Esperion Therapeutics, Inc.
Form 10-K
March 13, 2014

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UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2013

or

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

For the transition period from to
Commission file number: 001-35986

Esperion Therapeutics, Inc.

(Exact Name of Registrant as Specified in its Charter)

Delaware	**26-1870780**
(State or Other Jurisdiction of Incorporation or Organization)	(I.R.S. Employer Identification No.)
46701 Commerce Center Drive Plymouth, Michigan 48170	**48170**
(Address of Principal Executive Offices)	(Zip Code)

(743) 862-4840
(Registrant's Telephone Number, Including Area Code)

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

Title of each class	**Name of each exchange on which registered**
Common Stock, $0.001 par value	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 o No ý

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 o No ý

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

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

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

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

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

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

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.

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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.

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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.

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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

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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

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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

Patient Cardiovascular Disease Risk	LDL-C Goal
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 ≥ 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.

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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%	Skeletal muscle effects (e.g., myopathy and rhabdomyolysis) 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
Fixed combination therapies	Reduction in LDL-C	Up to 63%	Includes a statin as one of the underlying therapies and therefore contains the same side effects outlined above
Bile acid sequestrants	Reduction in LDL-C(1)	Up to 20%	Gastrointestinal disorders
Cholesterol absorption inhibitors	Reduction in LDL-C	Up to 18%	Limited
Niacin	Reduction in LDL-C; Reduction in recurrent myocardial infarction	Up to 17%	Flushing (i.e., warmth or redness) hepatic toxicity and skeletal muscle effects
Fibrates	Reduction in triglycerides and LDL-C	Up to 21%	Gallstones, skeletal muscle effects and liver disorders

(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.

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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.

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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.

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Completed Clinical Studies

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

Description	Title	Treatment Duration	Subjects Total	Subjects Treated
	Phase 2a Clinical Study of Safety and Pharmacokinetic Interaction in Patients with Hypercholesterolemia on a Background of Atorvastatin 10 mg			
ETC-1002-007	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 **Phase 2a Proof of Concept Clinical Study in Patients with Hypercholesterolemia and a History of Statin Intolerance**	8 Weeks	58	42
ETC-1002-006	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 **Phase 2a Proof of Concept Clinical Study in Patients with Hypercholesterolemia and Type 2 Diabetes**	8 Weeks	56	37
ETC-1002-005	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 **Phase 1b Multiple-Dose Tolerance Greater Than 120 mg Clinical Study**	4 Weeks	60	30
ETC-1002-004	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 **Phase 2a Proof of Concept Clinical Study in Hypercholesterolemic Patients**	2 Weeks	24	18
ETC-1002-003	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 **Phase 1b Multiple-Dose Tolerance Clinical Trial**	12 Weeks	177	133
ETC-1002-002	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 **Phase 1a Single-Dose Tolerance Clinical Trial**	2 Weeks / 4 Weeks	53	39
ETC-1002-001	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.

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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

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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.

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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

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

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

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.

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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 of 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.

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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

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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

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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

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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.

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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;

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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.

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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

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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.

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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

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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.

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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

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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,

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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.

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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

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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

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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;

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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;

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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

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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.

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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

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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

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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

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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

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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

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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

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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

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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.

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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

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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.

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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.

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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

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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.

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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

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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.

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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;

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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

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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.

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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 Ranchero 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

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PART II

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

Market Information

Our common stock 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.

Year Ended December 31, 2013	High	Low
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.

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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

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*
$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.

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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

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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. JMP 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.

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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)	
Balance Sheet Data:		
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)
	59	

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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;

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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

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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.

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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

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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

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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,		
	2013	2012	Change
	(in thousands)		
Operating Expenses:			
Research and development	$ 16,014	$ 7,998	$ 8,016
General and administrative	6,745	2,206	4,539
Loss from operations	(22,759)	(10,204)	(12,555)
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
Net loss	$ (26,088)	$ (11,742)	$ (14,346)

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.

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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,		
	2012	2011	Change
	(in thousands)		
Operating Expenses:			
Research and development	$ 7,998	$ 7,807	$ 191
General and administrative	2,206	2,357	(151)
Loss from operations	(10,204)	(10,164)	(40)
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)
Net loss	$ (11,742)	$ (10,817)	$ (925)

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.

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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 decreases 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.

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

	Year Ended December 31,	
	2013	2012
	(in thousands)	
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
Net increase (decrease) in cash and cash equivalents	$ 50,026	$ 4,940

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

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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:

	Total	Less than 1 Year	1 - 3 Years	3 - 5 Years	More than 5 Years
			(in thousands)		
Operating lease	$ 101	$ 101	$	$	$