	237,525	—	—
U.S. treasury notes . . . . .	11,187,774	11,187,774	—	—
U.S. government agency securities . . . . .	9,637,486	—	9,637,486	—
Total assets at fair value . . . . .	<u>\$26,419,238</u>	<u>\$16,781,752</u>	<u>\$9,637,486</u>	<u>\$ —</u>
<b>December 31, 2012</b>				
Assets:				
Money market funds . . . . .	\$ 6,357,542	\$ 6,357,542	\$ —	\$ —
Total assets at fair value . . . . .	<u>\$ 6,357,542</u>	<u>\$ 6,357,542</u>	<u>\$ —</u>	<u>\$ —</u>
Liabilities:				
Warrants . . . . .	\$ 265,323	\$ —	\$ —	\$265,323
Total liabilities at fair value . . . . .	<u>\$ 265,323</u>	<u>\$ —</u>	<u>\$ —</u>	<u>\$265,323</u>

There were no transfers between Levels 1, 2 or 3 during the year ended December 31, 2013 or December 31, 2012.

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

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

The following table summarizes the changes in the fair value of the Company's Level 3 warrant liability for the years ended December 31, 2012 and 2013:

	<b>Warrant Liability</b>
Level 3 Liabilities:	
As of January 1, 2012 . . . . .	\$ —
Issuance of warrants . . . . .	297,690
Change in fair value . . . . .	(32,367)
As of December 31, 2012 . . . . .	\$ 265,323
Change in fair value . . . . .	2,586,865
Reclassification of warrants liabilities to additional paid-in capital in conjunction with the conversion of the convertible preferred stock into common stock upon the closing of the Company's IPO . . . . .	(2,852,188)
As of December 31, 2013 . . . . .	\$ —

**Fair Value Measurements on a Nonrecurring Basis**

In addition to items that are measured at fair value on a recurring basis, the Company also measures assets held for sale at the lower of its carrying amount or fair value on a nonrecurring basis. The Company recognized an impairment expense and other losses relating to assets held for sale during the year ended December 31, 2013, 2012, 2011 and the period from Inception through December 31, 2013 of \$27,000, \$86,887, \$108,308 and \$214,393 based on recent market sales data for similar equipment less the related costs to sell and recent purchase offers. The fair value of assets held for sale at December 31, 2013 was estimated at \$29,108 using a market approach, considering the estimated fair value for other comparable equipment which are Level 3 inputs.

**11. Convertible Preferred Stock and Stockholders' Deficit**

On January 22, 2008, HDL was incorporated in the state of Delaware with 1,000 shares of authorized common stock. In April 2008, the Board of Directors approved an amended and restated certificate of incorporation. The amendment increased HDL's authorized number of shares of common stock to a total of 44,025,145 and authorized two new series of preferred stock designated as Series A and Series A-1 preferred stock consisting of 33,250,000 shares of Series A preferred stock and 6,475,145 shares of Series A-1 preferred stock. In April 2008, HDL sold 286,286 shares of common stock and 10,000,000 shares of Series A preferred stock in a private offering (the Initial Financing), raising net proceeds of \$200 and \$10,000,000, respectively. In the Initial Financing, the Company converted an outstanding promissory note in the principal amount of \$250,000 from an officer of the Company into 250,000 shares of Series A preferred stock.

As a result of commencing Phase 1 clinical trials in December 2009, the Company issued 6,000,000 additional shares of Series A preferred stock, raising net proceeds of \$6,000,000 in January 2010 (Second Tranche Shares in the Initial Financing agreement).

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

**11. Convertible Preferred Stock and Stockholders' Deficit (Continued)**

In April 2010, the Company issued an additional 1,000,000 shares of Series A preferred stock to an officer and new investors for \$1,000,000 in net proceeds. In connection with this sale, the Initial Financing agreement was amended to allow for the additional issuance of shares. The Company also amended its certificate of incorporation to increase its number of authorized shares to 45,025,145 shares of common stock and 40,725,145 shares of preferred stock, including 34,250,000 shares of Series A preferred stock and 6,475,145 shares of Series A-1 preferred stock.

In November 2010, the Company issued an additional 25,000 shares of Series A preferred stock to an officer of the Company in exchange for \$25,000. As a result of commencing Phase 2a clinical trials in December 2010, the Company issued 6,700,000 shares of Series A preferred stock, raising net proceeds of \$6,700,000 in January 2011 (Third Tranche Shares in the Initial Financing agreement). The Company also amended its certificate of incorporation to increase the number of authorized shares to 50,000,000 shares of common stock and 41,682,329 shares of preferred stock, including 34,785,000 shares of Series A preferred stock and 6,897,329 shares of Series A-1 preferred stock.

In September 2012, the Company amended its certificate of incorporation to increase the number of authorized preferred shares to 42,647,283, including 34,785,000 shares of Series A preferred stock and 7,862,283 shares of Series A-1 preferred stock.

As of December 31, 2012, the Company did not have sufficient preferred and common shares authorized under its certificate of incorporation to permit the conversion of the outstanding convertible promissory notes issued during 2012. Pursuant to the terms of the note purchase agreements, in the event any or all of the notes were to be converted, the purchasers and the Company agreed to take all action necessary to amend the certificate of incorporation to increase the number of authorized shares of Series A preferred stock and common stock to permit such conversion. The Company subsequently amended its certificate of incorporation to increase the number of shares of Series A preferred stock authorized to 41,636,970 and number of shares of common stock authorized to 56,519,253 in connection with the conversion of the notes on February 12, 2013 into 16,623,092 shares of Series A preferred stock.

In March 2013 and April 2013, the Company amended its certificate of incorporation to increase the number of shares of Series A preferred stock authorized to 42,538,092 and 59,538,092, respectively, and the number of shares of common stock authorized to 58,220,375 and 75,220,375, respectively. On April 19, 2013, the Company issued and sold an aggregate of 17,000,000 shares of Series A preferred stock at a price of \$1.00 per share for proceeds of \$16,880,463, which is net of issuance costs of \$119,537, to funds affiliated with Longitude Capital and certain existing investors. Each share of Series A preferred stock issued in the financing was convertible into 0.143 shares of common stock upon the closing of the Company's IPO.

On May 29, 2013, the Company entered into a stock purchase agreement with Pfizer Inc. and issued and sold 6,750,000 shares of Series A-1 preferred stock at a price of \$1.1560 per share. The purchase price was paid through the cancellation of all outstanding indebtedness, including accrued interest, under the Pfizer convertible promissory note, which had an aggregate balance, including accrued interest, of \$7,803,000 as of May 29, 2013. Each share of Series A-1 preferred stock issued in the agreement was convertible into 0.143 shares of common stock upon the closing of the Company's IPO.

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

**11. Convertible Preferred Stock and Stockholders' Deficit (Continued)**

Upon the closing of the Company's IPO on July 1, 2013, all of the outstanding shares of convertible preferred stock were converted into 9,210,999 shares of common stock. As of December 31, 2013, the Company did not have any convertible preferred stock issued or outstanding.

**Convertible Preferred Stock**

As of December 31, 2013, the Company had authorized a total of 5,000,000 undesignated preferred shares. As of December 31, 2012, the Company had 42,647,283 shares of preferred stock designated in various series. The preferred stock designated as of December 31, 2012 is summarized as follows:

	December 31, 2012		
	Shares Designated	Liquidation Preference Per Share	Shares Issued and Outstanding
Series A . . . . .	34,785,000	\$1.00	23,975,000
Series A-1 . . . . .	7,862,283	—	—
	42,647,283	\$1.00	23,975,000

**Voting**

The holders of preferred stock have various rights and preferences. Each share of Series A and Series A-1 preferred stock has certain voting rights equal to the number of shares of common stock into which it is convertible and votes together as one class with the common stock.

A separate vote of a majority of the Series A preferred stock, equal to the number of shares of common stock into which it is convertible, is required for certain activities, including certain issuances of common stock; for any redemption, repurchase, dividend, or other distribution with respect to the common stock; any agreement by the Company or its stockholders regarding certain mergers or consolidations of the Company; a sale of all or substantially all of the assets of the Company; or any redemption, repurchase, dividend, or other distribution with respect to any shares of preferred stock.

As the Series A preferred stock could be redeemed in a "deemed liquidation" in the event of a change of control and the redemption features are considered to be outside the control of the Company, all shares of Series A preferred stock have been presented outside of permanent equity in accordance with ASC 480.

**Liquidation**

In the event of any liquidation, dissolution, or winding-up of the Company, including a merger, acquisition, or sale of assets where the holders of the Company's common stock and preferred stock own less than 50% of the resulting voting power of the surviving entity, the holders of Series A preferred stock shall be entitled to receive prior and in preference to any distribution of the assets of the Company to the holders of Series A-1 preferred stock and common stock, an amount equal to \$1.00 for each share of Series A preferred stock held, plus any declared but unpaid dividends. After payment of the full liquidation preference to holders of Series A preferred stock, but prior to any distribution or payment to holders of common stock, the holders of Series A-1 preferred shall be

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

**11. Convertible Preferred Stock and Stockholders' Deficit (Continued)**

entitled to receive a distribution equal to the original issue price of a share of Series A-1 preferred stock plus any declared but unpaid dividends. After payment of the full liquidation preference(s) to the Series A and Series A-1 stockholders, the remaining assets legally available for distribution shall be distributed ratably to the holders of common stock and preferred stock on an as if converted to common stock basis.

**Dividends**

Holders of Series A and Series A-1 preferred stock, in preference to the holders of common stock, are entitled to receive cash dividends at the rate of eight percent of the respective original issue price per annum on each outstanding preferred share on a pari passu basis. Such dividends are payable only when, as and if declared by the Board of Directors and are non-cumulative. There have been no dividends declared, accrued or paid during the period from Inception through December 31, 2013.

**Conversion**

Any share of Series A or Series A-1 preferred stock may be converted at the option of the holder at any time into shares of common stock at the Series A preferred conversion price or the Series A-1 preferred conversion price then in effect.

Each share of Series A and Series A-1 preferred stock shall automatically be converted into shares of common stock based upon the then-effective Series A preferred conversion price and the Series A-1 preferred conversion price, respectively, upon the affirmative election of the holders of at least 60% of the outstanding shares of the Series A preferred stock and Series A-1 preferred stock voting as a single class.

Each share of Series A preferred stock shall automatically convert into shares of common stock based upon the effective Series A preferred conversion price upon (i) the affirmative election of the holders of at least two-thirds of the outstanding shares of the Series A preferred stock; (ii) the Company's sale of its common stock in a firmly underwritten public offering in which the per share price is at least three times the Series A original issue price adjusted for stock splits, dividends, recapitalizations, and the like, and which would result in gross proceeds to the Company of at least \$40 million (prior to deducting underwriting discounts and commissions); or (iii) the affirmative election of at least a majority of the outstanding shares of the Series A preferred stock following the closing of a firmly underwritten public offering that covers the offer and sale of common stock for the Company that does not meet the three times original issue price or gross proceeds requirements above. Upon an automatic conversion, any declared and unpaid dividends shall be paid to the holders of Series A preferred stock.

The Series A preferred conversion rate is the \$1.00 Series A original issue price, divided by the Series A preferred conversion price, which was initially set at \$1.00 and was adjusted to \$6.986, on June 11, 2013 when the Company effected its 1-for-6.986 reverse stock split, making the Series A preferred conversion rate equal to 0.143. The Series A-1 preferred conversion rate is the \$1.1560 Series A-1 original issue price, divided by the Series A preferred conversion price, initially set at \$1.1560 and was adjusted to \$8.076 on June 11, 2013 when the Company effected its 1-for-6.986 reverse stock split, making the Series A-1 preferred conversion rate equal to 0.143. Upon closing of the



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

**11. Convertible Preferred Stock and Stockholders' Deficit (Continued)**

Company's IPO, all outstanding shares of preferred stock converted into 9,210,999 shares of common stock (see Note 1).

**12. Stock Compensation**

**2013 Stock Option and Incentive Plan**

On June 7, 2013, the Company's stockholders approved the 2013 Stock Option and Incentive Plan (the 2013 Plan), which became effective on June 25, 2013. The number of shares of stock reserved and available for issuance under the 2013 Plan is the sum of (i) 1,100,000, plus (ii) 54,129 shares originally reserved under the Company's 2008 Incentive Stock Option and Restricted Stock Plan (the 2008 Plan) that became available for issuance under the 2013 Plan upon completion of the Company's initial public offering, plus (iii) the shares underlying any awards granted under the 2008 Plan that are forfeited, canceled, held back upon the exercise of an option 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 stock or otherwise terminated (other than by exercise). Additionally, on January 1, 2014 and each January 1 thereafter, the number of shares reserved and available for issuance under the 2013 Plan shall be cumulatively increased by two and a half percent of the number of shares issued and outstanding on the immediately preceding December 31 or such lesser number of shares as determined by the plan administrator.

**2008 Stock Option and Restricted Stock Plan**

In April 2008, the Company adopted the 2008 Incentive Stock Option and Restricted Stock Plan (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 (ISOs), restricted stock awards (RSAs) or nonqualified stock options (NQSOs). 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.

Under the 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 10 year life and expire if not exercised within that period, or if not exercised within 90 days of cessation of employment with the Company.

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

**12. Stock Compensation (Continued)**

The following table summarizes the activity relating to the Company's options to purchase common stock for the year ended December 31, 2013:

	Number of Options	Weighted-Average Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding at December 31, 2012 . . . . .	211,500	\$ 1.43	7.5	\$ 141,389
Granted . . . . .	1,251,749	\$10.62		
Forfeited or expired . . . . .	(12,212)	\$ 2.03		
Exercised . . . . .	(49,936)	\$ 2.47		
Outstanding at December 31, 2013 . . . . .	<u>1,401,101</u>	\$ 9.59	8.95	\$7,755,321

The following table summarizes information about the Company's stock option plan as of December 31, 2013:

	Number of Options	Weighted-Average Price Per Share	Weighted-Average Remaining Contractual Term (Years)	Aggregate Intrinsic Value
Vested and expected to vest at December 31, 2013 . . . . .	<u>1,334,221</u>	\$9.49	8.92	\$7,505,458
Exercisable at December 31, 2013 . . . . .	<u>594,176</u>	\$2.81	8.16	\$6,572,563

The following table shows the weighted-average assumptions used to compute the share-based compensation costs for the stock options granted to employees and non-employees during the period from Inception to December 31, 2013, using the Black-Scholes option pricing model:

	Year ended December 31,			Period From January 22, 2008 (Inception) Through December 31, 2013
	2013	2012	2011	
Risk-free interest rate . . . . .	1.45%	0.85%	2.50%	1.58%
Dividend yield . . . . .	—	—	—	—
Weighted-average expected life of options (years) . . . . .	6.26	6.25	6.25	6.26
Volatility . . . . .	74%	80%	80%	75%

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 simplified method as prescribed by the Securities and Exchange Commission (SEC) 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.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

**12. Stock Compensation (Continued)**

The weighted-average grant-date fair values of stock options granted during the years ended December 31, 2013, 2012, 2011, and the period from Inception through December 31, 2013 were \$7.14, \$1.33, \$1.12 and \$5.94, respectively. During the years ended December 31, 2013, 2012, 2011, and the period from Inception through December 31, 2013, the Company recognized stock-based compensation expense of \$1,226,988, \$79,861, \$78,451, and \$1,484,096, respectively.

As of December 31, 2013, there was approximately \$7,346,725 of unrecognized compensation cost related to unvested options, adjusted for forfeitures, which will be recognized over a weighted-average period of approximately 3.5 years.

**13. 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. There have been no Company contributions to the 401(k) Plan during 2013, 2012, 2011, or from Inception through December 31, 2013.

**14. Income Taxes**

There was no provision for income taxes for the year ended December 31, 2013, 2012 and 2011 because the Company has incurred operating losses since inception. At December 31, 2013, the Company has concluded that it is more likely than not that the Company will not realize the benefit of its deferred tax assets due to its history of losses. Accordingly, the net deferred tax assets have been fully reserved.

As of December 31, 2013, 2012 and 2011, the Company had deferred tax assets, before valuation allowance of approximately \$22,804,000, \$14,351,000 and \$10,386,000, 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, 2013, 2012 and 2011, the Company had federal net operating loss carryforwards of approximately \$62,272,000, \$40,465,000 and \$29,206,000, respectively. The federal net operating loss will expire at various dates beginning in 2028, if not utilized. As of December 31, 2013, 2012 and 2011, the Company had state net operating loss carryforwards of approximately \$33,129,000, \$11,322,000 and \$0, respectively. The state net operating loss will expire at various dates beginning in 2022, if not utilized. We have \$191,000 of NOLs related to excess tax benefits generated upon the settlement of stock awards that increased a current year net operating loss. We cannot record the benefit of these losses in the financial statements until the losses are utilized to reduce our income taxes payable at which time we will recognize the tax benefit in equity.

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

**14. Income Taxes (Continued)**

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

	December 31,		
	2013	2012	2011
Federal income tax (benefit) at statutory rate . . . . .	(34.0)%	(34.0)%	(34.0)%
State income tax benefit, net of federal benefit . . . . .	—%	—%	(0.7)%
Permanent items . . . . .	4.9%	0.4%	(0.1)%
Other . . . . .	—%	(0.2)%	3.2%
Change in valuation allowance . . . . .	29.1%	33.8%	31.6%
Effective income tax rate . . . . .	0.0%	0.0%	0.0%

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 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, 2013, 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:

	December 31,	
	2013	2012
Deferred tax assets:		
Federal and state operating loss carryforwards . . . . .	\$ 22,485,000	\$ 14,207,000
Temporary differences . . . . .	319,000	144,000
Total deferred tax assets . . . . .	22,804,000	14,351,000
Valuation allowance . . . . .	(22,804,000)	(14,351,000)
Net deferred tax assets . . . . .	\$ —	\$ —

**15. License Agreement**

In December 2011, the Company entered into a license agreement for certain U.S. and foreign patents and patent applications regarding new high-density lipoprotein therapies to treat cardiovascular disease in exchange for 2,862 shares of common stock, plus an issue fee of \$50,000. The license agreement will expire in 2028, which is the date of the last to expire of the licensed patents. The

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

**15. License Agreement (Continued)**

Company recorded the common stock, which was valued at its fair value of \$4,400, and the issue fee within general and administrative expenses in the statements of operations.

The license agreement provides for a minimum annual payment of \$50,000 for any years in which a milestone is not achieved, fully creditable against any earned royalties per calendar year. In addition, the Company is also contractually obligated to issue up to an aggregate of 11,451 shares of common stock upon various milestones set forth in the agreement.

Milestone achievement payments are due within 30 days of the milestone achievement. No milestones have been achieved to date under the license agreement. Additionally, the agreement provides for the Company to reimburse the patent holder for certain patent costs during the term of the agreement. The Company recognized expenses associated with this license agreement of \$50,000, \$50,000, \$54,400, and \$154,400 during the years ended December 31, 2013, 2012, 2011, and the period from Inception through December 31, 2013, respectively, in general and administrative expenses.

**16. 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 share equivalents outstanding for the period determined using the treasury-stock method. For purposes of this calculation, convertible preferred stock, convertible debt, warrants for preferred stock and stock options 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. Interest expense for convertible debt that is dilutive is added back to net income in the calculation of diluted net loss per share.

The shares outstanding at the end of the respective periods presented below, after giving effect for the 1-for-6.986 reverse stock split, were excluded from the calculation of diluted net loss per share due to their anti-dilutive effect:

	<u>December 31, 2013</u>	<u>December 31, 2012</u>
Convertible preferred stock . . . . .	—	3,431,865
Warrants for common stock . . . . .	277,690	277,690
Common shares under option . . . . .	1,401,101	211,500
Unvested restricted stock . . . . .	16,703	—
Convertible debt . . . . .	—	3,430,723
Total potential dilutive shares . . . . .	<u>1,695,494</u>	<u>7,351,778</u>

**Esperion Therapeutics, Inc.**  
**(A Development Stage Company)**  
**Notes to the Financial Statements (Continued)**

**17. Selected Quarterly Financial Data (Unaudited)**

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

	2013			
	March 31	June 30	September 30	December 31
<b>Operating expenses:</b>				
Research and development . . . . .	\$ 2,092,593	\$ 3,100,422	\$ 3,482,673	\$ 7,338,517
General and administrative . . . . .	1,251,419	1,171,425	1,924,150	2,397,499
Total operating expenses . . . . .	3,344,012	4,271,847	5,406,823	9,736,016
<b>Loss from operations:</b> . . . . .	<u>(3,344,012)</u>	<u>(4,271,847)</u>	<u>(5,406,823)</u>	<u>(9,736,016)</u>
Interest expense . . . . .	(828,223)	(108,357)	—	—
Change in fair value of warrant liability . . . . .	(41,958)	(2,544,907)	—	—
Other income, net . . . . .	(24,984)	4,035	168,389	46,570
Net loss . . . . .	<u>\$(4,239,177)</u>	<u>\$(6,921,076)</u>	<u>\$(5,238,434)</u>	<u>\$(9,689,446)</u>
Net loss per common share (basic and diluted) . . . . .	\$ (12.24)	\$ (19.82)	\$ (0.34)	\$ (0.63)
Weighted-average shares outstanding (basic and diluted) . . . . .	346,478	349,170	15,253,704	15,340,713
	2012			
	March 31	June 30	September 30	December 31
<b>Operating expenses:</b>				
Research and development . . . . .	\$ 1,557,211	\$ 2,330,223	\$ 2,456,412	\$ 1,654,282
General and administrative . . . . .	632,372	533,658	533,837	505,765
Total operating expenses . . . . .	2,189,583	2,863,881	2,990,249	2,160,047
<b>Loss from operations:</b> . . . . .	<u>(2,189,583)</u>	<u>(2,863,881)</u>	<u>(2,990,249)</u>	<u>(2,160,047)</u>
Interest expense . . . . .	(260,428)	(303,167)	(361,426)	(561,675)
Change in fair value of warrant liability . . . . .	—	—	—	32,367
Other income, net . . . . .	1,059	894	401	(86,001)
Net loss . . . . .	<u>\$(2,448,952)</u>	<u>\$(3,166,154)</u>	<u>\$(3,351,274)</u>	<u>\$(2,775,356)</u>
Net loss per common share (basic and diluted) . . . . .	\$ (7.96)	\$ (9.94)	\$ (10.31)	\$ (8.12)
Weighted-average shares outstanding (basic and diluted) . . . . .	307,742	318,654	325,023	341,935

**18. Subsequent Events**

On February 4, 2014, the Company entered into an operating lease agreement to lease a new office facility in Ann Arbor, MI. The term of this lease is for 63 months, commencing on April 1, 2014. The Company's facility lease provides for a fixed monthly rent for the term of the lease, with monthly rent increasing every 12 months subsequent to the first 3 months of the lease, and also provides for certain rent adjustments to be paid as determined by the landlord.



## Exhibit List

Exhibit No.	Exhibit Index
3.1	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)
3.2	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)
4.1	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)
4.2	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)
4.3	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)
4.4	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)
4.5	Registration Rights and Securityholder Agreement by and between the Registrant and Pfizer Inc. dated April 28, 2008 (incorporated by reference to Exhibit 4.6 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)
10.1*	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)
10.2	Lease by and between the Registrant and Michigan Life Science and Innovation Center LLC dated October 2, 2008 and amended on November 15, 2011 (incorporated by reference to Exhibit 10.10 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)
10.3	Second Amendment to Lease by and between the Registrant and the Michigan Land Bank Fast Track Authority dated August 26, 2013 (incorporated by reference to Exhibit 10.1 to the Registrant's Current Report on Form 8-K, File No. 001-35986, filed on August 27, 2013)
10.4	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)
10.5	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)
10.6	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)

Exhibit No.	Exhibit Index
10.7#	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)
10.8#	2013 Stock Option and Incentive Plan and forms of agreements thereunder (incorporated by reference to Exhibit 10.2 to the Registrant's Amendment No. 2 to the Registration Statement on Form S-1, File No. 333-188595, filed on June 12, 2013)
10.9#	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)
10.10#	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)
10.11#	Employment Agreement by and between the Registrant and Tim M. Mayleben dated December 3, 2012 (incorporated by reference to Exhibit 10.4 to the Registrant's Registration Statement on Form S-1, File No. 333-188595, filed on May 14, 2013)
10.12#	Transitional Services and Letter Agreement by and between Esperion Therapeutics, Inc. and Troy A. Ignelzi, dated August 8, 2013. (incorporated by reference to Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q for the Quarter ended June 30, 2013, File No, 001-35986, filed on August 12, 2013)
10.13#	Transitional Services and Letter Agreement by and between Esperion Therapeutics, Inc. and Noah L. Rosenberg, M.D., dated February 26, 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 28, 2014)
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 Principle 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 Principle 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.
(#)	Management contract or compensatory plan or arrangement.
(*)	Confidential treatment has been granted by the Securities and Exchange Commission as to certain portions.
(***)	Pursuant to Rule 406T of Regulation S-T, these interactive data files are deemed not filed or part of a registration statement or prospectus for purposes of Sections 11 or 12 of the Securities Act of 1933 or Section 18 of the Securities Exchange Act of 1934 and otherwise are not subject to liability under these sections.

**Consent of Independent Registered Public Accounting Firm**

We consent to the incorporation by reference in the 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 report dated March 13, 2014 with respect to the financial statements of Esperion Therapeutics, Inc. included in this Annual Report (Form 10-K) for the year ended December 31, 2013.

/s/ Ernst & Young LLP

Detroit, Michigan  
March 13, 2014

**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)) 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) 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
  - c) 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: March 13, 2014

/s/ TIM M. MAYLEBEN

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

**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, 2013 (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: March 13, 2014

/s/ TIM M. MAYLEBEN

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

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## ESPERION MANAGEMENT TEAM



**TIM MAYLEBEN**  
PRESIDENT AND  
CHIEF EXECUTIVE OFFICER



**MARIANNE ANDREACH**  
VICE PRESIDENT,  
STRATEGIC MARKETING AND  
PRODUCT PLANNING



**ROGER NEWTON, PHD, FAHA**  
EXECUTIVE CHAIRMAN AND  
CHIEF SCIENTIFIC OFFICER



**CAROL KARP**  
SENIOR VICE PRESIDENT,  
REGULATORY AFFAIRS  
AND COMPLIANCE

---

## BOARD OF DIRECTORS

**TIM MAYLEBEN**  
PRESIDENT AND  
CHIEF EXECUTIVE OFFICER

**ROGER NEWTON, PHD, FAHA**  
EXECUTIVE CHAIRMAN AND  
CHIEF SCIENTIFIC OFFICER

**PATRICK ENRIGHT**  
MANAGING DIRECTOR, LONGITUDE  
CAPITAL MANAGEMENT CO., LLC

**DOV GOLDSTEIN, MD**  
PARTNER, AISLING CAPITAL

**ANTONIO GOTTO, JR., MD, DPHIL**  
DEAN EMERITUS AND CO-CHAIR  
OF BOARD OF OVERSEERS, WEILL  
CORNELL MEDICAL COLLEGE

**DAN JANNEY**  
MANAGING DIRECTOR  
ALTA PARTNERS

**LOU LANGE, MD, PHD**  
PARTNER  
ASSET MANAGEMENT COMPANY

**MARK MCGOVERN, MD, FACC, FACP**  
FORMER EXECUTIVE VICE  
PRESIDENT, MEDICAL AFFAIRS  
AND CHIEF MEDICAL OFFICER  
KOS PHARMACEUTICALS

**NICOLE VITULLO**  
DIRECTOR  
DOMAIN ASSOCIATES, LLC

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General shareholder inquiries, including requests for the Company's Annual Report on Form 10-K, should be directed to:

### Investor Relations

Esperion Therapeutics, Inc.  
3891 Ranchero Drive, Suite 150  
Ann Arbor, MI 48108  
Phone: (734) 862-4840  
Email: [investorrelations@esperion.com](mailto:investorrelations@esperion.com)  
[investors.esperion.com](http://investors.esperion.com)

### Independent Registered Public Accounting Firm

Ernst & Young  
777 Woodward Ave  
Detroit, MI 48226  
Phone: (313) 628-7100

### General Counsel

Goodwin Procter LLP  
53 State Street  
Boston, MA 02109  
Phone: (617) 570-1000

### Registrar and Transfer Agent

Computershare  
250 Royall Street  
Canton, MA 02021  
Phone: (312) 360-5195



3891 Rancho Drive, Suite 150, Ann Arbor, MI 48108 | [www.esperion.com](http://www.esperion.com)  