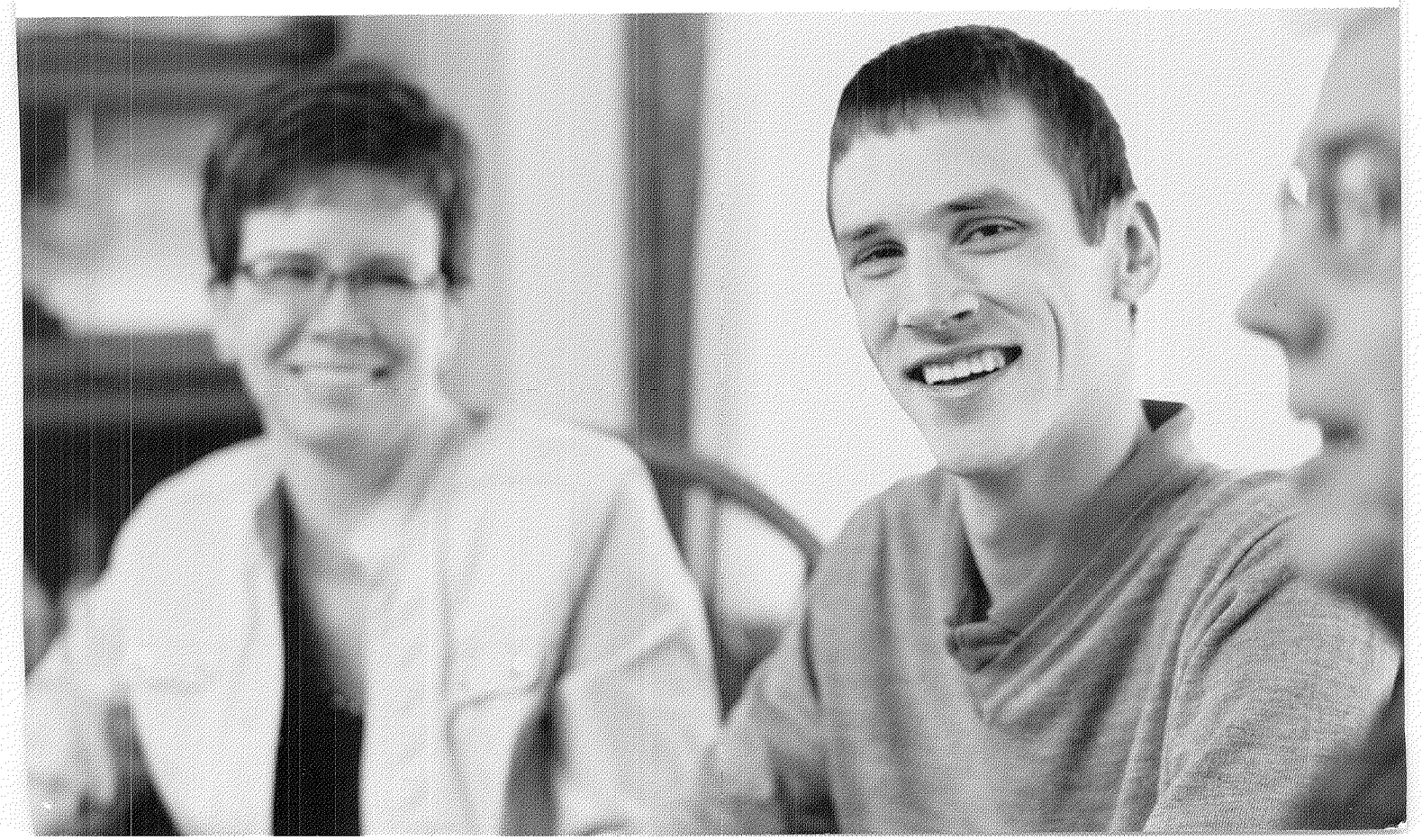
Aegerion®
Pharmaceuticals

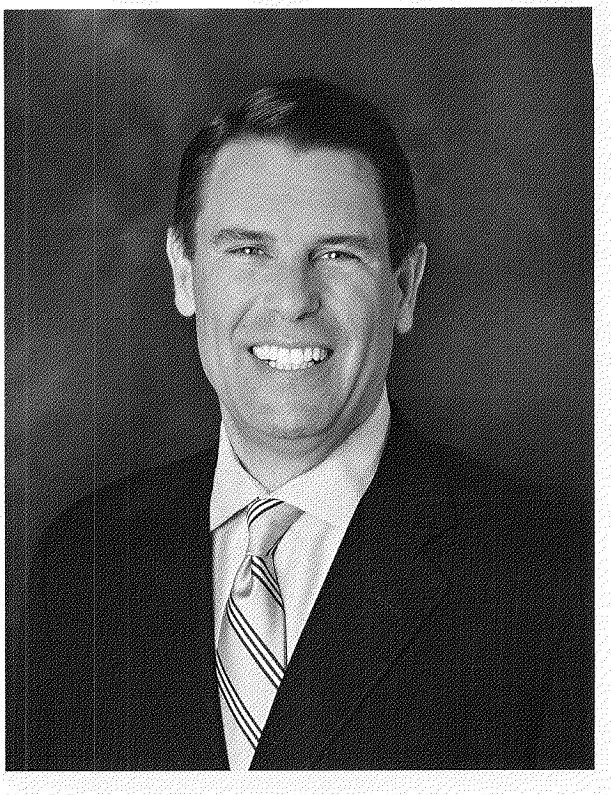
2012 Annual Report



Aegerion Pharmaceuticals is a biopharmaceutical company dedicated to the development and commercialization of innovative, life-altering therapies for patients with debilitating often fatal, rare diseases.

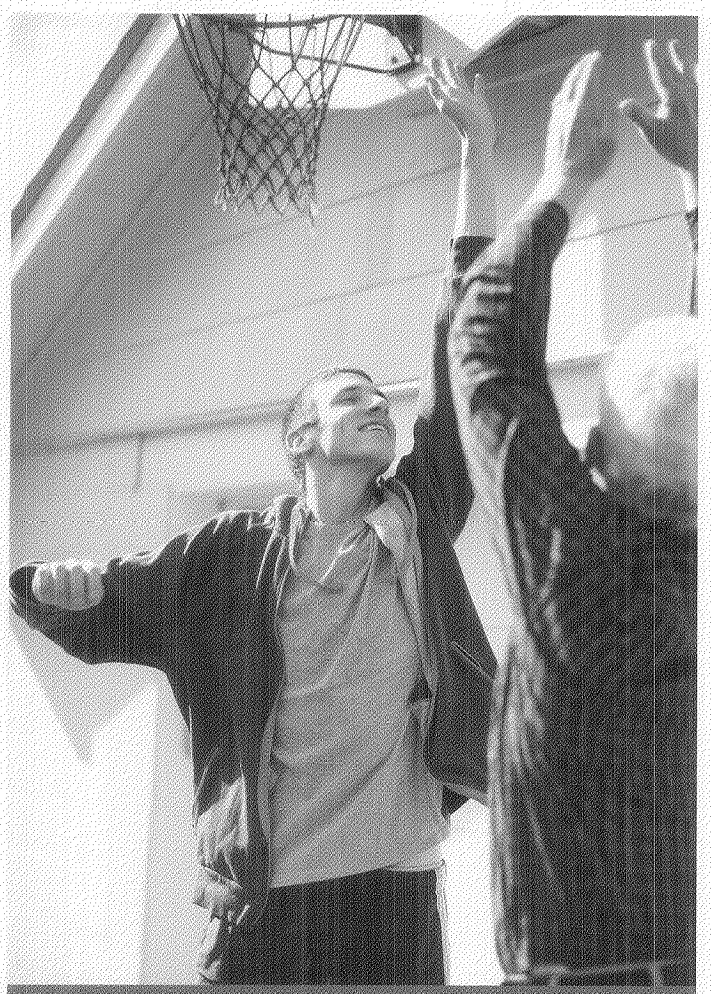
Dear Shareholders,

2012 was a transformational year for Aegerion and for homozygous familial hypercholesterolemia (HoFH) patients. We set a goal to obtain FDA approval for JUXTAPID™ (lomitapide) capsules in HoFH in the United States, and in late 2012 we achieved that goal through the extraordinary effort of the Aegerion team. We grew our organization from 30 employees at the beginning of 2012 to approximately 100 employees by year's end. Through the efforts of our extraordinary workforce, we achieved remarkable success in hitting each of the milestones we set out to accomplish in 2012, leaving us well prepared for the launch of JUXTAPID in the United States in January of this year.



Marc D. Beer *Chief Executive Officer*

Our mission from the beginning has been to deliver JUXTAPID to HoFH patients who have been waiting for a new treatment for this serious, life-threatening disease. 2012 began with an important step along that path: the submission of a New Drug Application to the U.S. Food and Drug Administration (FDA) and a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) within three business days of one another. While the regulatory team worked diligently to prepare those filings, and to respond to the requests from the FDA and EMA as they reviewed the applications, the commercial team was working in parallel to understand the market and the patients we sought to reach both in the U.S. and globally. In October, with extensive preparation, we executed an almost flawless FDA Advisory Committee meeting, resulting in a 13-2 vote in favor of approval, and leading ultimately to first-cycle approval of JUXTAPID in the U.S. in December. During 2012, the team worked diligently to ensure we were well prepared for launch, and well prepared to be able to reach HoFH patients as soon as possible after approval. Through these planning efforts and the high-level of execution of our pre-launch activities in 2012, our commercial launch of JUXTAPID in the U.S., which began in January of this year, has shown great early success. Most importantly, HoFH patients entered 2013 with renewed hope based on this treatment option.



Christian's Story

Diagnosed with HoFH at the age of two, Christian, now 21, has endured weekly apheresis treatments, has had five cardiac procedures and has been on up to eight medications at a time. More than just the physical burden of the disease, he has had to manage the emotional effects, both on himself and his family. At the age of 19, he sought to achieve a lifelong dream of becoming a pilot. After successfully passing his exam to begin training, he was informed that he was ineligible due to his condition. Now he has renewed hope for managing his high LDL-C levels, and he has become a great advocate for promoting awareness of HoFH.

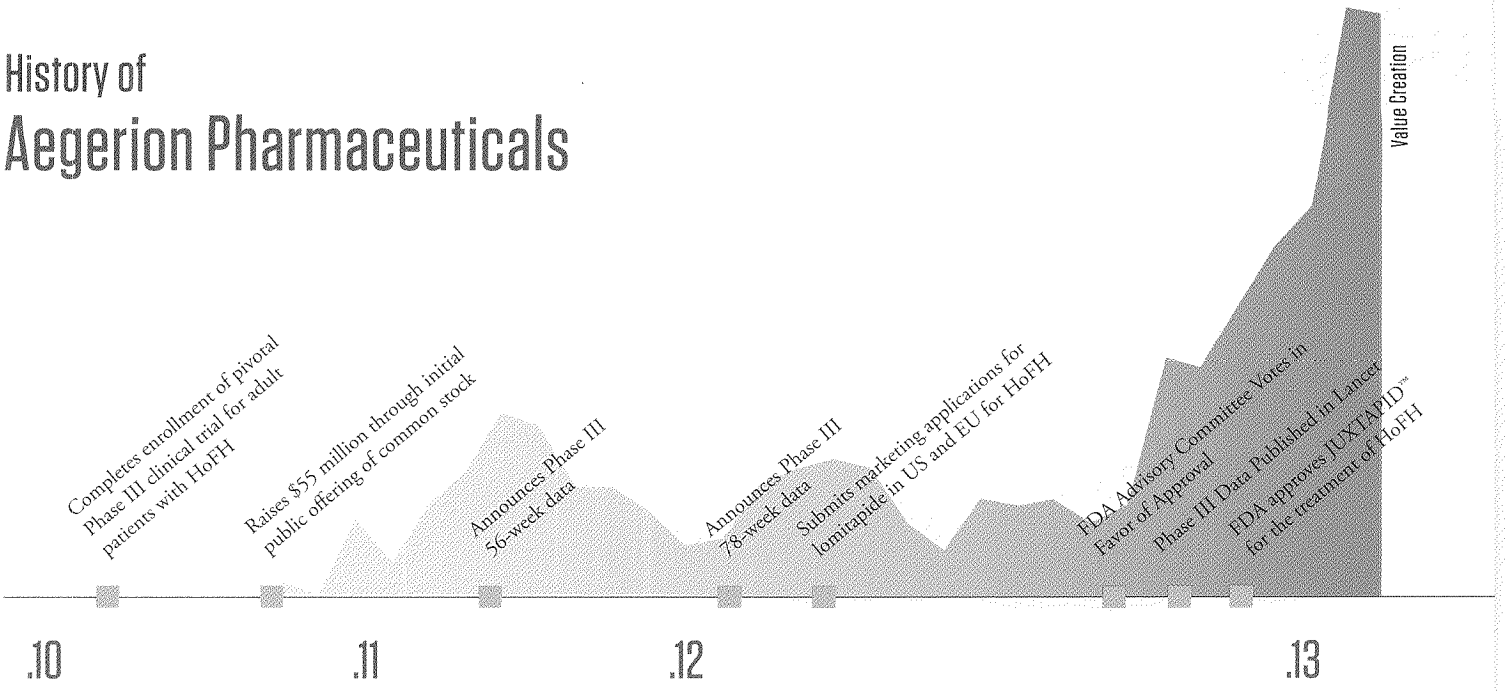
Our diligent, best-in-class preparation has positioned us well as we work to achieve our commercial and financial goals for 2013 and beyond. With the important milestone of FDA approval behind us, and the fruits of our commercial preparation appearing early in our U.S. launch, we move on to phase two of our plan which includes efforts to expand access to lomitapide for HoFH patients globally. This will be a multi-year, global initiative upon which we intend to remain keenly focused until our goals are met. The capable team we have established is motivated by the HoFH patients still waiting for treatment.

We continue to expect a mid-2013 decision by the EMA on our MAA filing for lomitapide, and we have begun the important commercial preparatory work in Europe as we did in the U.S. prior to launch. We have country managers in place in key markets building relationships with KOLs who are helping us understand the HoFH market in the EU. If lomitapide is approved in the EU, we will be prepared to pursue reimbursement on a country-by-country basis, positioning us well in our efforts to achieve our commercial and financial goals in 2014.

In 2012, we also initiated a pharmacokinetic and pharmacodynamic study of lomitapide in Japanese and Caucasian HoFH patients to further the work necessary to file for marketing authorization in Japan. This work will also include a small bridging study of lomitapide in Japanese HoFH patients, which we expect to be less than ten patients in size. As we have said in the past, Japan is an important market for lomitapide, and we have the potential to help a meaningful number of HoFH patients there.

We also plan to study JUXTAPID in pediatric HoFH patients who are in need of treatment. Managing this disease in patients from an early age is important, and we are highly focused on bringing JUXTAPID to the pediatric population. As part of our post-marketing commitments to the FDA, we have agreed to conduct a juvenile

History of
Aegerion Pharmaceuticals



toxicology study in rodents in 2013, and expect to start a clinical study in pediatric patients in 2014.

We anticipate filing for marketing approval of lomitapide in a number of additional countries outside the U.S. and EU, and are working diligently toward that end. We have now submitted filings in Canada and Brazil, and are optimistic about the potential to deliver JUXTAPID to HoFH patients in these markets. We are also supporting expanded access to JUXTAPID in countries such as Brazil where named patient supply or compassionate use can occur prior to approval in that country as a result of the FDA approval of JUXTAPID. Delivering JUXTAPID to HoFH patients in need globally is our goal. We are working to establish the infrastructure and relationships to achieve this objective.

An important ongoing initiative that we will continue to pursue in 2013 is increasing awareness of HoFH. Working with the FH Foundation and the National Lipid Association, among others, we hope to bring greater attention to this devastating rare disease, and to help patients and their physicians understand that there are now treatment options available.

Looking beyond 2013, we plan to focus on leveraging the dedicated and experienced Aegerion team we have established to pursue new opportunities. We have put in place a best-in-class organization of regulatory, medical affairs, commercial and operational experts. From that team arose a solid set of principles and processes upon which to build. In the future, we intend to seek product candidates targeted at life-threatening or substantially debilitating rare diseases; treatment for patients who, similar to HoFH patients, are in desperate need of new therapies. We have the infrastructure in place to support this initiative and Aegerion's growth.

Reflecting back to when I joined the company just prior to the initial public offering in October 2010, with under 10 people in the company and a product candidate still in Phase 3 development, I am proud of the success achieved in a short two and a half years. The milestones we accomplished in 2012 give me great confidence in our ability to continue this momentum. Fundamentally it comes down to our greatest asset, our people, and their dedication and drive to create value and succeed for the patients.

I thank you for your continued support of Aegerion. We look forward to updating you on our progress throughout the coming year.

Sincerely,

Marc D. Beer
Chief Executive Officer
April 23, 2013

What is HoFH?

HoFH is a serious, rare genetic disease that impairs the function of the receptor responsible for removing LDL-C ("bad" cholesterol) from the blood. A loss of low density lipoprotein receptor ("LDL-R") function results in extreme elevation of blood cholesterol levels. As a result of elevated levels of LDL-C, HoFH patients often develop premature and progressive atherosclerosis, a narrowing or blocking of the arteries, and are at very high risk of experiencing premature cardiovascular events, such as heart attack or stroke, often experiencing their first cardiovascular event in their twenties.

U.S. SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, DC 20549

FORM 10-K

(Mark One)

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

For the fiscal year ended December 31, 2012

☐ **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: 001-34921

AEGERION PHARMACEUTICALS, INC.

(Exact Name of Registrant as Specified in Its Charter)

Delaware	**20-2960116**
(State or Other Jurisdiction of Incorporation or Organization)	**(IRS Employer Identification Number)**

101 Main Street, Suite 1850, Cambridge, Massachusetts 02142
(Address of Principal Executive Offices, including Zip Code)

617-500-7867
(Registrant's telephone number, including area code)

SECURITIES REGISTERED PURSUANT TO SECTION 12(b) OF THE ACT:

Common Stock, $0.001 Par Value	**The NASDAQ Global Select Market**

SECURITIES REGISTERED PURSUANT TO SECTION 12(g) OF THE ACT:

None
(Title of Class)

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

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

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

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

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

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

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

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

The aggregate market value of the voting and non-voting common equity held by non-affiliates of the registrant as of June 29, 2012 was approximately $357,420,821, based upon the closing price on the NASDAQ Global Market reported for such date.

As of March 8, 2013, 28,810,589 shares of the registrant's common stock were outstanding.

DOCUMENTS INCORPORATED BY REFERENCE

Portions of the registrant's definitive Proxy Statement for its 2013 Annual Meeting of Shareholders are incorporated by reference into Part III of this Annual Report on Form 10-K

FORM 10-K

TABLE OF CONTENTS

PART I

PART II

PART III

PART IV

Forward-Looking Statements

All statements included or incorporated by reference into this Annual Report on Form 10-K, or Annual Report, other than statements or characterizations of historical fact, are forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Forward-looking statements are often identified by words such as "anticipates," "expects," "intends," "plans," "predicts," "believes," "seeks," "estimates," "forecasts," "may," "will," "should," "would," "could," "potential," "continue," "ongoing" and similar expressions, and variations or negatives of these words. Examples of forward-looking statements contained in this Annual Report include our statements regarding: the commercial potential for JUXTAPID™ (lomitapide) capsules, also referred to as lomitapide ("JUXTAPID"); our estimates as to the potential number of patients with a clinical or laboratory diagnosis consistent with homozygous familial hypercholesterolemia ("HoFH"); the possibility of named patient sales outside the United States ("U.S."); the potential for and possible timing of approval of lomitapide in the European Union ("EU") and other international markets; plans for further clinical development of JUXTAPID; our expectations regarding a possible future filing for approval in Japan; our plans for commercial marketing, sales, manufacturing and distribution; our expectations with respect to reimbursement of JUXTAPID in the U.S. and elsewhere; our expectations with respect to the impact of competition on our future operations and results; our beliefs with respect to our intellectual property portfolio and the extent to which it protects us; our expectations regarding the availability of data and marketing exclusivity in various countries; and our forecasts regarding our future expenses, our cash position and the timing of any future need for additional capital to fund operations.

The forward-looking statements contained in this Annual Report and in the documents incorporated into this Annual Report by reference are based on our current beliefs and assumptions with respect to future events, all of which are subject to change. Forward-looking statements are not guarantees of future performance, and are subject to risks, uncertainties and assumptions that are difficult to predict, including those discussed in "*Risk Factors*" in Part I, Item 1A of this Annual Report. It is not possible for us to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors may impact our operations or results. New risks may emerge from time to time. Past financial or operating performance is not necessarily a reliable indicator of future performance. Given these risks and uncertainties, we can give no assurances that any of the events anticipated by the forward-looking statements will occur or, if any of them does, what impact it will have on our results of operations and financial condition. Our actual results could differ materially and adversely from those expressed in any forward-looking statement in this Annual Report or in our other filings with the Securities and Exchange Commission ("SEC").

Except as required by law, we undertake no obligation to revise our forward-looking statements to reflect events or circumstances that arise after the date of this Annual Report or the respective dates of documents incorporated into this Annual Report by reference that include forward-looking statements. Thus, you should not assume that our silence over time means that actual events are bearing out as expressed or implied in these forward-looking statements.

In this Annual Report, "Aegerion Pharmaceuticals, Inc.," "Aegerion," the "Company," "we," "us" and "our" refer to Aegerion Pharmaceuticals, Inc. taken as a whole, unless otherwise noted.

PART I

Item 1. Business.

Overview

We are a biopharmaceutical company dedicated to the development and commercialization of novel, life-altering therapies for patients with debilitating, often fatal, rare diseases.

Our first product, JUXTAPID received marketing approval from the U.S. Food and Drug Administration ("FDA") on December 21, 2012, as an adjunct to a low-fat diet and other lipid-lowering treatments, including low-density lipoprotein ("LDL") apheresis, where available, to reduce low-density lipoprotein cholesterol ("LDL-C"), total cholesterol ("TC"), apolipoprotein B ("apo B") and non-high-density lipoprotein cholesterol ("non-HDL-C") in patients with homozygous familial hypercholesterolemia ("HoFH"). We launched JUXTAPID in the U.S. in late January 2013. In the first quarter of 2012, we submitted a Marketing Authorization Application ("MAA") to the European Medicines Agency ("EMA") requesting approval to market lomitapide as an adjunct to a low-fat diet and other lipid-lowering therapies, with or without apheresis, to reduce LDL-C, TC, apo B and triglycerides ("TG") in adults with HoFH.

We expect that our near-term efforts will be focused on:

- commercializing JUXTAPID as a treatment for HoFH in the U.S.;
- gaining regulatory approval of lomitapide for adult patients with HoFH in the EU and in other international markets, and launching lomitapide in those countries in which we receive marketing approval;
- supporting and facilitating expanded access to JUXTAPID in countries where named patient supply or compassionate use can occur as a result of the FDA approval of JUXTAPID;
- clinical development activities to support a potential marketing authorization application for lomitapide in HoFH in Japan; and
- activities in support of our planned clinical study of lomitapide in pediatric HoFH patients.

We also expect to build our business in the future by acquiring rights to one or more product candidates targeted at life-threatening or substantially debilitating rare diseases that leverage our infrastructure and expertise.

As of December 31, 2012, we had not generated revenue from the sale of any product. In the near-term, our ability to generate revenues is entirely dependent upon sales of JUXTAPID in the U.S. and in countries where JUXTAPID is available for sale on a named patient basis as a result of the approval of JUXTAPID in the U.S. As of December 31, 2012 we had an accumulated deficit of approximately $192.7 million and approximately $82.2 million in cash, cash equivalents and marketable securities. In January 2013, we sold 3,110,449 shares of our common stock in an underwritten public offering at a price to the public of $26.64 per share. The net proceeds to us from this offering were approximately $78.3 million after deducting underwriting discounts and commissions.

HoFH

HoFH is a serious, rare genetic disease that impairs the function of the receptor responsible for removing LDL-C ("bad" cholesterol) from the blood. A loss of low density lipoprotein receptor ("LDL-R") function results in extreme elevation of blood cholesterol levels.

Cholesterol is a naturally occurring molecule which is transported in the blood. The liver and the intestines are the two main sites where cholesterol is packaged and released within the body. The liver synthesizes cholesterol, and provides the body's intrinsic supply. The intestines are the conduit through which cholesterol

enters the body for metabolism. The delivery of cholesterol to peripheral cells in the body provides necessary sources of cellular energy and cell structure. However, excess levels of cholesterol in the blood, also known as hypercholesterolemia, can be the source of significant diseases in humans.

HoFH is most commonly caused by genetic mutations in both alleles of the LDL-R gene, but can also be caused by mutations in other genes. To date, greater than 1,600 mutations have been identified that can impair the functioning of the LDL-R, with some mutations leading to a total lack of LDL-R activity and others leading to severely reduced activity in LDL-R. Untreated HoFH patients typically have LDL-C levels in the range of 400 mg/dL to 1,000 mg/dL. As a result of elevated levels of LDL-C, HoFH patients often develop premature and progressive atherosclerosis, a narrowing or blocking of the arteries, and are at very high risk of experiencing premature cardiovascular events, such as heart attack or stroke, often experiencing their first cardiovascular event in their twenties. If untreated, patients with HoFH generally die before the age of 30.

There are no universally accepted criteria for the diagnosis of HoFH. Diagnosis is typically made clinically, using the following criteria:

- Assessment of cholesterol levels (TC or LDL-C);
- Physical examination for the presence of xanthomas; and
- Assessment of the family history of the patient.

Although not widely used, HoFH may also be diagnosed through an assessment of LDL-R function in cultured skin fibroblasts. Genetic testing may be performed to make a diagnosis of HoFH, but current genetic tests only detect approximately 80% of cases. Genetic testing is not widely available and is not routinely used if there are sufficient clinical findings and family history to make a clinical diagnosis of HoFH.

Physicians treating patients with hypercholesterolemia, including HoFH, are highly focused on lowering levels of LDL-C in their patients. In the U.S., for example, organizations such as the National Cholesterol Education Program ("NCEP"), the American Heart Association, and the American College of Cardiology have emphasized aggressive management of LDL-C. NCEP guidelines currently recommend that patients at high risk of experiencing a heart attack achieve LDL-C levels of 100 mg/dL or lower through lifestyle changes and drug therapy as appropriate based on their starting levels. Both the Canadian Cardiovascular Society and the Joint British Society have supported LDL-C treatment targets as low as 70mg/dL for high-risk patients. The clinical approach taken with HoFH patients has typically involved an aggressive treatment plan to reduce lipid levels as much as possible through dietary modifications and a combination of available lipid lowering drug therapies. Conventional drug therapies include statins, cholesterol absorption inhibitors and bile acid sequestrants. Less frequently, other drugs, such as niacin and fibrates, have been added to provide some incremental reductions in LDL-C levels, although these agents are typically used to modify lipids other than LDL-C. Because many of these therapies, including statins, act by increasing the activity of LDL-R, HoFH patients, given their defective LDL-R function, are often resistant, or refractory, to standard therapies. For example, high dose statin therapies that typically produce 46% to 55% reductions in LDL-C levels in the broad hypercholesterolemic patient population, on average, produce 14% to 30% reductions in patients with HoFH. Patients with HoFH who are unable to reach their recommended target LDL-C levels on drug therapy are sometimes treated using LDL apheresis in which cholesterol is removed from the body through mechanical filtration. Although levels of LDL-C are reduced acutely using apheresis, there is a rapid rebound. Because apheresis provides only temporary reductions in LDL-C levels, it must be repeated frequently, typically one or two times per month. In addition, apheresis is not readily available to all patients, particularly in the U.S. due to the limited number of treatment centers that perform this procedure.

JUXTAPID

Mechanism of Action

JUXTAPID is a small molecule microsomal triglyceride transfer protein, or MTP, inhibitor, or MTP-I. MTP exists in both the liver and intestines where it plays a role in the formation of cholesterol. Given the fact that MTP is involved in the formation of cholesterol-carrying lipoproteins from both liver-related, or hepatic, and intestinal sources, we believe the inhibition of MTP makes an attractive target for cholesterol-lowering therapy. JUXTAPID is the only MTP-I approved by the FDA for any indication.

United States

The FDA approved JUXTAPID on December 21, 2012, as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, TC, apo B and non-HDL-C in patients with HoFH. We launched JUXTAPID in the U.S. in late January 2013. The FDA has granted seven years of orphan drug exclusivity for JUXTAPID in the U.S. in the treatment of HoFH.

The safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH or in pediatric patients. The effect of JUXTAPID on cardiovascular morbidity and mortality has not been determined. The prescribing information for JUXTAPID contains a boxed warning citing the risk of hepatic toxicity. The boxed warning warns physicians that JUXTAPID can cause hepatotoxicity as manifested by elevations in transaminases and increases in hepatic fat, and recommends that physicians measure alanine aminotranferease ("ALT"), aspartate aminotransferase ("AST"), alkaline phosphatase, and total bilirubin before initiating treatment and then ALT and AST regularly during treatment.

Because of the risk of liver toxicity, JUXTAPID is available only through the approved Risk Evaluation and Mitigation Strategies ("REMS") program. We will certify all qualified healthcare providers who prescribe JUXTAPID and the pharmacies that will dispense the medicine. The goals of the REMS program are: to educate prescribers about the risk of hepatotoxicity associated with the use of JUXTAPID and the need to monitor patients during treatment with JUXTAPID as per product labeling; and to restrict access to therapy with JUXTAPID to patients with a clinical or laboratory diagnosis consistent with HoFH.

European Union

In the first quarter of 2012, we submitted an MAA to the EMA, requesting approval to market lomitapide in the EU as an adjunct to a low-fat diet and other lipid-lowering therapies, with or without apheresis, to reduce LDL-C, TC, apo B and TG in adults with HoFH. In March 2012, the EMA accepted the MAA for review with a review start date of March 21, 2012. We expect a decision on our MAA filing in mid-2013. Lomitapide does not have orphan drug designation for the treatment of HoFH in the EU since the EMA considers the relevant condition for orphan drug purposes to include both HoFH and heterozygous familial hypercholesterolemia ("HeFH").

Expanded Access

We plan to file for marketing approval of lomitapide in a number of additional countries outside the U.S. and EU. We also plan to make lomitapide available in certain countries that allow use of a drug, on a named patient basis or under a compassionate use or other type of so-called expanded access program, before it has obtained marketing approval in such countries. We plan to seek reimbursement for lomitapide for authorized pre-approval uses in some of these countries, to the extent permitted by applicable law and local regulatory authorities. In other countries or under certain circumstances, we may provide lomitapide free of charge for permitted pre-approval uses.

Other Development

During the fourth quarter of 2012, we initiated enrollment of patients into a Phase 1 study of the pharmacokinetic and pharmacodynamic properties of JUXTAPID in Japanese patients. Depending on the outcome of this study, we plan to conduct a therapeutic bridging study of JUXTAPID in Japanese HoFH patients in support of a planned filing for marketing authorization in Japan.

We also plan to conduct a clinical study of lomitapide in pediatric HoFH patients. The FDA has established a post-marketing requirement for us to conduct a juvenile toxicology study in rodents. The study will seek to ascertain the impact, if any, of JUXTAPID on growth and development prior to initiating the clinical study of lomitapide in pediatric patients. In 2011, the Paediatric Committee of the EMA ("PDCO") issued a positive opinion on our Pediatric Investigation Plan ("PIP") for lomitapide. This enabled us to file the MAA for lomitapide without pediatric data. The PDCO opinion requires that, prior to initiation of a pediatric study in the EU, the data on lomitapide generated in the adult HoFH population must be evaluated by the Committee for Medicinal Products for Human Use ("CHMP") and a positive conclusion on the benefit/risk balance and therapeutic benefit must be found. The pediatric study will then be reevaluated by the PDCO. In conjunction with our planning for a potential clinical study of lomitapide in pediatric patients, we are evaluating our formulation for lomitapide to determine whether any changes are necessary to facilitate administration to pediatric patients.

If, in the future, we elect to develop lomitapide for broader patient populations, such as for those patients with HeFH, who have severely elevated LDL-C levels despite current therapies, we would plan to do so selectively depending on, among other factors, the applicable indications, the related development costs and our available resources.

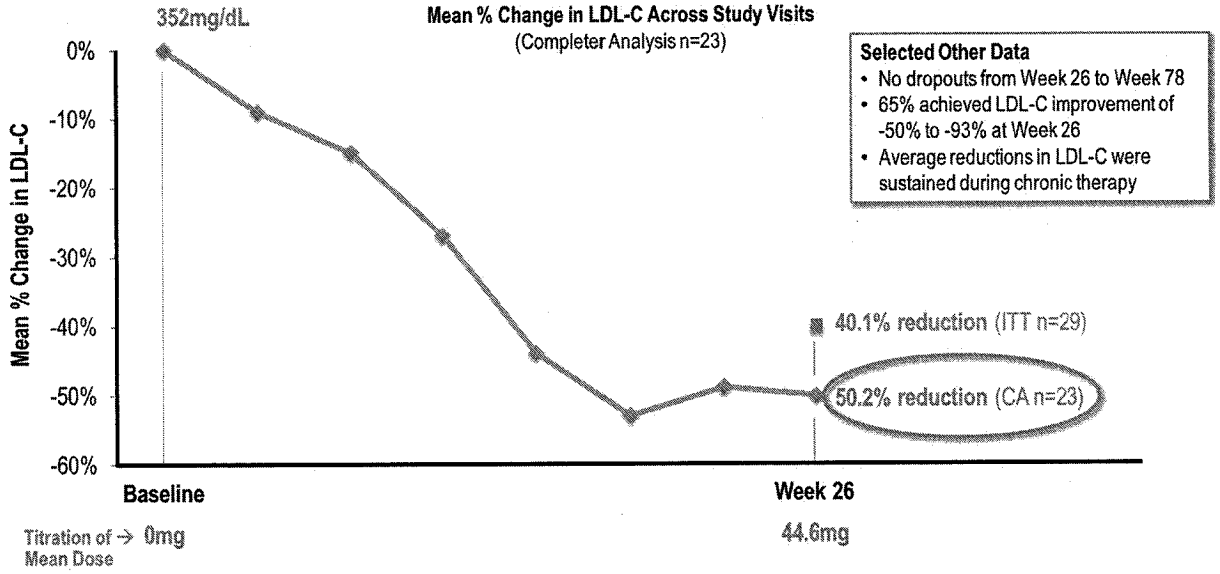
Patient Registry

We have made a post-marketing commitment to the FDA to conduct an observational cohort study, or registry study, in order to further understand JUXTAPID's long-term safety and effectiveness. The target enrollment for this study is 300 patients and patients will be enrolled globally, and followed by investigators in the study for a period of 10 years. The registry is voluntary, but patients who are treated with JUXTAPID will be encouraged to participate in the study.

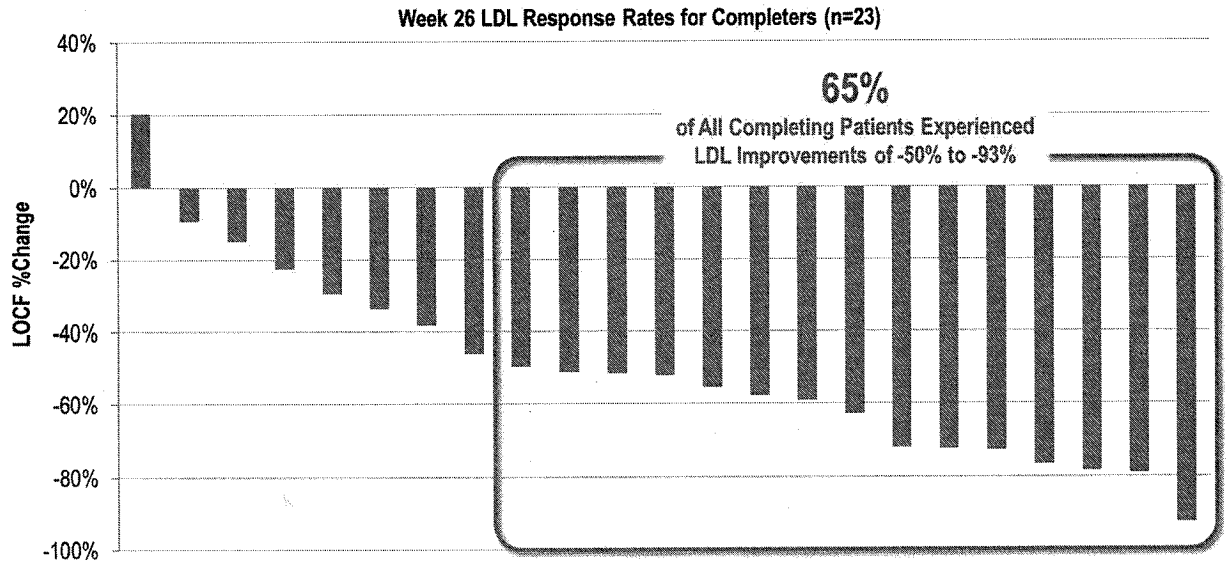
Phase 3 Clinical Study (HoFH)

The FDA based its approval of JUXTAPID on our Phase 3 clinical study, which evaluated the safety and effectiveness of JUXTAPID to reduce LDL-C levels in 29 adult patients with HoFH. The study was a multinational, single-arm, open-label, 78-week trial. The results of the study were published in the November 2, 2012 online version of the *Lancet*.

In the Phase 3 study, each patient's background lipid-lowering therapies were stabilized during a six-week run-in phase prior to dosing, and were maintained through at least the end of the 26-week efficacy phase. All patients received dietary counseling and were instructed to consume a diet containing <20% of energy from total dietary fat. JUXTAPID was initiated at a dose of 5 mg daily and gradually escalated to doses of 10 mg, 20 mg, 40 mg, up to 60 mg daily, based on tolerability and acceptable liver enzymes levels. As set forth in the table below, when added to the existing lipid-lowering therapy of the HoFH patients in the study, JUXTAPID reduced LDL-C by an average of 40% at Week 26 in the intent-to-treat population with last observation carried forward for the patients who discontinued prematurely; and reduced LDL-C by an average of 50% for the 23 patients who completed the study through Week 26.



As shown in the table below, approximately 65% of all patients completing the study experienced LDL-C reductions of 50% to 93% from their baseline as measured at the end of Week 26.



After Week 26, during the 52-week safety phase of the study, adjustments to concomitant lipid-lowering treatments were allowed. Average reductions in LDL-C were sustained during chronic therapy.

The most common adverse reactions in the Phase 3 study were gastrointestinal, reported by 27 (93%) of 29 patients. Adverse reactions, reported by greater than or equal to 8 patients (28%) in the HoFH clinical trial, included diarrhea, nausea, vomiting, dyspepsia and abdominal pain. Other common adverse reactions, reported by five to seven (17-24%) patients, included weight loss, abdominal discomfort, abdominal distension, constipation, flatulence, increased ALT chest pain, influenza, nasopharyngitis, and fatigue. Elevations in liver enzymes and hepatic (liver) fat were also observed. Ten of the 29 patients in the study had at least one elevation in liver enzymes greater than or equal to three times the upper limit of normal ("ULN"), including four patients who experienced liver enzymes greater than or equal to five times the ULN. During the clinical trial, liver enzyme elevations were managed through dose reduction or temporary discontinuation of dose. There were no clinically meaningful elevations of total bilirubin, international normalized ratio ("INR") or alkaline phosphatase, which are other markers of potential harmful effects on the liver. Hepatic fat increased from a baseline of 1% to a median absolute increase of 6% at 26 and 78 weeks.

Historical Clinical Development

Lomitapide has also been evaluated in 14 Phase 1 and eight Phase 2 clinical trials, including a Phase 2 trial in HoFH patients. Approximately 940 patients have been treated with lomitapide as part of the clinical development program.

Estimated Prevalence of HoFH

There is no patient registry or other method of establishing with precision the actual number of patients with HoFH in any geography. Medical literature has historically reported the prevalence rate of genotypic HoFH as one person in a million. However, we believe that the prevalence rate of HoFH is higher. The historically reported definition of HoFH used a narrow genotypic definition of HoFH. At the time the rate was first reported, many of the genetic mutations leading to defects in LDL-R function were not characterized, and some mutations remain uncharacterized even today. In addition, many physicians use a broader definition of HoFH that includes patients diagnosed through phenotypic criteria. In 2010, we commissioned an independent consultant in the healthcare industry to prepare a commercial assessment of the HoFH market for us. In its report, this consultant estimated that the total number of patients likely to seek treatment with symptoms, signs or laboratory findings consistent with HoFH in each of the U.S. and the EU is approximately 3,000 patients. This consultant's estimates, however, included a segment of severe HeFH patients whose levels of LDL-C are not controlled by current therapies. These patients may be phenotypically indistinct from HoFH patients. JUXTAPID is indicated solely for HoFH. Our prescribing information in the U.S. specifies that the safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH. We are not permitted to promote JUXTAPID for any indication other than HoFH. In addition, as part of the prescriber authorization form under our REMS program in the U.S., the prescriber must affirm that the patient has a clinical or laboratory diagnosis consistent with HoFH. We do not know how many patients will be determined to have a clinical or laboratory diagnosis consistent with HoFH, and there is no generally accepted and referenced definition of HoFH matching these criteria. However, rare diseases are often found to have a higher than expected prevalence rate once the first product available to treat the disease is introduced. We expect this may also be true for HoFH. As a result, we believe that, even if we exclude the patients who have a clinical phenotype consistent with HoFH, but as to whom the prescriber cannot conclude and affirm that the patient has a clinical or laboratory diagnosis consistent with HoFH, there still may be as many as 3,000 HoFH patients in the U.S. based on our belief that the base prevalence rate may be higher than our consultant estimated. There is no guarantee that our assumptions and beliefs are correct. The number of patients with HoFH in the U.S. could actually be significantly lower than we expect, and could be closer to the historically reported rates than to our estimate of 3,000 patients. Ultimately the actual size of the total addressable market in the U.S. will be determined only after we have substantial commercial history selling JUXTAPID and we are able to assess how it is being used

clinically. We believe that the prevalence rate in the EU is likely to be consistent with the prevalence rate in the U.S. However, the total addressable HoFH market in the EU will depend ultimately on whether the EMA requires a genetic diagnosis or imposes other narrow diagnosis criteria.

Commercialization

We believe that the key priorities for a successful commercial launch of JUXTAPID in the U.S. include:

- identifying patients with HoFH;
- educating and training healthcare providers about JUXTAPID;
- supporting access to and obtaining coverage for JUXTAPID; and
- providing patients with support services to help them maximize the benefits of and compliance with treatment while minimizing the risks and possible side effects.

Our commercial initiatives are designed to support these priorities. We believe that it will be possible to commercialize JUXTAPID in the U.S. with a relatively small specialty sales force. As a result, we have 25 sales representatives who are experienced in marketing drugs for the treatment of rare disorders. Our sales representatives are responsible for identifying patients with HoFH, and educating and training healthcare providers about JUXTAPID. We have also hired a small team of national account managers, who are primarily responsible for working with insurance plans, health maintenance organizations and other payers on securing reimbursement and formulary status for JUXTAPID. We have developed a comprehensive patient support program, which includes educational resources about JUXTAPID and HoFH; insurance verification and reimbursement support; nutritional support and counseling; monitoring and support of adherence; providing patients with options for seeking financial assistance from independent organizations for co-payments and other out-of-pocket payments for JUXTAPID treatment; and a free drug program for certain eligible uninsured and underinsured patients. We have a small team of customer care managers who are responsible for working with patients who are prescribed JUXTAPID and for coordinating our patient support services.

We believe our pricing for JUXTAPID in the U.S. is consistent with the level of pricing for other ultra-orphan drugs that treat diseases with a comparable prevalence rate as HoFH. Based on our interactions with payers and market research, we believe that payers in the U.S. will generally provide coverage for JUXTAPID. We believe that most payers will not make coverage decisions based on price differentials between available HoFH therapies. Based on our discussions with key payers in the U.S., we do not expect genotyping will routinely be required in the U.S. to determine a diagnosis of HoFH for reimbursement purposes, although there will be some exceptions. Since we expect most commercial payers to handle each patient who is prescribed JUXTAPID on a case-by-case basis, we estimate that, at least initially, the time from prescription to the patient starting treatment with JUXTAPID may take up to three or four months on average.

We also have an in-house marketing team that supports our commercialization efforts in the U.S, and permitted educational and disease awareness activities in the EU and other parts of the world. We have been engaged in dialogue with many of the specialists in the U.S., EU and elsewhere who treat patients with HoFH about the disease itself and the number of HoFH patients they may treat. We believe that these activities have provided us with a growing knowledge of the physicians as we commercialize JUXTAPID in the U.S. and prepare for a potential launch in the EU.

Outside the U.S., we plan to hire sales representatives to commercialize lomitapide, if approved, in certain key countries worldwide. In other countries, we will likely seek to engage a local distributor to commercialize lomitapide, if approved in that country.

Manufacturing, Supply and Distribution

JUXTAPID is a small molecule drug that is synthesized with readily available raw materials using conventional chemical processes. Hard gelatin capsules are prepared in 5 mg, 10 mg and 20 mg strengths.

Validation of the manufacturing process for JUXTAPID drug substance has been completed. With respect to JUXTAPID drug product, the FDA has permitted us to conduct concurrent validation of each strength of our JUXTAPID drug product which allows for the concurrent release and commercial distribution of each successful validation batch once completed. We are in the process of completing this concurrent validation work.

We currently have no manufacturing facilities and limited personnel with manufacturing experience. We rely on a contract manufacturer to produce drug substance for JUXTAPID and another contract manufacturer for drug product for our clinical trials and for commercial supplies. We have entered into long-term commercial supply agreements with each manufacturer. We intend to store our supply of JUXTAPID drug substance at multiple sites. We do not have any agreements or arrangements in place for redundant supply or a second source for JUXTAPID drug substance or drug product.

In the U.S., we distribute JUXTAPID through a specialty pharmacy that distributes JUXTAPID directly to patients and, under limited circumstances, to other purchasers. The specialty pharmacy does not take title to JUXTAPID. Title transfers upon delivery of JUXTAPID to the purchaser. The specialty pharmacy also provides certain patient program support services, accounts receivables and other order-to-cash services on our behalf. In the EU, if lomitapide is approved, we intend to deploy a similar distribution model under which a third party logistics provider will distribute the product to purchasers without taking title to the product. For named patient sales and other expanded access distribution, we intend to use a third party logistics provider to distribute JUXTAPID either directly to the purchaser in the applicable country or to our local third party distributor for such country.

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. All of these competitors currently engage in, have engaged in or may engage in the future in the development, manufacturing, marketing and commercialization of new pharmaceuticals, some of which may compete with JUXTAPID or other product candidates. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Key competitive factors affecting the commercial success of JUXTAPID and any other product candidates that we develop are likely to be efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement. The market for cholesterol-lowering therapeutics is large and competitive with many drug classes. JUXTAPID is approved in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, TC, apo B and non-HDL-C in HoFH patients. As a treatment for HoFH, JUXTAPID competes in the U.S. with Kynamro™ (mipomersen sodium) Injection ("Kynamro"), which is being commercialized by Genzyme Corporation ("Genzyme"), now part of Sanofi-Aventis ("Sanofi") under a collaboration with Isis Pharmaceuticals, Inc. ("Isis"). Kynamro is an antisense apolipoprotein B-100 inhibitor which is taken as a weekly subcutaneous injection. On January 29, 2013, the FDA approved Kynamro as an adjunct to lipid-lowering medications and diet to reduce LDL-C, apo B, TC, and non HDL-C in patients with HoFH. If Isis and Genzyme obtain marketing approval of Kynamro for the treatment of patients with HoFH in any country prior to us, they could obtain a competitive advantage associated with being the first to market.

We believe that JUXTAPID will face additional competition for the treatment of HoFH. Although there are no other MTP-I compounds currently approved by the FDA for the treatment of hyperlipidemia, there may be other MTP-I compounds in development. We are aware of other pharmaceutical companies that are developing product candidates that may compete with JUXTAPID in the treatment of HoFH, including Regeneron Pharmaceuticals,

Inc. ("Regeneron"), in collaboration with Sanofi, Roche Holding AG, Amgen Inc. ("Amgen") and Alnylam Pharmaceuticals, Inc. in collaboration with The Medicines Company, all of which are developing molecules that attempt to mimic the impact observed in patients with defects in their PCSK9 gene. Such patients have lower LDL-C levels and an observed reduction in cardiovascular events, and some believe that medicines that duplicate this behavior may effectively reduce LDL-C levels with a similar benefit. In 2011, Regeneron and Sanofi announced positive results from Phase 2 clinical trials of its anti-PCSK9 antibody in patients with HeFH and primary hypercholesterolemia. In July 2012, Regeneron and Sanofi announced commencement of patient enrollment for a 22,000 patient Phase 3 clinical program to evaluate its anti-PCSK9 antibody in several patient populations, including those with HeFH and primary hypercholesterolemia. Amgen is also conducting a clinical trial of its anti-PCSK9 antibody in multiple patient populations, including HoFH patients, with initial clinical data expected to be reported as early as this year. We expect that some HoFH patients who might otherwise be candidates for treatment with JUXTAPID will be committed to clinical studies of anti-PCSK9 antibodies. Given the rarity of HoFH, this may make it more difficult for us to generate revenues and achieve profitability. Regeneron and Sanofi have indicated that their PCSK-9 product could receive approval as a treatment for HeFH as early as 2015.

Many of our potential competitors have substantially greater financial, technical and human resources than we do, and significantly greater experience in the discovery and development of drug candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Accordingly, our competitors may be more successful than we may be in obtaining marketing approvals for drugs and achieving widespread market acceptance. Our competitors' drugs may be more effective or have a more favorable safety profile, or be more effectively marketed and sold, or be less expensive, than any drug we may commercialize, and may render JUXTAPID or any other product candidate that we develop obsolete or non-competitive before we can recover the expenses of developing and commercializing the product. 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 JUXTAPID or any other product candidate that we develop non-competitive or obsolete.

Intellectual Property

We plan to pursue patents, developed internally and licensed from third parties, and other means to protect our technology, inventions and improvements that are commercially important to our business. We also rely on trade secrets that may be important to our business.

Our success will depend significantly on our ability to:

- obtain and maintain patent and other proprietary protection for the technology, inventions and improvements we consider important to our business;
- defend our patents;
- preserve the confidentiality of our trade secrets; and
- operate without infringing the patents and proprietary rights of third parties.

Our JUXTAPID patent portfolio consists of five issued U.S. patents and issued patents in parts of Europe, Canada, Israel, Australia, New Zealand and Japan and pending applications in the U.S., Europe, Australia, Japan, Canada, India and South Korea, all of which have been licensed to us in a specific field. The U.S. patent covering the composition of matter of JUXTAPID is scheduled to expire in 2015. However, we have filed an application requesting a five-year patent term extension for this patent under the Drug Price Competition and Patent Term Restoration Act of 1984, referred to as the Hatch-Waxman Amendments. The non-U.S. patents directed to the composition of matter of JUXTAPID are scheduled to expire in 2016, but may also be eligible for extensions in certain countries. Our method of use patent covering certain dosing regimens for JUXTAPID expires in 2027 in the U.S., and in 2025 in the EU.

License

University of Pennsylvania

In May 2006, we entered into a license agreement with The Trustees of the University of Pennsylvania, ("UPenn") pursuant to which we obtained an exclusive, worldwide license from UPenn to certain know-how and a range of patent rights applicable to lomitapide. In particular, we obtained a license to certain patents and patent applications owned by UPenn relating to the dosing of MTP-I compounds, including lomitapide, and certain patents and patent applications and know-how covering the composition of matter of lomitapide that were assigned to UPenn by Bristol-Myers Squibb Company ("BMS") for use either as a monotherapy or with other dyslipidemic therapies to treat patients with HoFH . We also have the right to use lomitapide in the field of monotherapy or in combination with other dyslipidemic therapies for treatment of patients with other severe forms of hypercholesterolemia unable to come within 15% of NCEP's LDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe combined hyperlipidemia unable to come within 15% of NCEP's non-HDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe hypertriglyceridemia unable to reduce TG <1,000 on maximal tolerated therapy. We refer to the patents and patent applications assigned by BMS to UPenn and licensed to us by UPenn as the BMS-UPenn assigned patents.

To the extent that rights under the BMS-UPenn assigned patents were not licensed to us under our license agreement with UPenn or were retained by UPenn for non-commercial education and research purposes, those rights, other than with respect to lomitapide, were licensed by UPenn back to BMS on an exclusive basis pursuant to a technology donation agreement between UPenn and BMS. In the technology donation agreement, BMS agreed not to develop or commercialize any compound, including lomitapide, covered by the composition of matter patents included in the BMS-UPenn assigned patents in the field licensed to us exclusively by UPenn. Through our license with UPenn, as provided in the technology donation agreement, we have the exclusive right with respect to the BMS-UPenn assigned patents regarding their enforcement and prosecution in the field licensed exclusively to us by UPenn.

The license from UPenn covers, among other things, the development and commercialization of lomitapide alone or in combination with other active ingredients in the licensed field. The license is subject to customary non-commercial rights retained by UPenn for non-commercial educational and research purposes. We may grant sublicenses under the license, subject to certain limitations.

During 2012, we paid UPenn $50,000 upon filing the new drug application ("NDA") for JUXTAPID in the U.S. and at December 31, 2012, we accrued an additional $0.1 million due to UPenn for the approval of JUXTAPID in the U.S. We will be required to make development milestone payments to UPenn of up to an aggregate of approximately $2.6 million if we decide to develop JUXTAPID for indications within the licensed field other than HoFH, and we achieve certain milestones in such development efforts. All such development milestone payments for these other indications are payable only once, no matter how many licensed products for these other indications are developed. In addition, we will be required to make royalty payments in a range of levels not greater than 10% on net sales of JUXTAPID, and of any other products covered by the license (subject to a variety of customary reductions), and share with UPenn specified percentages of sublicensing royalties and other consideration that we receive under any sublicenses that we may grant.

This license agreement will remain in effect on a country-by-country basis until the expiration of the last-to-expire licensed patent right covering the product in the applicable country. We have the right to terminate this license agreement for convenience upon 60 days prior written notice to UPenn or for UPenn's uncured material breach of the license agreement. UPenn may terminate this license agreement for our uncured material breach of the license agreement, our uncured failure to make payments to UPenn or if we are the subject of specified bankruptcy or liquidation events.

Regulatory Matters

Government Regulation and Product Approval

Government authorities in the U.S., 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 and export and import of lomitapide and other products that we may develop. Our drugs must be approved by the FDA through the NDA process before they may be legally marketed in the U.S. and must be approved by foreign regulatory authorities via various procedures before they can be marketed in the applicable country.

U.S. Drug Development Process

In the U.S., the FDA regulates drugs under the Federal Food, Drug, and Cosmetic Act, ("FDCA") and implementing regulations. The process of obtaining marketing approvals and the subsequent 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 a company to administrative or judicial sanctions. These sanctions could include, among other things, the FDA's refusal to approve pending applications, withdrawal of an approval, imposition of a clinical hold, warning letters, product recalls, product seizures, total or partial suspension of production or distribution, injunctions, civil money penalties, fines, refusals of government contracts, debarment, restitution, disgorgement, or civil or criminal penalties. Any agency or judicial enforcement action could have a material adverse effect on us.

The process required by the FDA before a drug may be marketed in the U.S. generally involves the following:

- completion of preclinical laboratory tests, animal studies and formulation studies according to Good Laboratory Practices and other applicable regulations;
- submission to the FDA of an investigational new drug application, ("IND") which must become effective before human clinical trials may begin;
- performance of human clinical trials, including adequate and well-controlled trials, according to Good Clinical Practices ("GCP") to establish the safety and efficacy of the proposed drug for its intended use;
- submission to the FDA of an NDA;
- completion of registration batches and validation of the manufacturing process to show that we are capable of consistently producing quality batches of product;
- satisfactory completion of an FDA inspection of the manufacturing facility or facilities at which the drug is produced to assess compliance with current good manufacturing practice ("cGMP") to assure that the facilities, methods and controls are adequate to preserve the drug's identity, strength, quality and purity; 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, and for what indications they will be approved, if any.

Once a pharmaceutical candidate is identified for development it enters the preclinical testing stage. Preclinical tests include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies. An IND sponsor must submit the results of the preclinical tests, together with manufacturing information and analytical data, to the FDA as part of the IND. The sponsor will also include a protocol detailing, among other things, the objectives of the first phase of the clinical trial, the parameters to be used in monitoring safety,

and the effectiveness criteria to be evaluated, if the first phase lends itself to an efficacy evaluation. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA, within the 30-day time period, places the clinical trial on a clinical hold. 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 studies due to safety concerns or non-compliance.

All clinical trials must be conducted under the supervision of one or more qualified investigators. The conduct of clinical trials is subject to extensive regulation, including FDA's bioresearch monitoring regulations and GCP requirements, which establish standards for conducting, recording data from, and reporting the results of, clinical trials, and are intended to assure that the data and reported results are credible and accurate, and that the rights, safety, and well-being of study participants are protected. These regulations include the requirement that all research subjects provide informed consent. Further, an investigational review board ("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 trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the information regarding the trial and the consent form that must be provided to each trial subject or his or her legal representative, and must monitor the study until completed.

Each new clinical 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 study, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety.

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

- ***Phase 1.*** The drug 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 is often conducted in patients with the target diseases.
- ***Phase 2.*** This phase involves trials 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.
- ***Phase 3.*** This phase involves trials undertaken to further evaluate dosage, clinical efficacy and safety in an expanded patient population, often at geographically dispersed clinical trial sites. These trials are intended to establish the overall risk/benefit ratio of the product, and to provide an adequate basis for product labeling.

Progress reports detailing developments associated with the clinical testing program 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 or animal test results that suggest a significant risk to human subjects, such as carcinogenicity. 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 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. Sponsors of all controlled clinical trials, except for Phase 1 trials, are required to submit certain clinical trial information for inclusion in the public clinical trial registry and results data bank maintained by the National Institutes of Health.

Concurrent with clinical trials, companies usually complete additional animal studies, and must also develop additional information about the chemistry and physical characteristics of the drug, 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 drug. 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, preclinical studies and clinical trials, along with descriptions of the manufacturing process, analytical tests conducted on the chemistry of the drug, proposed labeling, and other relevant information are submitted to the FDA as part of an NDA requesting approval to market the product. The submission of an NDA generally is subject to the payment of a user fee, although NDAs for designated orphan drugs are exempt from this fee.

In addition, under the Pediatric Research Equity Act of 2007 ("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 drug is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any drug for an indication for which orphan designation has been granted.

The FDA conducts a preliminary review of 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 resubmitted with the additional information. The resubmitted 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. FDA is required to refer an NDA for a new chemical entity to an advisory committee for review, evaluation and recommendation as to whether the application should be approved and under what conditions, or explain why such review is not necessary. The FDA is not bound by the recommendation of an advisory committee, but it generally follows such recommendations. 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 or other data and information. Even if such data and information is submitted, the FDA may ultimately decide that the NDA does not satisfy the criteria for approval. Data obtained from clinical trials are not always conclusive; and the FDA may interpret data differently than we interpret the same data. The FDA may issue a complete response letter, which may require additional clinical or other data or impose other conditions that must be met in order to secure final approval of the NDA. 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 typically inspect the facility or facilities where the product is manufactured. In addition, FDA often will conduct a bioresearch monitoring inspection of the clinical trial sites involved in conducting pivotal studies to ensure data integrity and compliance with applicable GCP requirements.

NDAs receive either standard or priority review. A drug representing a major advance in treatment or treatment where no adequate therapy exists may receive priority review. Under the goals and policies agreed to by the FDA under the Prescription Drug User Fee Act ("PDUFA"), FDA has twelve months in which to complete its initial review of a standard NDA and respond to the applicant, and six months for a priority review NDA. FDA does not always meet its PDUFA goal dates and in certain circumstances the PDUFA goal date may be extended. In addition, products studied for their safety and effectiveness in treating serious or life-threatening illnesses and that provide meaningful therapeutic benefit over existing treatments, may receive accelerated approval, and may be approved on the basis of adequate and well-controlled clinical trials establishing that the drug product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on the basis of an effect on a clinical endpoint other than survival or irreversible morbidity. As a condition of approval, a sponsor of a drug receiving accelerated approval must perform post-marketing studies to validate the surrogate

endpoint or otherwise confirm the effect of the drug on a clinical endpoint, and the drug may be subject to accelerated withdrawal procedures. Priority review and accelerated approval do not change the standards for approval, but may expedite the approval process.

If a product receives marketing 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. In addition, the FDA may require post-approval commitments, including the completion of additional clinical studies. These are often referred to as Phase 4 or post-marketing studies, and may involve clinical trials 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 which have been commercialized. In addition, FDA may impose distribution and use restrictions and other limitations on labeling and communication activities with respect to an approved drug product via a REMS program, to mitigate serious risks, which could include medication guides, physician communication plans, or elements to assure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. Because of the risk of liver toxicity, JUXTAPID is available in the U.S. only through the REMS program under which we will certify all qualified healthcare providers who prescribe JUXTAPID and the pharmacies that will dispense the medicine. The goals of the REMS program are: to educate prescribers about the risk of hepatotoxicity associated with the use of JUXTAPID and the need to monitor patients during treatment with JUXTAPID as per product labeling; and to restrict access to therapy with JUXTAPID to patients with a clinical or laboratory diagnosis consistent with HoFH. We have also made a post-marketing commitment to the FDA to conduct an observational cohort study, or registry study, in order to further understand JUXTAPID's long-term safety and effectiveness. The target enrollment for this study is 300 patients and patients will be followed by investigators in the study for a period of 10 years. The registry is voluntary, but patients who are treated with JUXTAPID will be encouraged to participate in the study.

Patent Term Restoration and Marketing Exclusivity

The Drug Price Competition and Patent Term Restoration Act of 1984, or the Hatch-Waxman Act, establishes two abbreviated approval pathways for drug products that are in some way follow-on versions of already approved NDA products.

Generic Drugs. A generic version of an approved drug is approved by means of an Abbreviated New Drug Application ("ANDA") by which the sponsor demonstrates that the proposed product is the same as the approved, brand-name drug, which is referred to as the "reference listed drug," or RLD. Generally, an ANDA must contain data and information showing that the proposed generic product and RLD (1) have the same active ingredient, in the same strength and dosage form, to be delivered via the same route of administration, (2) are intended for the same uses, and (3) are bioequivalent. This is instead of independently demonstrating the proposed product's safety and effectiveness, which are inferred from the fact that the product is the same as the RLD, which the FDA previously found to be safe and effective.

505(b)(2) NDAs. If a product is similar, but not identical, to an already approved product, it may be submitted for approval via an NDA under FDC Act section 505(b)(2). Unlike an ANDA, the sponsor is permitted to rely to some degree on the FDA's finding that the RLD is safe and effective, and must submit its own product-specific data of safety and effectiveness to an extent necessary because of the differences between the products.

RLD Patents. An NDA sponsor must identify to the FDA any patents that claim the drug substance or drug product or a method of using the drug. When the drug is approved, those patents are among the information about the product that is listed in the FDA publication, *Approved Drug Products with Therapeutic Equivalence Evaluations*, which is referred to as the *Orange Book*. The sponsor of an ANDA or 505(b)(2) application seeking to rely on an approved product as the RLD must make one of several certifications regarding each listed patent. A "Paragraph III" certification is the sponsor's statement that it will wait for the patent to expire before obtaining approval for its product. A "Paragraph IV" certification is an assertion that the patent does not block approval of the later product, either because the patent is invalid or unenforceable or because the patent, even if valid, is not infringed by the new product.

Regulatory Exclusivities. The Hatch-Waxman Act provides periods of regulatory exclusivity for products that would serve as RLDs for an abbreviated new drug application, or ANDA, or 505(b)(2) application. If a drug is a new chemical entity ("NCE"), generally meaning that 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, there is a period of five years from the product's approval during which the FDA may not accept for filing any ANDA, or a 505(b)(2) application for a drug with the same active moiety. However, an ANDA or 505(b)(2) NDA may be submitted after four years if it contains a Paragraph IV certification of patent invalidity or non-infringement. According to the *Orange Book*, JUXTAPID has NCE exclusivity that will expire on December 21, 2017.

A product that is not an NCE may qualify for three years of marketing exclusivity if new clinical investigations, other than bioavailability studies, were conducted or sponsored by the applicant and are deemed by the FDA to be essential to the approval of the application, for example, for new indications, dosages, strengths or dosage forms of an existing drug. This exclusivity period does not preclude filing or review of an ANDA or 505(b)(2) application; rather, FDA is precluded from granting final approval to the ANDA or 505(b)(2) application until three years after approval of the RLD. In addition, this three-year exclusivity covers only the conditions of use associated with the new clinical investigations and, as a general matter, does not prohibit the FDA from approving ANDAs or 505(b)(2) NDAs for generic versions of the original, unmodified drug product.

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 preclinical studies and adequate and well-controlled clinical trials necessary to demonstrate safety and effectiveness.

Patent Term Restoration. The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. The maximum period of restoration is five years, and the patent 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 extension must be applied for prior to expiration of the patent and within 60 days of the NDA approval. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. Our U.S. composition of matter patent for JUXTAPID is scheduled to expire in 2015, and we have filed an application for five-year patent term extension for this patent.

In the EU, certain patents may qualify for a supplemental protection certificate that would extend patent protection for up to five years after patent expiration upon marketing approval in the EU.

In the EU, new chemical entities qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the EU from assessing a generic application for eight years, after which generic marketing authorization can be submitted, but a generic may not be marketed for two years.

Pediatric exclusivity is another type of exclusivity available in the U.S. Pediatric exclusivity, if granted, provides an additional six months to an existing exclusivity period, including orphan drug exclusivity, or statutory delay in approval resulting from certain patent certifications. 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 trial or trials and submission of pediatric data that fairly responds to an FDA-issued "Written Request" for such a trial or trials. The data need not show the product to be effective in the pediatric population studied; rather, if the clinical trial is deemed to fairly respond to the FDA's request, the additional protection is granted. If reports of requested pediatric studies are submitted to and accepted by FDA within the statutory time

limits, whatever statutory or regulatory periods of exclusivity or Orange Book listed patent protection cover the drug are extended by six months. This is not a patent term extension, but it effectively extends the regulatory period during which the FDA cannot approve an ANDA or 505(b)(2) application owing to regulatory exclusivity or listed patents. As noted above, we plan to seek pediatric exclusivity for JUXTAPID. The current pediatric exclusivity provision was permanently reauthorized on July 9, 2012 under the Food and Drug Administration Safety and Innovation Act of 2012.

Orphan Drug Designation

Under the Orphan Drug Act, the FDA may grant orphan drug designation to a drug intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the U.S., or for which there is no reasonable expectation that the cost of developing and making available in the U.S. a drug for this type of disease or condition will be recovered from sales in the U.S. for that drug. Orphan drug designation must be requested before submitting an NDA. After the FDA grants orphan drug designation, the identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications to market the same drug for the same indication, except in very limited circumstances, for seven years. Orphan drug exclusivity, however, also could block the approval of one of our products for seven years if a competitor obtains approval of the same drug, as defined by the FDA, or if our product candidate is determined to be contained within the competitor's product for the same indication or disease. If a drug designated as an orphan drug receives marketing approval for an indication broader than what is designated, it may not be entitled to orphan drug exclusivity. The FDA has granted seven years of orphan drug exclusivity for JUXTAPID in the treatment of HoFH.

The EMA grants orphan drug designation to promote the development of products that may offer therapeutic benefits for life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the EU. In addition, orphan drug designation can be granted if the drug is intended for a life threatening, seriously debilitating or serious and chronic condition, and if without incentives it is unlikely that sales of the drug in the EU would be sufficient to justify developing the drug. Orphan drug designation is only available if there is no other satisfactory method approved in the EU of diagnosing, preventing or treating the condition, or if such a method exists, the proposed orphan drug will be of significant benefit to patients. Orphan drug designation provides 10 years of market exclusivity following drug approval. The exclusivity period may be reduced to six years if the designation criteria are no longer met, including where it is shown that the product is sufficiently profitable not to justify maintenance of market exclusivity. Despite the prevalence rate, lomitapide will not have orphan drug exclusivity in the EU for the treatment of HoFH because the EMA considers the relevant condition, for orphan drug purposes, to include both HoFH and HeFH.

Post-Approval Requirements

Once an approval is granted, products are subject to continuing regulation by the FDA. The FDA may withdraw the approval, following notice and an opportunity for a hearing, if, among other things, compliance with certain regulatory standards is not maintained or if new information indicates that the drug is not safe or effective. 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. If new safety issues are identified following approval, the FDA can require the NDA sponsor to revise the approved labeling to reflect the new safety information; conduct post-market studies or clinical trials to assess the new safety information; and implement a REMS program to mitigate newly-identified risks. After approval, most changes to the approved product, such as adding new indications, manufacturing changes and additional labeling claims, are subject to prior FDA review and approval. Drug manufacturers and other entities involved in the manufacture 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 requirements and other laws.

Companies engaged in manufacturing drug products or their components must comply with applicable cGMP requirements and product-specific regulations enforced by the FDA and other regulatory agencies. Compliance with cGMP includes adhering to requirements relating to organization of personnel, buildings and facilities, equipment, control of components and drug product containers and closures, production and process controls, packaging and labeling controls, holding and distribution, laboratory controls, and records and reports. The FDA regulates and inspects equipment, facilities, and processes used in manufacturing pharmaceutical products prior to approval. If, after receiving approval, a company makes a material change in manufacturing equipment, location, or process (all of which are, to some degree, incorporated in the NDA or BLA), additional regulatory review and approval may be required.

The FDA also conducts regular, periodic visits to re-inspect equipment, facilities, and processes following the initial approval of a product. Failure to comply with applicable cGMP requirements and conditions of product approval may lead the FDA to seek sanctions, including fines, civil penalties, injunctions, suspension of manufacturing operations, operating restrictions, withdrawal of FDA approval, seizure or recall of products, and criminal prosecution.

In addition, any drug products manufactured or distributed by us pursuant to FDA approvals are subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the drug, providing the FDA with updated safety and efficacy information, drug sampling and distribution requirements, complying with certain electronic records and signature requirements, and complying with FDA promotion and advertising requirements. 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 labeling. FDA may impose significant civil and monetary penalties for the dissemination of false or misleading direct-to-consumer advertisements. Manufacturers of approved drug products also are subject to annual establishment and product user fees. In addition, FDA approvals of drug product may be conditioned upon certain post-marketing commitments. For example, as part of the FDA approval of JUXTAPID, we are required to conduct an observational cohort study to generate more data on the long-term safety profile of JUXTAPID, the patterns of use and compliance and the long-term effectiveness of controlling LDL-C levels. The patient registry study will have a target enrollment of 300 HoFH patients worldwide. In the study, investigators will follow each patient for a period of 10 years to track malignancies, teratogenicity and hepatic effects. We also have a post-marketing commitment to the FDA to conduct a juvenile toxicology study in rodents prior to initiating a clinical study of JUXTAPID in pediatric HoFH patients. The juvenile animal toxicology study will seek to ascertain the impact, if any, of JUXTAPID on growth and development.

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

Foreign Regulation

In addition to regulations in the U.S., our business will be subject to a variety of foreign regulations governing clinical trials and commercial sales and distribution of our products. 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 trials or marketing of the product in those countries. For example, in the EU, a clinical trial application ("CTA") must be submitted to each member state's national health authority and an independent ethics committee prior to commencement of clinical trials of a product. The CTA must be approved by both the national health authority and the independent ethics committee prior to the commencement of a clinical trial in the member state. The approval process varies from country to country, and the time may be

longer or shorter than that required for FDA approval. In addition, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary greatly from country to country. In all cases, clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain marketing approval of a drug under EU regulatory systems, we may submit marketing authorization applications either under a centralized or decentralized procedure. The centralized procedure provides for the grant of a single marketing authorization that is valid for all EU member states. The centralized procedure is compulsory for medicines produced by certain biotechnological processes, products designated as orphan medicinal products, and products with a new active substance indicated for the treatment of certain diseases, and optional for those products that are highly innovative or for which a centralized process is in the interest of patients. Under the centralized procedure in the EU, the maximum timeframe for the evaluation of a marketing authorization application is 210 days (excluding clock stops, when additional written or oral information is to be provided by the applicant in response to questions asked by the Scientific Advice Working Party of the CHMP). Accelerated evaluation might be granted by the CHMP in exceptional cases, when a medicinal product is expected to be of a major public health interest, defined by three cumulative criteria: the seriousness of the disease, such as heavy disabling or life-threatening diseases, to be treated; the absence or insufficiency of an appropriate alternative therapeutic approach; and anticipation of high therapeutic benefit. In this circumstance, the EMA ensures that the opinion of the CHMP is given within 150 days.

The decentralized procedure provides for approval by one or more other, or concerned, member states of an assessment of an application performed by one member state, known as the reference member state. Under this procedure, an applicant submits an application, or dossier, and related materials, including a draft summary of product characteristics, and draft labeling and package leaflet, to the reference member state and concerned member states. The reference member state prepares a draft assessment and drafts of the related materials within 120 days after receipt of a valid application. Within 90 days of receiving the reference member state's assessment report, each concerned member state must decide whether to approve the assessment report and related materials. If a member state cannot approve the assessment report and related materials on the grounds of potential serious risk to public health, the disputed points may eventually be referred to the European Commission, whose decision is binding on all member states.

Expanded Access

In certain countries, drug products approved in the U.S. or EU can be accessed by patients before the drug has obtained marketing approval in such country. There are various forms of this access. They include the actual purchase of product by the purchaser, which is often times the government, and providing the product on a compassionate use basis. Each such country has its own laws and regulations that apply to these forms of access and the extent and nature of such laws and regulations vary by country. We are planning to make JUXTAPID available in certain countries that allow use of a drug before it has obtained marketing approval in such countries and, in doing so, will need to comply with all such applicable laws and regulations.

Pharmaceutical Coverage, Pricing and Reimbursement

Sales of pharmaceutical products depend in significant part on the availability of third-party reimbursement. Third-party payers include government health administrative authorities, including at the federal and state level, managed care providers, private health insurers and other organizations. Third-party payers are increasingly challenging the price and examining the cost-effectiveness of medical products and services.

In the U.S., the Medicare program is administered by the Centers for Medicare & Medicaid Services ("CMS"). Coverage and reimbursement for products and services under Medicare are determined in accordance with the Social Security Act and pursuant to regulations promulgated by CMS, as well as the agency's subregulatory coverage and reimbursement determinations. Under Part D, Medicare beneficiaries may enroll in

prescription drug plans offered by private entities which will provide coverage of outpatient prescription drugs. Part D plans include both stand-alone prescription drug benefit plans and prescription drug coverage as a supplement to Medicare Advantage plans. Unlike Medicare Part A and B, Part D coverage and reimbursement are not standardized. Part D prescription drug plan sponsors are not required to pay for all covered Part D drugs, and each drug plan can develop its own drug formulary that identifies which drugs it will cover and at what tier or level. However, Part D prescription drug formularies must include drugs within each therapeutic category and class of covered Part D drugs, though not necessarily all the drugs in each category or class. Any formulary used by a Part D prescription drug plan must be developed and reviewed by a pharmacy and therapeutic committee.

Government payment for some of the costs of prescription drugs may increase demand for products for which we receive marketing approval. However, any negotiated prices for our products covered by a Part D prescription drug plan will likely be lower than the prices we might otherwise obtain. Moreover, while the Part D benefit applies only to drug benefits for Medicare beneficiaries, private payers often follow Medicare coverage policy and payment limitations in setting their own payment rates.

In general, state Medicaid programs are required to cover drugs and biologicals of manufacturers that have entered into a Medicaid Drug Rebate Agreement, as discussed below, although such drugs and biologicals may be subject to prior authorization or other utilization controls. Private payers have their own processes for determining whether or not a drug or biological will be covered, often based on the available medical literature.

Beginning April 1, 2013, Medicare payments for all items and services under Part A and B, including drugs and biologicals, and payments to plans under Medicare Part D, will be reduced by up to 2% under the sequestration required by the Budget Control Act of 2011, Pub. L. No. 112-25 ("BCA"), as amended by the American Taxpayer Relief Act of 2012, Pub. L. 112-240 ("ATRA"), unless Congress acts to prevent the cuts. If these cuts are implemented, Part D plans could seek to reduce their negotiated prices for drugs. If Congress enacts legislation to prevent sequestration, it could enact different cuts to Medicare payments for items and services and to plans.

Third-party payers other than Medicare have a variety of methodologies for paying for drugs and biologicals. Payers also are increasingly considering new metrics as the basis for reimbursement rates, such as average sales price ("ASP"), average manufacturer price ("AMP") or actual acquisition cost ("AAC"). The existing data for reimbursement based on these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement mechanics on the willingness of payers to cover JUXTAPID, and the amount of coverage.

We participate in the Medicaid Drug Rebate Program, established by the Omnibus Budget Reconciliation Act of 1990 and amended by the Veterans Health Care Act of 1992 as well as subsequent legislation. We may also participate in and have certain price reporting obligations to state Medicaid supplemental rebate programs and other governmental pricing programs, Under the Medicaid Drug Rebate Program, we are required to pay a rebate to each state Medicaid program for our covered outpatient drugs that are dispensed to Medicaid beneficiaries and paid for by a state Medicaid program as a condition of having federal funds being made available to the states for our drugs under Medicaid and Medicare Part B. Those rebates are based on pricing data that we report on a monthly and quarterly basis to CMS, the federal agency that administers the Medicaid Drug Rebate Program. These data include the AMP and, in the case of innovator products, such as JUXTAPID, the best price for each drug.

Federal law requires that any company that participates in the Medicaid Drug Rebate Program also participate in the Public Health Service's 340B drug pricing discount program in order for federal funds to be available for the manufacturer's drugs under Medicaid and Medicare Part B. The 340B pricing program requires participating manufacturers to agree to charge statutorily-defined covered entities no more than the 340B "ceiling price" for the manufacturer's covered outpatient drugs.

The 340B ceiling price is calculated using a statutory formula, which is based on the AMP and rebate amount for the covered outpatient drug as calculated under the Medicaid Drug Rebate Program. Changes to the definition of AMP and the Medicaid rebate under the federal Patient Protection and Affordable Care Act ("PPACA") and CMS's issuance of final regulations implementing those changes also could affect our 340B ceiling price calculations and negatively impact our results of operations once we begin to participate in the 340B program.

In order to be eligible to have our products paid for with federal funds under the Medicaid and Medicare Part B programs and purchased by four federal agencies (noted below) and certain federal grantees, we anticipate that we will participate in the Department of Veterans Affairs ("VA") Federal Supply Schedule ("FSS") pricing program, established by Section 603 of the Veterans Health Care Act of 1992. Under this program, we will be obligated to make our product available for procurement on an FSS contract and charge a price to four federal agencies—VA, Department of Defense, Public Health Service, and Coast Guard—that is no higher than the statutory Federal Ceiling Price ("FCP"). The FCP is based on the non-federal average manufacturer price ("Non-FAMP"), which we will be required to calculate and report to the VA on a quarterly and annual basis.

The cost of pharmaceuticals continues to generate substantial governmental and third-party payer interest. We expect that the pharmaceutical industry will experience pricing pressures due to the trend toward managed healthcare, the increasing influence of managed care organizations, and additional legislative proposals. Indeed, we expect that there will continue to be a number of federal and state proposals to implement governmental pricing controls and limit the growth of healthcare costs, including the cost of prescription drugs. At the present time, Medicare is prohibited from negotiating directly with pharmaceutical companies for drugs. However, Congress could pass legislation that would lift the ban on federal negotiations. While we cannot predict whether such legislative or regulatory proposals will be adopted, the adoption of such proposals could have a material adverse effect on our business, financial condition and profitability.

Some third-party payers also require pre-approval of coverage for new or innovative drug therapies before they will reimburse healthcare providers or patients that use such therapies. While we cannot predict whether any cost-containment measures will be adopted or otherwise implemented in the future, the announcement or adoption of these measures could have a material adverse effect on our ability to obtain adequate prices for our products and to operate profitably.

In addition, in some foreign countries, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement, and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. In some countries, pricing negotiations with governmental authorities can take six to 12 months or longer after the receipt of marketing approval. To obtain reimbursement or pricing approval in some countries, we may be required to conduct a clinical trial that compares the cost-effectiveness of our product candidate to other available therapies. There can be no assurance that any country that has price controls or reimbursement limitations for pharmaceutical products will allow favorable reimbursement and pricing arrangements for any of our products.

United States Healthcare Reform

In addition, the U.S. Congress and state legislatures from time to time propose and adopt initiatives aimed at cost containment, which could impact our ability to sell our products profitably. For example, in March 2010, President Obama signed into law the PPACA, as amended by the Health Care and Education Reconciliation Act of 2010 and subsequent legislation, referred herein as PPACA or the Healthcare Reform Act. The Healthcare Reform Act contains a number of provisions that are expected to impact our business and operations, in some cases in ways we cannot currently predict. Changes that may affect our business in the U.S. include those

governing expanded enrollment in federal and private healthcare programs, new Medicare reimbursement methods and rates, increased rebates and taxes on pharmaceutical products, and revised fraud and abuse and enforcement requirements. These changes will impact existing government healthcare programs and will result in the development of new programs. Additional provisions of the Healthcare Reform Act, some of which have already taken effect, may negatively affect our future revenues. For example, as part of the Healthcare Reform Act's provisions closing a funding gap that currently exists in the Medicare Part D prescription drug program (commonly known as the "donut hole"), manufacturers are required to provide a 50% discount on branded prescription drugs dispensed to beneficiaries within this donut hole. The Healthcare Reform Act also makes changes to the Medicaid Drug Rebate Program, including revising the definition of AMP and increasing the minimum rebate from 15.1% to 23.1% of the AMP, for most innovator products and from 11% to 13% for non-innovator products. The increased minimum rebate of 23.1% applies to JUXTAPID. Further, the new law imposes a significant annual fee on companies that manufacture or import branded prescription drug products. We do not know the full effects that the Healthcare Reform Act will have on our commercialization efforts.

Many of the Healthcare Reform Act's most significant reforms do not take effect until 2014 and thereafter, and their details will be shaped significantly by implementing regulations that have yet to be finalized. In 2012, the Supreme Court of the United States heard challenges to the constitutionality of the individual mandate and the viability of certain provisions of the Healthcare Reform Act. The Supreme Court's decision upheld most of the Healthcare Reform Act and determined that requiring individuals to maintain "minimum essential" health insurance coverage or pay a penalty to the Internal Revenue Service was within Congress's constitutional taxing authority. However, the Supreme Court struck down a provision in the Healthcare Reform Act that penalized states that choose not to expand their Medicaid programs through an increase in the Medicaid eligibility income limit from a state's current eligibility levels to 133% of the federal poverty limit. As a result of the Supreme Court's ruling, it is unclear whether states will expand their Medicaid programs by raising the income limit to 133% of the federal poverty level and whether there will be more uninsured patients in 2014 than anticipated when Congress passed the Healthcare Reform Act. For each state that does not choose to expand its Medicaid program, there will be fewer insured patients overall. The reduction in the number of insured patients could impact our sales, business and financial condition.

Promotional Activities and Interactions with Healthcare Providers and Patients

The FDA and other regulatory agencies strictly regulate the promotional claims that may be made about prescription products, including JUXTAPID, through, among other things, standards and regulations for direct-to-consumer advertising, communications regarding unapproved uses, industry-sponsored scientific and educational activities, and promotional activities involving the Internet. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling or other prescribing information. Healthcare providers are generally permitted to prescribe drugs for "off-label" uses – that is, uses not approved by the FDA and therefore not described in the drug's labeling – because the FDA does not regulate the practice of medicine. However, FDA regulations impose stringent restrictions on manufacturers' communications regarding off-label uses. Broadly speaking, a manufacturer may not promote a drug for off-label use, but may engage in non-promotional, balanced communication regarding off-label use under certain conditions. If a company is found to have promoted off-label uses, it may become subject to adverse publicity and enforcement action by the FDA, the Department of Justice, or the Office of the Inspector General of the Department of Health and Human Services, as well as state authorities. This could subject a company to a range of penalties that could have a significant commercial impact, including civil and criminal fines and agreements that materially restrict the manner in which a company promotes or distributes drug products. The federal government has levied large civil and criminal fines against companies for alleged improper promotion and has enjoined several companies from engaging in off-label promotion. The FDA has also requested that companies enter into consent decrees or permanent injunctions under which specified promotional conduct is changed or curtailed.

Healthcare providers, physicians and others will play a primary role in the recommendation and prescription of JUXTAPID and any other products for which we obtain marketing approval. Our future arrangements with

third-party payers and customers will expose us to broadly applicable fraud and abuse and other healthcare laws and regulations that may constrain the business or financial arrangements and relationships through which we market, sell and distribute the products for which 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 offering, paying, soliciting, or receiving remuneration, directly or indirectly, in cash or in kind, to induce or reward the purchasing, leasing, ordering, or arranging for the purchase, lease, or order of any healthcare item or service reimbursable under federal healthcare programs such as Medicare and Medicaid. This statute has been interpreted to apply to arrangements between pharmaceutical manufacturers on the one hand and prescribers, purchasers and formulary managers on the other. Violations of the federal Anti-Kickback Statute are punishable by imprisonment, criminal fines, civil monetary penalties and exclusion from participation in federal healthcare programs. The Healthcare Reform Act, among other things, amends the intent requirement of the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, PPACA provides that the government may assert that a claim including items or services resulting from a violation of the federal anti-kickback statute constitutes a false or fraudulent claim for purposes of the false claims statutes. There are a number of statutory exceptions and regulatory safe harbors protecting certain common activities from prosecution or other regulatory sanctions, however, the exceptions and safe harbors are drawn narrowly, and practices that do not fit squarely within an exception or safe harbor may be subject to scrutiny.
- The federal False Claims Act imposes criminal and civil penalties and provides for 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 knowingly making, or causing to be made, a false record or statement material to a false or fraudulent claim to avoid, decrease, or conceal an obligation to pay money to the federal government. Several pharmaceutical and other healthcare companies have faced enforcement actions under these laws for allegedly inflating drug prices they report to pricing services, which in turn were used by the government to set Medicare and Medicaid reimbursement rates, and for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. In addition, federal Anti-Kickback Statute violations and certain marketing practices, including off-label promotion, may also implicate the federal False Claims Act. Federal False Claims Act violations may result in imprisonment, criminal fines, civil monetary damages and penalties and exclusion from participation in federal healthcare programs.
- The federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, among other things, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, and also imposes obligations with respect to safeguarding the privacy, security and transmission of individually identifiable health information.
- The federal Physician Payment Sunshine Act will require extensive tracking of payments to physicians and teaching hospitals, maintenance of a payments database, and public reporting of the payment data. CMS recently issued a final rule implementing the Physician Payment Sunshine Act provisions, and clarified the scope of the reporting obligations, as well as that manufacturers must begin tracking on August 1, 2013 and must begin reporting payment data to CMS by March 31, 2014. Failure to comply with the reporting obligations may result in civil monetary penalties.
- Analogous state laws and regulations, such as state anti-kickback and false claims laws, apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by Medicaid or other state programs or, in several states, apply regardless of the payer. Several states now require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products in those states and to report gifts and payments to individual health care providers in those states. Some of these states also prohibit certain marketing-related activities including the provision of gifts, meals, or other items to certain health care providers. In addition, several states require pharmaceutical companies to implement compliance programs or marketing codes.

Efforts to ensure that our business activities and business arrangements will comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including activities conducted by our sales team, are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid, and the curtailment or restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

Corporate Information

We were incorporated in February 2005 under the laws of the State of Delaware. Our principal executive offices are located at 101 Main Street, Suite 1850, Cambridge, Massachusetts 02142. Our website address is www.aegerion.com. Our website address is included in this document as an inactive textual reference only and information appearing on our website is not part of, and is not incorporated by reference in, the Annual Report.

Employees

As of December 31, 2012, we had 98 employees. Our employees are engaged in the following functions: administration, finance, legal, clinical, medical affairs, regulatory, manufacturing/supply chain, technical support, and commercial functions. Our employee relations are positive across all operations globally.

Where You Can Find More Information

You are advised to read this Annual Report on Form 10-K in conjunction with other reports and documents that we file from time to time with the SEC. You may obtain copies of these reports after the date of this Annual Report directly from us or from the SEC at the SEC's Public Reference Room at 100 F Street, N.E. Washington, D.C. 20549. In addition, the SEC maintains information for electronic filers (including Aegerion) at its website at www.sec.gov. The public may obtain information regarding the operation of the Public Reference Room by calling the SEC at 1-800-SEC-0330. We make our periodic and current reports available on our internet website, free of charge, as soon as reasonably practicable after such material is electronically filed with, or furnished to, the SEC.

Financial Information

The financial information required under this Item 1 is incorporated herein by reference to Item 8 of this Annual Report on Form 10-K.

Item 1A. Risk Factors

Risks Associated with Product Development and Commercialization

Our business currently depends entirely on the success of JUXTAPID. We may not be able to successfully commercialize JUXTAPID, to meet expectations with respect to sales of JUXTAPID and revenues from such sales or to attain profitability or positive cash flow from operations in the time periods we anticipate, or at all.

Our business currently depends entirely on the successful development and commercialization of our first product, JUXTAPID. In December 2012, the FDA approved JUXTAPID in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, TC, apo B and non-HDL-C in patients with HoFH. We launched JUXTAPID in the U.S. in late January 2013. We have submitted an MAA for approval to market JUXTAPID in the EU for a similar indication. We also expect to file for marketing approval in certain other countries where, in light of the potential size of the market and other relevant commercial and regulatory factors, it makes business sense to do so. We have not yet generated any revenue from the sale of JUXTAPID. Our ability to meet expectations with respect to sales of JUXTAPID and revenues from such sales, and to attain profitability and positive cash flow from operations, in the time periods we anticipate, or at all, will depend on a number of factors, including the following:

- our ability to gain market acceptance of JUXTAPID;
- the degree to which physicians and patients determine that the safety and side effect profile of JUXTAPID are manageable, and that the benefits of JUXTAPID in reducing LDL-C levels outweigh the risks, including those risks set forth in the boxed warning for JUXTAPID, which cites the risk of liver toxicity;
- the prevalence of HoFH being significantly higher than the historically reported rate of one person in one million, and more consistent with management's estimates;
- a safety and side effect profile for JUXTAPID in commercial use that is not less manageable than that seen in our pivotal trial;
- the degree to which patients comply with the dosing and dietary restrictions for JUXTAPID contained in the product label;
- the fact that JUXTAPID is to be used as a chronic therapy and the long-term ability of patients who use JUXTAPID as a chronic therapy to tolerate the drug and stay on medication;
- the willingness of insurance companies, managed care organizations and other companies or government entities that provide reimbursement for medical costs to provide reimbursement for JUXTAPID at the prices at which we offer JUXTAPID without requiring genotyping or imposing any additional major hurdles to access JUXTAPID;
- the level of acceptance by physicians of the efficacy data from our pivotal trial, which is based on the surrogate endpoints of LDL-C lowering, and which was not designed to show clinical outcome data as to the effect of the LDL-C lowering on cardiac outcomes in HoFH patients;
- the mix of government and private payers providing coverage and reimbursement for JUXTAPID;
- the extent to which our pricing decisions, including use of price caps, and the prevalence among the patient population of dosing levels which involve use of multiple capsules to reach a given strength, have an impact on best price ("BP") for purposes of the Medicaid rebate;
- our ability to be able to sell JUXTAPID on a named patient sales basis or through an equivalent mechanism in certain countries where such sales are permitted based on U.S. approval, and where such activities are commercially attractive;
- our ability to gain approval of JUXTAPID outside the U.S. without restrictions that are substantially more onerous or manufacturing specifications that are more difficult to consistently achieve than those imposed in the U.S.;

- our ability to successfully gain approval of JUXTAPID in pediatric patients, and to generate revenues from sales in the pediatric indications;

  our ability to manufacture sufficient quantities of each strength of JUXTAPID to meet demand;

- our ability to obtain patent term extension on our composition of matter patent, and other forms of data and marketing exclusivity in the U.S. and in key markets outside the U.S.; and
- our ability to execute successfully on our commercial launch plan and other key activities, and the level of cost required to conduct such activities;
- our ability to effectively differentiate JUXTAPID from Kynamro and products directed at the PCSK9 gene if approved for HoFH.

We may not be able to gain market acceptance for JUXTAPID.

The commercial success of JUXTAPID will depend upon the acceptance of the product by the medical community, including physicians, patients and healthcare payers.

Some physicians and patients may determine that the benefits of JUXTAPID in reducing LDL-C levels do not outweigh the risks, including those risks set forth in the boxed warning for JUXTAPID. The boxed warning for JUXTAPID warns physicians that JUXTAPID can cause hepatotoxicity as manifested by elevations in transaminases and increases in hepatic fat, and that physicians are recommended to measure ALT, AST, alkaline phosphatase, and total bilirubin before initiating treatment and then ALT and AST regularly during treatment.

Because of the risk of liver toxicity, JUXTAPID is available only through the REMS program. We will certify all qualified healthcare providers who prescribe JUXTAPID and the pharmacies that will dispense the medicine. The goals of the REMS program are:

- to educate prescribers about the risk of hepatotoxicity associated with the use of JUXTAPID and the need to monitor patients during treatment with JUXTAPID as per product labeling; and
- to restrict access to therapy with JUXTAPID to patients with a clinical or laboratory diagnosis consistent with HoFH.

During the first year of treatment, the physician must conduct a liver-related test prior to each increase in dose or monthly, whichever occurs first. After the first year, physicians are required to perform these tests every three months and before increases in dose. Physicians may be hesitant to prescribe JUXTAPID, and patients may be hesitant to take JUXTAPID, because of the boxed warning, the requirements for liver testing or the existence of the REMS program. There are also a number of additional contraindications and warnings in the prescribing information that could limit the market acceptance of JUXTAPID. For example, GI adverse reactions are common with JUXTAPID, occurring in 27 out of 29 patients in our pivotal trial. We expect that GI events may lead to treatment discontinuation in some patients. To reduce the risk of GI adverse reactions, patients should adhere to a low-fat diet supplying less than 20% of calories from fat and the dosage of JUXTAPID should be increased gradually. Patients on JUXTAPID are also advised not to consume more than one alcoholic drink per day. These requirements may make it more difficult for a patient to decide to begin therapy or to stay on therapy.

The degree of market acceptance of JUXTAPID will also depend on a number of other factors, including:

- physicians' views as to the scope of the approved indication and limitations on use and warnings and precautions contained in JUXTAPID's approved labeling;
- the efficacy and safety of competitive therapies;
- pricing and the perception of physicians and payers as to cost effectiveness;
- the existence of sufficient third-party coverage or reimbursement; and
- the effectiveness of our sales, marketing and distribution strategies.

If we are not able to achieve a high degree of market acceptance of JUXTAPID in the treatment of HoFH, we may not be able to achieve our revenue goals or other financial goals or to achieve profitability or cash-flow break-even in the time periods we expect, or at all.

The number of patients suffering from HoFH is small, and has not been established with precision. We believe that the patient population is significantly larger than the reported prevalence indicates, but our assumptions and estimates may be wrong. If the actual number of patients is smaller than we estimate or if any approval outside the U.S. is based on a narrower definition of these patient populations, our revenue and ability to achieve profitability and cash-flow break-even will be adversely affected, possibly materially.

There is no patient registry or other method of establishing with precision the actual number of patients with HoFH in any geography. Medical literature has historically reported the prevalence rate of genotypic HoFH as one person in a million. However, we believe that the prevalence rate of HoFH is higher. The historically reported definition of HoFH used a narrow genotypic definition of HoFH. At the time the rate was first reported, many of the genetic mutations leading to defects in LDL-receptor function were not characterized, and some mutations remain uncharacterized even today. In addition, many physicians use a broader definition of HoFH that includes patients diagnosed through phenotypic criteria. In 2010, we commissioned an independent consultant in the healthcare industry to prepare a commercial assessment of the HoFH market for us. In its report, this consultant estimated that the total number of patients likely to seek treatment with symptoms, signs or laboratory findings consistent with HoFH in each of the U.S. and the EU is approximately 3,000 patients. This consultant's estimates, however, included a segment of severe HeFH patients whose levels of LDL-C are not controlled by current therapies. These patients may be phenotypically indistinct from HoFH patients. JUXTAPID is indicated solely for HoFH. Our prescribing information specifies that the safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH. We are not permitted to promote JUXTAPID for any indication other than HoFH. In addition, as part of the prescriber authorization form under our REMS program in the U.S., the prescriber must affirm that the patient has a clinical or laboratory diagnosis consistent with HoFH. We do not know how many patients will be determined to have a clinical or laboratory diagnosis consistent with HoFH, and there is no generally accepted and referenced definition of HoFH matching these criteria. However, rare diseases are often found to have a higher than expected prevalence rate once the first product available to treat the disease is introduced. We expect this may also be true for HoFH. As a result, we believe that, even if we exclude the patients who have a clinical phenotype consistent with HoFH, but as to whom the prescriber cannot conclude and affirm that the patient has a clinical or laboratory diagnosis consistent with HoFH, there still may be as many as 3,000 HoFH patients in the U.S. based on our belief that the base prevalence rate may be higher than our consultant estimated. There is no guarantee that our assumptions and beliefs are correct. The number of patients with HoFH could actually be significantly lower than we expect, and could be closer to the historically reported rates than to our estimate of 3,000 patients. Ultimately the actual size of the total addressable market in the U.S. will be determined only after we have substantial commercial history selling JUXTAPID and we are able to assess how it is being used clinically. We believe that the prevalence rate in the EU is likely to be consistent with the prevalence rate in the U.S. However, the total addressable HoFH market in the EU will depend ultimately on whether the EMA requires a genetic diagnosis or imposes other narrow diagnosis criteria. If the total addressable market in the U.S. and the EU is lower than we expect, then it may be more difficult for us to generate revenues and to achieve or maintain profitability.

We have studied JUXTAPID initially for the treatment of patients with HoFH who are 18 years of age or older. The label for JUXTAPID in the U.S. specifies that the safety and effectiveness of JUXTAPID have not been established in pediatric patients. We have a post-marketing commitment to the FDA to conduct a juvenile toxicology study in rodents prior to initiating a clinical study of JUXTAPID in pediatric patients. The juvenile animal toxicology study will seek to ascertain the impact, if any, of JUXTAPID on growth and development.

If the results of the juvenile animal toxicology study support proceeding forward, we plan to conduct a clinical trial of JUXTAPID for the treatment of pediatric HoFH patients. There is no guarantee that the results of the juvenile animal toxicology study will justify proceeding to a study of JUXTAPID in pediatric patients. In

addition, while the PDCO previously issued a positive opinion on our Pediatric Investigation Plan for lomitapide in the EU, the PDCO opinion requires that, prior to initiation of a pediatric study in the EU, the data on lomitapide generated in the adult HoFH population must be evaluated by the CHMP of the EMA, and a positive conclusion on the benefit/risk balance and therapeutic benefit must be found, at which point the pediatric study will be reevaluated by PDCO. There is no guarantee that PDCO will permit a pediatric study to proceed in the EU even if we receive a positive opinion on lomitapide from the CHMP. Even if we conduct a study in pediatric patients, we may not be able to show, to the satisfaction of the FDA or EMA or regulatory authorities in other countries, that JUXTAPID is safe and effective in pediatric patients, and we may never receive approval for this indication. The lack of approval to market JUXTAPID for the pediatric HoFH population will limit our product revenue potential, and may make it more difficult for us to achieve or maintain profitability.

We do not have regulatory approval for commercial distribution of JUXTAPID outside the U.S.

We are not permitted to market or sell JUXTAPID in the EU or in any other countries outside the U.S. on a commercial basis until we receive the requisite approval from such countries. In order to market any product outside of the U.S., we must establish and comply with numerous and varying regulatory requirements of other countries regarding safety and efficacy and governing, among other things, clinical trials, pricing and distribution of the product. Approval procedures vary among countries, and can involve additional product testing and additional administrative review periods. The time required to obtain approval in other countries might differ from that required to obtain FDA approval. In particular, in many countries outside the U.S., it is required that a product receives pricing and reimbursement approval before the product can be commercialized. This can result in substantial delays in such countries, and the price that is ultimately approved may be lower than the price for which we expect to offer JUXTAPID.

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 or marketing approval process in others. Failure to obtain marketing approval in other countries or any delay or setback in obtaining such approval would impair our ability to develop foreign markets for JUXTAPID. Similarly, any significant differences between the requirements imposed by the FDA and the EMA with respect to regulatory approval of JUXTAPID might delay approval or launch in the EU. Any such differences may reduce our target market and delay or limit the full commercial potential of JUXTAPID. Many countries are undertaking cost-containment measures that could affect pricing or reimbursement of JUXTAPID.

Obtaining approval of an MAA or any other filing for approval in a foreign country is an extensive, lengthy, expensive and uncertain process, and the regulatory authority may reject a filing or delay, limit or deny approval of JUXTAPID for many reasons, including:

- we may not be able to demonstrate to the satisfaction of regulatory authorities outside the U.S. that JUXTAPID is safe and effective for any indication;
- the results of clinical trials may not meet the level of statistical significance or clinical significance required by regulatory authorities outside the U.S. for approval;
- regulatory authorities outside the U.S. may disagree with the number, design, size, conduct or implementation of our clinical trials, including the use of LDL-C lowering as a surrogate endpoint without any data on clinical outcomes;
- regulatory authorities may not find the data from preclinical studies and clinical trials sufficient to demonstrate that JUXTAPID's clinical and other benefits outweigh its safety risks; or such regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials and require that we conduct one or more additional trials;
- regulatory authorities outside the U.S. may not accept data generated at our clinical trial sites;

- regulatory authorities outside the U.S. may require additional preclinical or clinical studies or other data prior to granting approval, and we may not be able to generate the required data on a timely basis, or at all;
- regulatory authorities outside the U.S. may impose limitations on the approved labeling of JUXTAPID, such as requiring a genetic diagnosis or otherwise narrowing the diagnosis criteria for HoFH thus limiting intended users or providing an additional hurdle for market acceptance of the product;
- regulatory authorities outside the U.S. may require a more onerous risk mitigation and management plan than the REMS program we have in place in the U.S., as a condition of approval, may not approve lomitapide because the regulator's legal mandate does not permit them to impose a REMS-like program or may otherwise disagree with our proposals to address risk mitigation and management;
- regulatory authorities outside the U.S. may identify deficiencies in the manufacturing processes or in the facilities of our third-party contract manufacturers, or may require us to manufacture additional registration batches or change our manufacturing process or specifications;
- we may not be able to validate our manufacturing process to the satisfaction of regulatory authorities outside the U.S.; or
- regulatory authorities outside the U.S. may change approval policies or adopt new regulations.

It is possible that the EMA or other regulatory authorities outside the U.S. may not consider the data from our pivotal Phase 3 clinical trial of JUXTAPID in patients with HoFH to be sufficient for approval of lomitapide for this indication, or may not consider LDL-C lowering alone sufficient for approval without demonstrating a beneficial effect on a clinical outcome.

It is possible that the EMA or other regulatory authorities outside the U.S. may not agree with our assessment that certain changes made to lomitapide's physical parameters and specifications as compared to the material used in the pivotal trial are not clinically meaningful. If the EMA or other regulatory authorities require additional studies or trials or changes to specifications, we would incur increased costs and delays in the marketing approval process. For example, Japanese regulatory authorities have required us to conduct two studies prior to our submission of an application for marketing authorization for lomitapide in Japan: a Phase 1 bridging study of the pharmacokinetic and pharmacodynamic ("PK/PD") properties of lomitapide in Japanese and Caucasian patients, and, following the outcome of that PK/PD study, a small therapeutic study of lomitapide in Japanese HoFH patients. The results of the PK/PD study may show that there are differences in the pharmacokinetic or pharmacodynamic effects of lomitapide in Japanese and Caucasian patients which may cause us to have to develop a new dose strength for Japanese patients and/or to change the dosing schedule. Any additional work to refine dosing for Japanese patients as a result of the PK/PD study would likely delay the start of the small clinical study in HoFH patients in Japan. There is no assurance that we will be successful in our efforts to generate the data we need to submit a marketing authorization application in Japan or to achieve regulatory approval in Japan on a reasonable timeline, or at all.

In certain countries where permitted based on U.S. approval of JUXTAPID, we plan to make lomitapide available on a named patient sales basis. There is no assurance that this mechanism will be available in any particular country, or that we will pursue such activity even if permitted to do so in a particular country. Even if named patient sales or their equivalent sales are permitted in a certain country and we elect to make lomitapide available on such basis in such country, there is no guarantee that the country will pay for the product or that we will generate sales or substantial revenue from such sales, if any. There may also be countries where we choose to make lomitapide available at no cost prior to approval in such country.

As a result of the side effects observed in the Phase 3 clinical study and other clinical and preclinical studies of JUXTAPID, the prescribing information for JUXTAPID in the U.S. contains a boxed warning, significant limitations on use and other important warning and precautions, and the distribution of JUXTAPID is subject to a REMS program as a result of concerns over liver toxicity. JUXTAPID may continue to cause such side effects or have other properties that could delay or prevent its marketing approval in territories outside the U.S. or result in adverse limitations in any approved labeling in the U.S. or in such other territories.

JUXTAPID contains a boxed warning in the U.S. citing the risk of liver toxicity. JUXTAPID can cause elevations in transaminases. In our pivotal trial, 10 of the 29 patients (34%) treated with JUXTAPID had at least one elevation in ALT or AST greater than or equal to 3x ULN, including four patients who experienced liver enzymes greater than or equal to 5x ULN. There were no concomitant clinically meaningful elevations of total bilirubin, INR, or alkaline phosphatase. JUXTAPID also has been shown to increase hepatic fat, with or without concomitant increases in transaminases. The median absolute increase in hepatic fat during the pivotal trial was 6% after both 26 and 78 weeks of treatment, from 1% at baseline, measured by magnetic resonance spectroscopy. Hepatic steatosis associated with JUXTAPID treatment may be a risk factor for progressive liver disease, including steatohepatitis and cirrhosis.

The most common adverse reactions in our pivotal trial of JUXTAPID were gastrointestinal, reported by 27 of 29 patients (93%). Adverse reactions reported by greater than or equal to 8 patients (28%) in the HoFH clinical trial included diarrhea, nausea, vomiting, dyspepsia and abdominal pain. Other common adverse reactions, reported by five to seven (17-24%) patients, included weight loss, abdominal discomfort, abdominal distension, constipation, flatulence, increased ALT, chest pain, influenza, nasopharyngitis and fatigue.

In a two-year dietary carcinogenicity study of lomitapide in mice, statistically significant increased incidences of tumors in the small intestine and liver were observed. The relationship of these findings in mice is uncertain with regard to human safety for a number of reasons, including the fact that they did not occur in a dose-related manner, and liver tumors are common spontaneous findings in the strain of mice used in this study. In a two-year oral carcinogenicity study of lomitapide in rats, there were no statistically significant increases in the incidences of any tumors, but there can be no assurance that long-term usage of lomitapide in humans will not be determined to cause an increase in tumors.

As part of our post-marketing commitment to the FDA, we will conduct an observational cohort study to generate more data on the long-term safety profile of JUXTAPID, the patterns of use and compliance and the long-term effectiveness of controlling LDL-C levels. The patient registry study will have a target enrollment of 300 HoFH patients worldwide. In the study, investigators will follow each patient for a period of 10 years to track malignancies, teratogenicity and hepatic effects.

As part of our observational cohort study or in the conduct of additional clinical studies or in post-marketing surveillance, we or others may identify additional safety information on known side effects or new undesirable side effects caused by JUXTAPID, and, in that event, a number of potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw their approval of JUXTAPID;
- regulatory authorities may require the addition of labeling statements, such as warnings or contraindications or distribution and use restrictions;
- regulatory authorities may require us to issue specific communications to healthcare professionals, such as "Dear Doctor" letters;
- regulatory authorities may issue negative publicity regarding JUXTAPID, including safety communications;
- we may be required to change the way JUXTAPID is administered, conduct additional preclinical studies or clinical trials or restrict the distribution or use of JUXTAPID;
- we could be sued and held liable for harm caused to patients;

- the regulatory authorities may amend the REMS; and
- our reputation may suffer.

As part of the development of the commercial manufacturing process, we tightened specifications for drug substance such that the commercial drug substance differs from the material used in our Phase 3 trial in certain physical parameters and specifications that we do not believe are clinically meaningful. While we do not expect the changes to have any efficacy or safety consequences, there is the risk that we may see unexpected differences in the type or severity of side effects with the commercial product.

Any known safety concerns for JUXTAPID or any unknown safety issues that may develop could prevent us from achieving or maintaining market acceptance of JUXTAPID and our financial goals, and could adversely affect our ability to obtain approval of JUXTAPID outside the U.S.

We currently depend on a single third-party manufacturer to produce our JUXTAPID drug substance and a different third-party manufacturer to produce our drug product. This may increase the risk that we will not have sufficient quantities of JUXTAPID or such quantities at an acceptable cost, which could delay, prevent or impair our clinical development and commercialization of JUXTAPID.

We currently have no manufacturing facilities and limited personnel with manufacturing experience. We rely on a contract manufacturer to produce drug substance for JUXTAPID and another contract manufacturer for drug product for our clinical trials and for commercial supplies. We have entered into a long-term commercial supply agreement for JUXTAPID drug substance and drug product. We do not have any agreements or arrangements in place for redundant supply or a second source for JUXTAPID drug substance or drug product. Any performance failure on the part of our existing or future manufacturers could delay further clinical development or marketing approval of JUXTAPID in countries and territories outside the U.S. or commercialization of JUXTAPID in the U.S. If for some reason either of our current contract manufacturers cannot perform as agreed, we may be required to replace that manufacturer. Although we believe there are a number of potential replacements that could manufacture the clinical and commercial supply of JUXTAPID drug substance or drug product, we may incur added costs and delays in identifying and qualifying any such replacements. Furthermore, although we generally do not begin a clinical trial unless we have a sufficient supply of a product candidate for the trial, any significant delay in the supply of a product candidate for an ongoing trial due to the need to replace a third-party manufacturer could delay completion of the trial.

We have not previously manufactured commercial supplies of JUXTAPID and will rely on our contract manufacturers to utilize processes that consistently produce drug substance and drug product to their required specifications, including those imposed by the FDA and other regulatory authorities. As part of the development of the commercial manufacturing process, we tightened specifications for drug substance such that the commercial drug substance differs from the material used in our Phase 3 trial in certain physical parameters and specifications that we do not believe are clinically meaningful. We have completed validation of the manufacturing process for drug substance. The FDA has permitted us to conduct concurrent validation of each strength of our JUXTAPID drug product which allows for the concurrent release and commercial distribution of each successful validation batch once completed. We are in the process of completing the final validation batches for drug product. Any delay or technical hurdle in our validation work may impact the availability of product, and may result in additional expense. There can be no assurance that our contractors will consistently be able to produce commercial supplies of drug substance or drug product meeting the approved specifications. Any failure by our third-party manufacturers to produce product that meets specifications could lead to a shortage of JUXTAPID.

If we are unable to maintain arrangements for third-party manufacturing, or are unable to do so on commercially reasonable terms, we may not be able to successfully commercialize JUXTAPID or complete development of JUXTAPID. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured JUXTAPID ourselves, including:

- reliance on the third party for regulatory compliance and quality assurance;

- the possibility of breach of the manufacturing agreement by the third party because of factors beyond our control; and
- the possibility of termination or nonrenewal of the agreement by the third party (including merger and acquisition activity, bankruptcy filing, and strategic shifts), based on its own business priorities, at a time that is costly or damaging to us.

In addition, the FDA and other regulatory authorities require that product candidates and drug products be manufactured according to cGMP. Any failure by our third-party manufacturers to comply with cGMP could lead to a shortage of JUXTAPID. In addition, such failure could be the basis for action by the FDA or EMA to withdraw approvals previously granted to us and for other regulatory action, including seizure, injunction or other civil or criminal penalties.

JUXTAPID and any other product candidate that we develop may compete with other products and product candidates for access to manufacturing facilities. There are a limited number of manufacturers that operate under cGMP regulations and that are both capable of manufacturing for us and willing to do so. If we need to find another source of drug substance or drug product for JUXTAPID, we may not be able to identify, or reach agreement with, commercial-scale manufacturers on commercially reasonably terms, or at all. If we are unable to do so, we will need to develop our own commercial-scale manufacturing capabilities, which would: impact commercialization of JUXTAPID in the U.S. and other territories and countries where it may be approved; require a capital investment by us that could be quite costly; and increase our operating expenses.

If our existing third-party manufacturers, or the third parties that we engage in the future to manufacture a product for commercial sale or for our clinical trials, should cease to continue to do so for any reason, we may experience significant delays in obtaining sufficient quantities of product for us to meet commercial demand or to advance our clinical trials while we identify and qualify replacement suppliers. If for any reason we are unable to obtain adequate supplies of JUXTAPID or any other product candidate that we develop, or the drug substances used to manufacture it, it will be more difficult for us to compete effectively, generate revenue, and further develop our products. In addition, if we are unable to assure a sufficient quantity of the drug for patients with rare diseases or conditions, we may lose any orphan drug exclusivity to which the product otherwise would be entitled.

Our market is subject to intense competition. If we are unable to compete effectively, JUXTAPID or any other product candidate that we develop may be rendered noncompetitive or obsolete.

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. All of these competitors currently engage in, have engaged in or may engage in the future in the development, manufacturing, marketing and commercialization of new pharmaceuticals, some of which may compete with JUXTAPID or other product candidates. Smaller or early stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Key competitive factors affecting the commercial success of JUXTAPID and any other product candidates that we develop are likely to be efficacy, safety and tolerability profile, reliability, convenience of dosing, price and reimbursement. The market for cholesterol-lowering therapeutics is large and competitive with many drug classes. JUXTAPID is approved in the U.S. as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, TC, apo B and non-HDL-C in HoFH patients. As a treatment for HoFH, JUXTAPID competes in the U.S. with Kynamro, which is being commercialized by Genzyme, now part of Sanofi under a collaboration with Isis. Kynamro is an antisense apolipoprotein B-100 inhibitor which is taken as a weekly subcutaneous injection. On January 29, 2013, the FDA approved Kynamro as an adjunct to lipid-lowering medications and diet to reduce low density LDL-C, apo B, TC, and non HDL-C in patients with HoFH. If Isis and Genzyme obtain marketing approval of Kynamro for the treatment of patients with HoFH in any country prior to us, they could obtain a competitive advantage associated with being the first to market.

We believe that JUXTAPID will face additional competition for the treatment of HoFH. Although there are no other MTP-I compounds currently approved by the FDA for the treatment of hyperlipidemia, there may be other MTP-I compounds in development. We are aware of other pharmaceutical companies that are developing product candidates that may compete with JUXTAPID in the treatment of HoFH, including Regeneron, in collaboration with Sanofi, Roche Holding AG, Amgen and Alnylam Pharmaceuticals, Inc., in collaboration with The Medicines Company, all of which are developing molecules that attempt to mimic the impact observed in patients with defects in their PCSK9 gene. Such patients have lower LDL-C levels and an observed reduction in cardiovascular events, and some believe that medicines that duplicate this behavior may effectively reduce LDL-C levels with a similar benefit. In 2011, Regeneron and Sanofi announced positive results from Phase 2 clinical trials of its anti-PCSK9 antibody in patients with HeFH and primary hypercholesterolemia. In July 2012, Regeneron and Sanofi announced commencement of patient enrollment for a 22,000 patient Phase 3 clinical program to evaluate its anti-PCSK9 antibody in several patient populations, including those with HeFH and primary hypercholesterolemia. Amgen is also conducting a clinical trial of its anti-PCSK9 antibody in multiple patient populations, including HoFH patients, with initial clinical data expected to be reported as early as this year. We expect that some HoFH patients who might otherwise be candidates for treatment with JUXTAPID will be committed to clinical studies of anti-PCSK9 antibodies. Given the rarity of HoFH, this may make it more difficult for us to generate revenues and achieve profitability. Regeneron and Sanofi have indicated that their PCSK-9 product could receive approval in the treatment for HeFH as early as 2015.

Many of our potential competitors have substantially greater financial, technical and human resources than we do, and significantly greater experience in the discovery and development of drug candidates, obtaining FDA and other regulatory approvals of products and the commercialization of those products. Accordingly, our competitors may be more successful than we may be in obtaining marketing approvals 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 JUXTAPID or any other product candidate that we develop obsolete or non-competitive before we can recover the expenses of developing and commercializing the product. 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 JUXTAPID or any other product candidate that we develop non-competitive or obsolete.

We may face resistance from certain payers given the price we expect to charge for JUXTAPID. It will be difficult for us to profitably sell JUXTAPID or any other product for which we obtain marketing approval if reimbursement for the product is limited or delayed.

Given that HoFH is a rare disease with a small patient population, we will need to set and charge a price for JUXTAPID that is significantly higher than that of most pharmaceuticals in order to generate enough revenue to fund our operating costs. We may face resistance from certain payers in the U.S. and in other countries to provide adequate coverage and reimbursement for JUXTAPID. Based on our discussions with key payers in the U.S., we do not expect that genotyping will typically be required in the U.S. to determine a diagnosis of HoFH for reimbursement purposes or that price differences among therapies will typically drive reimbursement decisions, although there will be some exceptions. Payers in the U.S. may, however, impose other requirements, conditions or limitations as conditions to coverage and reimbursement for JUXTAPID. Outside the U.S., the ongoing sovereign debt crisis and the macroeconomic climate in the EU may adversely affect our ability to set and charge a sufficiently high price to generate adequate revenue in those markets. Those countries may impose onerous conditions on reimbursement, which may include genotyping. In addition, we may face pricing and reimbursement pressure in the U.S., EU and other territories as a result of prices charged for competitive products.

We plan to make lomitapide available in certain countries that allow use of a drug, on a named patient basis or under a compassionate use or other type of so-called expanded access program, before it has obtained marketing approval in such country. We plan to seek reimbursement for lomitapide for authorized pre-approval uses in some of these countries to the extent permitted by applicable law and local regulatory authorities. In other

countries or under certain circumstances, we may provide lomitapide free of charge for permitted pre-approval uses. In certain countries where we seek reimbursement for the product during the pre-approval phase, we will be able to establish the price for lomitapide, while in other countries we will need to negotiate the price. Such negotiations may not result in a price acceptable to us, in which case we may elect to not pursue distribution of lomitapide in such country prior to approval or we may curtail distribution. Further, any negotiated price may adversely affect the market prices in other countries or jurisdictions where we may sell lomitapide, if such price is lower than the price that would have otherwise been set in such geographies.

Market acceptance and sales of JUXTAPID will depend on coverage and reimbursement policies and may be affected by healthcare reform measures. Government authorities and third-party payers, such as private health insurers and health maintenance organizations, decide which medications they will pay for and establish reimbursement levels. A primary trend in the U.S. healthcare industry and elsewhere is cost containment. Government authorities and these third-party payers have attempted to control costs by limiting coverage and limiting the amount of reimbursement for particular medications. We cannot be sure that coverage and reimbursement will be available for JUXTAPID or any other product that we develop and, if reimbursement is available, the level of reimbursement. Coverage and reimbursement may impact the demand for, or the price of, any product for which we obtain marketing approval. Obtaining coverage and reimbursement for JUXTAPID at all or at levels satisfactory to us may be particularly difficult because of the higher prices often associated with drugs directed at orphan populations, and the pricing of therapies, such as apheresis, or competitive products that may be deemed to be interchangeable or clinically equivalent to JUXTAPID by payers. In addition, third-party payers may impose strict requirements for reimbursement in order to limit use of a higher priced drug. If coverage and reimbursement is not available or is available only at limited levels, we may not be able to successfully commercialize JUXTAPID or any other product candidate that we develop. In addition, if we fail to successfully secure and maintain reimbursement coverage for JUXTAPID or any future products or are significantly delayed in doing so, we will have difficulty achieving market acceptance of our products and our business will be harmed. We expect to support independent patient foundations that assist eligible patients with certain co-payments or co-insurance requirements, and to assist certain eligible uninsured or underinsured patients. Our support of these programs could result in significant costs to us.

The availability and amount of reimbursement for JUXTAPID and our product candidates and the manner in which government and private payers may reimburse for our potential products is uncertain.

Beginning April 1, 2013, Medicare payments for all items and services under Part A and B, including drugs and biologicals, and payments to plans under Medicare Part D could be reduced by up to 2% under the sequestration (i.e., automatic spending reductions) required by the BCA, as amended by ATRA. The BCA requires sequestration for most federal programs, excluding Medicaid, Social Security, and certain other programs, because Congress failed to enact legislation by January 15, 2012, to reduce federal deficits by $1.2 trillion over ten years. The BCA caps the cuts to Medicare payments for items and services and payments to Part D plans at 2%, and requires the cuts to be implemented on the first day of the first month following the issuance of a sequestration order. The ATRA delayed implementation of sequestration from January 2, 2013, to March 1, 2013, and as a result, the Medicare cuts will take effect April 1, 2013, unless Congress enacts legislation to cancel or delay the cuts. If implemented, these cuts would adversely impact payment for JUXTAPID.

Payers also are increasingly considering new metrics as the basis for reimbursement rates, such as ASP, AMP and AAC. The existing data for reimbursement based on these metrics is relatively limited, although certain states have begun to survey acquisition cost data for the purpose of setting Medicaid reimbursement rates, and CMS has begun making pharmacy National Average Drug Acquisition Cost and National Average Retail Price data publicly available on at least a monthly basis. Therefore, it may be difficult to project the impact of these evolving reimbursement mechanics on the willingness of payers to cover our products. Recent legislative changes to the 340B drug pricing program, the Medicaid Drug Rebate Program, and the Medicare Part D prescription drug benefit also could impact our revenues. If reimbursement is not available or available only to limited levels, we may not be able to generate sufficient revenue to meet our operating costs or to achieve our revenue, cash flow breakeven or profitability goals in the timeframe that we expect, or at all.

Enacted and future legislation may increase the difficulty and cost for us to commercialize JUXTAPID or any other product candidate that we develop and affect the prices we may obtain.

In the U.S. and some foreign jurisdictions, there have been a number of legislative and regulatory changes and proposed changes regarding the healthcare system that restrict or regulate post-approval activities, and may affect our ability to profitably sell JUXTAPID or any other product candidate for which we obtain marketing approval.

Legislative and regulatory proposals have been made to expand post-approval requirements and restrict sales and promotional activities for pharmaceutical products. We are not sure whether additional legislative changes will be enacted, or whether the FDA regulations, guidance or interpretations will be changed, or what the impact of such changes on JUXTAPID may be. In addition, increased scrutiny by the U.S. Congress of the FDA's approval process may subject us to more stringent product labeling and post-marketing testing and other requirements.

In the U.S., the MMA changed the way Medicare covers and pays for pharmaceutical products. The legislation expanded Medicare coverage for drug purchases by those covered by Medicare and introduced a new reimbursement methodology based on average sales prices for drugs. In addition, this legislation authorized Medicare Part D prescription drug plans to use formularies where they can limit 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 contain and reduce costs. These cost reduction initiatives and other provisions of this legislation could decrease the coverage and 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 payers often follow Medicare coverage policy and payment limitations in setting their own reimbursement rates, and any reduction in reimbursement that results from the MMA may result in a similar reduction in payments from private payers.

More recently, in 2010, President Obama signed into law the Patient Protection and Affordable Care Act and the Health Care and Education Reconciliation Act of 2010 (together, the "Health Care Reform Law"), a sweeping law intended to broaden access to health insurance, reduce or constrain the growth of healthcare spending, enhance remedies against fraud and abuse, add new transparency requirements for healthcare and health insurance industries, impose new taxes and fees on the health industry and impose additional health policy reforms. The Health Care Reform Law revised the definition of "average manufacturer price" for reporting purposes, which could increase the amount of Medicaid drug rebates to states. The Health Care Reform Law also imposes a significant annual fee on companies that manufacture or import branded prescription drug products. We do not know the full effects that the Health Care Reform Law will have on our commercialization efforts with respect to JUXTAPID. Although it is too early to determine the effect of the Health Care Reform Law, the law appears likely to continue the pressure on pharmaceutical pricing, especially under the Medicare program, and may also increase our regulatory burdens and operating costs.

We face extensive regulatory requirements, and JUXTAPID may still face future development and regulatory difficulties.

Even after marketing approval, a regulatory authority may still impose significant restrictions on a product's indications, conditions for use, distribution or marketing or impose ongoing requirements for potentially costly post-marketing surveillance, post-approval studies or clinical trials. Because of the risk of liver toxicity, JUXTAPID is available only through the REMS program. We will certify all healthcare providers who prescribe JUXTAPID and the pharmacies that will dispense the medicine. The goals of the REMS program are:

- to educate prescribers about the risk of hepatotoxicity associated with the use of JUXTAPID and the need to monitor patients during treatment with JUXTAPID as per product labeling; and
- to restrict access to therapy with JUXTAPID to patients with a clinical or laboratory diagnosis consistent with HoFH.

As part of our post-marketing commitment to the FDA with respect to JUXTAPID, we will conduct an observational cohort study to generate more data on the long-term safety profile of JUXTAPID, the patterns of use and compliance and the long-term effectiveness of controlling LDL-C levels. The patient registry study will have a target enrollment of 300 HoFH patients worldwide. In the study, investigators will follow each patient for a period of 10 years to track malignancies, teratogenicity or hepatic effects. JUXTAPID will also be subject to ongoing FDA requirements governing the labeling, packaging, storage, advertising, distribution, promotion, recordkeeping and submission of safety and other post-marketing information, including adverse events, and any changes to the approved product, product labeling, or manufacturing process. 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 trials to evaluate serious safety risks related to the use of a drug. In addition, 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 cGMP, and other regulations.

If we, or our drug substance or drug product or the manufacturing facilities for our drug substance or drug product, fail to comply with applicable regulatory requirements, a regulatory agency may:

- issue warning letters or untitled letters;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend or withdraw or alter the conditions of our marketing approval;
- mandate modifications to promotional materials or require us to provide corrective information to healthcare practitioners;
- suspend any ongoing clinical trials;
- require us to enter into a consent decree, which can include imposition of various fines, reimbursements for inspection costs, required due dates for specific actions and penalties for noncompliance;
- refuse to approve pending applications or supplements to applications submitted by us;
- suspend or impose restrictions on operations, including costly new manufacturing requirements;
- seize or detain products, refuse to permit the import or export of products or request that we initiate a product recall; or
- refuse to allow us to enter into supply contracts, including government contracts.

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 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. In particular, a product may not be promoted for uses that are not approved by the FDA or such other regulatory agencies as reflected in the product's approved labeling or other prescribing information. Violations, including promotion of our products for unapproved (or off-label) uses, are subject to enforcement letters, inquiries and investigations, and civil and criminal sanctions by the FDA. Engaging in impermissible promotion of our products for off-label uses can also subject us to false claims litigation under federal and state statutes, which can lead to consent decrees, civil money penalties, restitution, criminal fines and imprisonment, and exclusion from participation in Medicare, Medicaid and other federal and state health care programs. These false claims statutes include the federal False Claims Act, which allows any individual to bring a lawsuit against a pharmaceutical company on behalf of the federal government alleging submission of false or fraudulent claims, or causing to present such false or fraudulent claims, for payment by a federal program such as Medicare or Medicaid. If the government prevails in the lawsuit, the individual will share in any fines or settlement funds. This growth in litigation has increased the risk that a pharmaceutical company will have to defend a false claim action, pay settlement fines or restitution, agree to comply with burdensome reporting and compliance

obligations, and be excluded from the Medicare, Medicaid, and other federal and state healthcare programs. If we do not lawfully promote our approved products or are subject to allegations that we do not lawfully promote our product, we may become subject to such litigation and, if we are not successful in defending against such actions, those actions may have a material adverse effect on our business, financial condition and results of operations.

If we are unable to execute effectively our sales and marketing activities, we may be unable to generate sufficient product revenue.

We have not yet demonstrated an ability to commercialize successfully any product candidate. JUXTAPID is our first product. As a result, we have only recently had to build our sales, marketing, managerial and other non-technical capabilities in the U.S.. We plan to continue to build commercial infrastructure in the EU and in other key countries in order to be ready to launch lomitapide with a relatively small specialty sales force if the product is ultimately approved in those jurisdictions. The establishment and development of our commercial infrastructure will continue to be expensive and time consuming, and we may not be able to successfully fully develop this capability in a timely manner or at all. We anticipate developing a commercial infrastructure across multiple jurisdictions, if JUXTAPID is approved in such jurisdictions. Doing so will require a high degree or coordination and compliance with laws and regulations in such jurisdictions. If we are unable to effectively coordinate such activities or comply with such laws and regulations, our ability to commercialize JUXTAPID in such jurisdictions will be adversely affected. Even if we are able to effectively hire a sales force and develop a marketing and sales infrastructure, our sales force may not be successful in commercializing JUXTAPID or any other product candidate that we develop. If we are unable to establish adequate sales, marketing and distribution capabilities, whether independently or with third parties, we may not be able to generate product revenue and may not become profitable.

We are evaluating markets outside the U.S. and major market countries in the EU to determine in which geographies we might choose to commercialize JUXTAPID ourselves, if approved, and in which geographies we might choose to collaborate with third parties. To the extent we rely on third parties to commercialize JUXTAPID, if marketing approval is obtained in the relevant country, we may receive less revenue than if we commercialized the product ourselves. In addition, we would have less control over the sales efforts of any third parties involved in our commercialization efforts. In the event we are unable to collaborate with a third-party marketing and sales organization to commercialize JUXTAPID, for certain geographies, our ability to generate product revenue may be limited internationally.

Our relationships with customers and payers will be subject to applicable anti-kickback, fraud and abuse and other 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 have and will continue to play an important role in the recommendation and prescription of JUXTAPID and any other products for which we obtain marketing approval. Our arrangements with third-party payers and customers have and will continue to 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 JUXTAPID and other products for which we may 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, paying, or receiving remuneration, directly or indirectly, in cash or in kind, to induce or in return for purchasing, leasing, ordering, or arranging for the purchase, lease or order of any healthcare item or service, for which payment may be made under federal healthcare programs such as Medicare and Medicaid. This statute has been interpreted to apply to arrangements between pharmaceutical companies on one hand and prescribers, purchasers and formulary managers on the other. Further, the Healthcare Reform Act, among other things, amends the intent requirement of

the federal Anti-Kickback Statute. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. In addition, the Healthcare Reform Act provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the false claims statutes. Although there are a number of statutory exemptions and regulatory safe harbors protecting certain common manufacturer business arrangements and activities from prosecution, the exemptions and safe harbors are drawn narrowly, and practices that involve remuneration may be subject to scrutiny if they do not qualify for an exemption or safe harbor. We intend to comply with the exemptions and safe harbors whenever possible, but our practices may not in all cases meet all of the criteria for safe harbor protection from anti-kickback liability and may be subject to scrutiny.

- The federal False Claims Act prohibits any person from knowingly presenting, or causing to be presented, to the federal government, a false claim for payment or knowingly making, or causing to be made, a false statement to get a false claim paid. Many pharmaceutical and other healthcare companies have been investigated and have reached substantial financial settlements with the federal government under the federal Anti-Kickback Statute and False Claims Acts for a variety of alleged marketing activities, including providing free product to customers with the expectation that the customers would bill federal programs for the product; providing consulting fees, grants, free travel, and other benefits to physicians to induce them to prescribe the company's products; and inflating prices reported to private price publication services, which are used to set drug payment rates under government healthcare programs. Companies have been prosecuted for causing false claims to be submitted because of the marketing of their products for unapproved uses. Pharmaceutical and other healthcare companies have also been prosecuted on other legal theories of Medicare and Medicaid fraud.
- The federal Health Insurance Portability and Accountability Act of 1996, as amended by the Health Information Technology for Economic and Clinical Health Act, among other things, imposes criminal and civil liability for executing a scheme to defraud any healthcare benefit program, and also imposes obligations with respect to safeguarding the privacy, security and transmission of individually identifiable health information.
- The federal Physician Payment Sunshine Act will require extensive tracking of payments to physicians and teaching hospitals, maintenance of a payments database, and public reporting of the payment data. CMS recently issued a final rule implementing the Physician Payment Sunshine Act provisions and clarified the scope of the reporting obligations, as well as that manufacturers must begin tracking on August 1, 2013 and must begin reporting payment data to CMS by March 31, 2014. Failure to comply with the reporting obligations may result in civil monetary penalties.
- Analogous state laws and regulations, such as state anti-kickback and false claims laws apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by Medicaid or other state programs or, in several states, apply regardless of the payer, Several states now require pharmaceutical companies to report expenses relating to the marketing and promotion of pharmaceutical products in those states and to report gifts and payments to individual health care providers in those states. Some of these states also prohibit certain marketing-related activities including the provision of gifts, meals, or other items to certain health care providers. In addition, several states require pharmaceutical companies to implement compliance programs or marketing codes.

Efforts to ensure that our business arrangements with third parties will continue to comply with applicable healthcare laws and regulations could be costly. It is possible that governmental authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law involving applicable fraud and abuse or other healthcare laws and regulations. If our operations, including activities conducted by our sales team, are found to be in violation of any of these laws or any other governmental regulations that may apply to us, we may be subject to significant civil, criminal and administrative penalties, damages, fines, exclusion from government-funded healthcare programs, such as Medicare and Medicaid, and the curtailment or

restructuring of our operations. If any of the physicians or other providers or entities with whom we expect to do business is found to be not in compliance with applicable laws, they may be subject to criminal, civil or administrative sanctions, including exclusions from government-funded healthcare programs.

We could become subject to government investigations and related subpoenas. Such subpoenas are often associated with previously filed qui tam actions, or lawsuits filed under seal under the Federal False Claims Act. Qui tam actions are brought by private plaintiffs suing on behalf of the federal government for alleged Federal False Claims Act violations. The time and expense associated with responding to such subpoenas, and any related qui tam or other actions, may be extensive, and we cannot predict the results of our review of the responsive documents and underlying facts or the results of such actions.

Responding to government investigations, defending any claims raised, and any resulting fines, restitution, damages and penalties, settlement payments or administrative actions, as well as any related actions brought by stockholders or other third parties, could have a material impact on our reputation, business and financial condition and divert the attention of our management from operating our business.

The number and complexity of both federal and state laws continues to increase, and additional governmental resources are being added to enforce these laws and to prosecute companies and individuals who are believed to be violating them. In particular, the Healthcare Reform Act includes a number of provisions aimed at strengthening the government's ability to pursue anti-kickback and false claims cases against pharmaceutical manufacturers and other healthcare entities, including substantially increased funding for healthcare fraud enforcement activities, enhanced investigative powers, and amendments to the False Claims Act that make it easier for the government and whistleblowers to pursue cases for alleged kickback and false claim violations. While it is too early to predict what effect these changes will have on our business, we anticipate that government scrutiny of pharmaceutical sales and marketing practices will continue for the foreseeable future and subject us to the risk of government investigations and enforcement actions. Responding to a government investigation or enforcement action would be expensive and time-consuming, and could have a material adverse effect on our business and financial condition and growth prospects.

If we fail to comply with our reporting and payment obligations under the Medicaid Drug Rebate Program or other governmental pricing programs, we could be subject to additional reimbursement requirements, penalties, sanctions and fines which could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We participate in the Medicaid Drug Rebate Program and a number of other Federal and state government pricing programs, and we may participate in additional government pricing programs in the future. These programs are described in detail in the "Business—Regulatory Matters" section of this Form 10-K and generally require us to pay rebates or provide discounts to government payers in connection with drugs, including JUXTAPID, that are dispensed to beneficiaries of these programs. In some cases, such as with the Medicaid Drug Rebate Program, the rebates are based on pricing and rebate calculations that we report on a monthly and quarterly basis to the government agencies that administer the programs. The terms, scope and complexity of these government pricing programs change frequently. Responding to current and future changes may increase our costs and the complexity of compliance, will be time-consuming, and could have a material adverse effect on our results of operations.

Pricing and rebate calculations vary among products and programs. The calculations are complex and are often subject to interpretation by governmental or regulatory agencies and the courts. For example, the Medicaid rebate amount is computed each quarter based on our submission to the CMS of our current AMP and BP for the quarter. If we become aware that our reporting for prior quarters was incorrect, or has changed as a result of recalculation of the pricing data, we will be obligated to resubmit the corrected data for a period not to exceed 12 quarters from the quarter in which the data originally were due. Such restatements and recalculations would serve to increase our costs for complying with the laws and regulations governing the Medicaid rebate program.

Any corrections to our rebate calculations could result in an overage or underage in our rebate liability for past quarters, depending on the nature of the correction. Price recalculations also may affect the price that we will be required to charge certain safety-net providers under the Public Health Service 340B drug discount program.

We are liable for errors associated with our submission of pricing data and for overcharging government payers. For example, in addition to retroactive rebates and the potential for 340B program refunds, if we are found to have knowingly submitted false AMP or BP information to the government, we may be liable for civil monetary penalties in the amount of $100,000 per item of false information. Our failure to submit monthly/quarterly AMP and BP data on a timely basis could result in a civil monetary penalty of $10,000 per day for each day the submission is late beyond the due date. In the event that CMS were to terminate our rebate agreement, no federal payments would be available under Medicaid or Medicare Part B for our covered out-patient drugs. In addition, if we overcharge the government in connection with our FSS contract or under any other government program, we will be required to refund the difference to the government. Failure to make necessary disclosures and/or to identify contract overcharges could result in allegations against us under the Federal False Claims Act and other laws and regulations.

Unexpected refunds to the government, and responding to a government investigation or enforcement action, would be expensive and time-consuming, and could have a material adverse effect on our business, financial condition, results of operations and growth prospects.

We are transitioning our business to focus on the commercialization of JUXTAPID, and we may require third-party relationships to enable this transition, which may have an adverse effect on our business.

We will need to continue to transition from a company with a development focus to a company focused on supporting commercial activities. We may not be successful in such a transition. We have not yet demonstrated an ability to successfully commercialize a product candidate. As a result, we may not be as successful as companies that have previously obtained marketing approval for drug candidates and commercially launched drugs. To maximize the commercial potential of JUXTAPID, we plan to utilize distributors and other third parties to help distribute and, in some cases, to commercialize the product, if approved, in certain geographic locations outside of the U.S. and the major market countries in the EU In those geographic locations in which we are using third parties to commercialize our product, we will be reliant on such strategic partners to generate revenue on our behalf.

If we enter into arrangements with third parties to perform sales, marketing or distribution services, our product revenues or the profitability of these product revenues to us are likely to be lower than if we were to market and sell any products that we develop ourselves. In addition, we may not be successful in entering into arrangements with third parties to sell and market our products or in doing so on terms that are favorable to us. We likely will have little control over such third parties, and any of them may fail to devote the necessary resources and attention to sell and market our products effectively. If we do not establish sales and marketing capabilities successfully, either on our own or in collaboration with third parties, we will not be successful in commercializing our products.

If we pursue development of JUXTAPID for broader patient populations, we likely will be subject to stricter regulatory requirements, product development will be more costly and commercial pricing for any approved indication would likely be lower.

Clinical development of JUXTAPID in broader HeFH patient populations would involve clinical trials with larger numbers of patients, with such patients possibly taking the drug for longer periods of time. This would be costly, and could take many years to complete. In addition, we believe that the FDA and, in some cases, the EMA would require a clinical outcomes study, for example, demonstrating a reduction in cardiovascular events in broader patient populations, either prior to or after the submission of an application for marketing approval for these broader

indications. Clinical outcomes studies are particularly expensive and time consuming to conduct because of the larger number of patients required to establish that the drug being tested has the desired effect. It may also be more difficult for us to demonstrate the desired outcome in these studies than to achieve validated surrogate endpoints, such as the primary efficacy endpoint of our pivotal Phase 3 clinical trial of JUXTAPID for the treatment of patients with HoFH of percent change in LDL-C levels from baseline. In addition, in considering approval of JUXTAPID for broader patient populations with less severely elevated lipid levels, the FDA and other regulatory authorities may place greater emphasis on the side effect and risk profile of the drug in comparison to the drug's efficacy and potential clinical benefit than in smaller, more severely afflicted patient populations. These factors may make it more difficult for us to achieve marketing approvals of JUXTAPID for these broader patient populations.

If we are able to successfully develop and obtain marketing approval of JUXTAPID in these broader patient populations, we may not be able to obtain the same pricing level that we secure for use of JUXTAPID for orphan indications. The pricing of some drugs intended for orphan populations is often related to the size of the patient population, with smaller patient populations often justifying higher prices. If the pricing for JUXTAPID is lower in broader patient populations, we may not be able to maintain higher pricing in the population of more severely afflicted patients. This would lead to a decrease in revenue from sales to more severely afflicted patients, and could make it more difficult for us to achieve or maintain profitability.

In addition, if one of our product candidates receives marketing approval for a broader indication than its orphan designation, we may not be able to maintain orphan drug exclusivity or such orphan drug exclusivity may be circumvented by a third-party competitor.

Failures or delays in the commencement or completion of clinical testing could result in increased costs to us and delay, prevent or limit our ability to generate revenue with respect to the relevant product candidate or new indication.

The commencement and completion of clinical trials may be delayed or prevented for a number of reasons, including:

- difficulties obtaining regulatory clearance to commence a clinical trial or complying with conditions imposed by a regulatory authority regarding the scope or term of a clinical trial;
- delays in reaching or failing to reach agreement on acceptable terms with prospective contract research organizations ("CROs"), and trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different CROs and trial sites;
- insufficient or inadequate supply or quality of a product candidate or other materials necessary to conduct our clinical trials, or other manufacturing issues;
- difficulties obtaining institutional review board ("IRB") approval to conduct a clinical trial at a prospective site;
- challenges recruiting and enrolling patients to participate in clinical trials for a variety of reasons, including the size and nature of a patient population, the proximity of patients to clinical sites, the eligibility criteria for the trial, the nature of trial protocol, the availability of approved effective treatments for the relevant disease and the competition from other clinical trial programs for similar indications;
- severe or unexpected drug-related side effects experienced by patients in a clinical trial; and
- difficulties retaining patients who have enrolled in a clinical trial but may be prone to withdraw due to the rigors of the trials, lack of efficacy, side effects or personal issues, or who are lost to further follow-up.

Clinical trials may also be delayed or terminated as a result of ambiguous or negative interim results or the results of other clinical, preclinical or nonclinical studies. In addition, a clinical trial may be suspended or terminated by us, the FDA, the IRBs at the sites where the IRBs are overseeing a trial, or a data safety monitoring board overseeing the clinical trial at issue, or other regulatory authorities due to a number of factors, including:

- failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols;
- inspection of the clinical trial operations or trial sites by the FDA or other regulatory authorities;
- unforeseen safety issues or lack of effectiveness; and
- lack of adequate funding to continue the clinical trial.

Positive results in preclinical studies and earlier clinical trials of our product candidates may not be replicated in later clinical trials, which could result in development delays or a failure to obtain marketing approval.

Positive results in preclinical or clinical studies of JUXTAPID or any other product candidate that we develop may not be predictive of similar results in humans during further clinical trials. A number of companies in the pharmaceutical and biotechnology industries have suffered significant setbacks in late-stage clinical trials even after achieving promising results in early-stage development. Accordingly, there is, for example, a possibility that our planned nonclinical study in rodent models or clinical studies of JUXTAPID in pediatric HoFH patients or our clinical program to seek approval of JUXTAPID in Japan in adult patients with HoFH may generate results that are not consistent with the results of our Phase 3 clinical study. Our preclinical studies or clinical trials for any product candidate may produce negative or inconclusive results, and we may decide, or regulators may require us, to conduct additional preclinical studies or clinical trials. Moreover, preclinical and clinical data are often susceptible to varying interpretations and analyses, and many companies that have believed their product candidates performed satisfactorily in preclinical studies and clinical trials have nonetheless failed to obtain FDA or other regulatory approval for their products.

Changes in regulatory requirements and guidance or unanticipated events during our clinical trials may occur, as a result of which we may need to amend clinical trial protocols. Amendments may require us to resubmit our clinical trial protocols to IRBs for review and approval, which may impact the cost, timing or successful completion of a clinical trial. If we experience delays in completion of, or if we terminate, any of our clinical trials of JUXTAPID the commercial prospects for JUXTAPID may be harmed.

If we fail to obtain or maintain orphan drug exclusivity for JUXTAPID in any country where exclusivity is available, we will have to rely on our data and marketing exclusivity, if any, and on our intellectual property rights, to the extent there is coverage in such country, which may reduce the length of time that we can prevent competitors from selling generic versions of JUXTAPID.

We have obtained orphan drug exclusivity for JUXTAPID in the U.S. for the treatment of HoFH. Under the Orphan Drug Act, the FDA may designate a product as an orphan drug if it is a drug intended to treat a rare disease or condition, defined, in part, as a patient population of fewer than 200,000 in the U.S. In the U.S., the company that first obtains FDA approval for a designated orphan drug for the specified rare disease or condition receives orphan drug marketing exclusivity for that drug for a period of seven years. This orphan drug exclusivity prevents the FDA from approving another application, including a full NDA, to market the same drug for the same orphan indication during the exclusivity period, except in very limited circumstances. For purposes of small molecule drugs, the FDA defines "same drug" as a drug that contains the same active molecule and is intended for the same use as the drug in question. A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, orphan drug exclusive marketing rights in the U.S. may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantity of the drug to meet the needs of patients with the rare disease or condition.

The EMA grants orphan drug designation to promote the development of products that may offer therapeutic benefits for life-threatening or chronically debilitating conditions affecting not more than five in 10,000 people in the EU. Orphan drug designation from the EMA provides ten years of marketing exclusivity following drug approval, subject to reduction to six years if the designation criteria are no longer met. Despite the prevalence rate, we will not have orphan drug exclusivity for lomitapide in the treatment of HoFH in the EU since the EMA views the relevant condition, for orphan drug purposes, to include HoFH and HeFH. Our failure to obtain orphan drug designation for lomitapide for the treatment of HoFH in the EU means that, if approved, we will not have the benefit of the orphan drug market exclusivity for this indication in the EU, and, as a result, will need to rely on our intellectual property rights and other exclusivity provisions. Our European patents directed towards the composition of matter of JUXTAPID are scheduled to expire in 2016, and may additionally qualify for a supplemental certificate that would provide extended patent protection for up to five years after patent expiration upon marketing approval in the EU. In addition, lomitapide qualifies as a new chemical entity in the EU. In the EU, new chemical entities qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the EU from assessing a generic application for eight years, after which generic marketing authorization can be submitted, but a generic may not be marketed for two years. If we do not obtain extended patent protection and data exclusivity for our product candidates, our business may be materially harmed.

There are many countries where neither orphan drug exclusivity nor data and marketing exclusivity are available. Even if we obtain orphan drug exclusivity for a product, that exclusivity may not effectively protect the product from competition because different drugs can be approved for the same condition. In the U.S., even after an orphan drug is approved, the FDA can subsequently approve the same drug for the same condition if the FDA concludes that the later drug is safer, more effective or makes a major contribution to patient care.

Recent federal legislation and actions by state and local governments may permit re-importation of drugs from foreign countries into the U.S., including foreign countries where the drugs are sold at lower prices than in the U.S., which could materially adversely affect our operating results and our overall financial condition.

We may face competition in the U.S. for JUXTAPID or any other product candidate, if approved, from lower priced products from foreign countries that have placed price controls on pharmaceutical products. This risk may be particularly applicable to drugs such as JUXTAPID that are formulated for oral delivery and expected to command a premium price. The MMA contains provisions that may change importation laws and expand pharmacists' and wholesalers' ability to import lower priced 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 may result in a significant reduction in the cost of products to consumers. The Secretary of Health and Human Services has not yet announced any plans to make this required certification.

A number of federal legislative proposals have been made to implement the changes to the U.S. importation laws without any certification, and to broaden permissible imports in other ways. Even if the changes do not take effect, and other changes are not enacted, imports from Canada and elsewhere may continue to increase due to market and political forces, and the limited enforcement resources of the FDA, Customs and Border Protection and other government agencies. For example, Pub. L. No. 112-74, which was signed into law in December 2011, and provides appropriations for the Department of Homeland Security for the 2012 fiscal year, expressly prohibits Customs and Border Protection from using funds to prevent individuals from importing from Canada less than a 90-day supply of a prescription drug for personal use, when the drug otherwise complies with the Federal Food, Drug, and Cosmetic Act ("FDCA"). Further, several states and local governments have implemented importation schemes for their citizens and, in the absence of federal action to curtail such activities, we expect other states and local governments to launch importation efforts.

The importation of foreign products that compete with JUXTAPID or any other product candidate for which we obtain marketing approval could negatively impact our revenue and profitability, possibly materially.

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

The use of JUXTAPID or any other product candidate in clinical trials and the sale of JUXTAPID or any other product candidate for which we obtain marketing approval expose us to the risk of product liability claims. Product liability claims might be brought against us by consumers, healthcare providers or others selling or otherwise coming into contact with our product and product candidates. If we cannot successfully defend ourselves against product liability claims, we could incur substantial liabilities. In addition, regardless of merit or eventual outcome, product liability claims may result in:

- decreased demand for JUXTAPID or any other product candidate for which we obtain marketing approval;
- impairment of our business reputation and exposure to adverse publicity;
- increased warnings on product labels;
- withdrawal of clinical trial participants;
- costs as a result of related litigation;
- distraction of management's attention from our primary business;
- substantial monetary awards to patients or other claimants;
- loss of revenue; and
- the inability to successfully commercialize JUXTAPID or any other product candidate for which we obtain marketing approval.

We have obtained product liability insurance coverage for both our clinical trials and our commercial exposures with a $20.0 million annual aggregate coverage limit. However, our insurance coverage may not be sufficient to reimburse us for any expenses or losses we may suffer. Moreover, insurance coverage is becoming increasingly expensive, and, 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 due to liability. On occasion, 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. A successful product liability claim or series of claims brought against us could cause our stock price to decline and, if judgments exceed our insurance coverage, could decrease our cash and adversely affect our business.

A variety of risks associated with our planned international business relationships could materially adversely affect our business.

We plan to seek approval to market JUXTAPID ourselves in certain countries outside the U.S., and to enter into agreements with third parties for the commercialization of JUXTAPID in other international markets, if approved. If we do so, we would be subject to additional risks related to entering into international business relationships, including:

- differing regulatory requirements for drug approvals in foreign countries;
- potentially reduced protection for intellectual property rights;
- the potential for so-called parallel importing, which is what happens when a local seller, faced with high or higher local prices, opts to import goods from a foreign market, with low or lower prices, rather than buying them locally;
- unexpected changes in tariffs, trade barriers and regulatory requirements;

- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees working or traveling abroad;
- foreign taxes;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the U.S.;
- production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- dependence upon third parties to perform distribution, quality control testing, collections and other aspects of the distribution, supply chain and commercialization of our products that are required to be performed in order to conduct such activities in international markets; and
- business interruptions resulting from geo-political actions, including war and terrorism, or natural disasters, including earthquakes, volcanoes, typhoons, floods, hurricanes and fires.

These and other risks of international business relationships may materially adversely affect our ability to attain or sustain profitable operations.

Risks Related to Our Intellectual Property

If our patent position does not adequately protect our product and product candidates, others could compete against us more directly, which would harm our business, possibly materially.

Our JUXTAPID patent portfolio consists of five issued U.S. patents and issued patents in parts of Europe, Canada, Israel, Australia, New Zealand and Japan and pending applications in the U.S., Europe, Australia, Japan, Canada, India and South Korea, all of which have been licensed to us in a specific field. The U.S. patent covering the composition of matter of JUXTAPID is scheduled to expire in 2015, but we have filed an application for patent term extension for this patent. The non-U.S. patents directed to the composition of matter of JUXTAPID are scheduled to expire in 2016, but may also be eligible for extensions in certain countries. Our method of use patent covering certain dosing regimens for JUXTAPID expires in 2027 in the U.S., and in 2025 in the EU. Our commercial success will depend significantly on our ability to obtain additional patents and protect our existing patent position as well as our ability to maintain adequate protection of other intellectual property for our technologies, product candidates and any future products in the U.S. and other countries. If we do not adequately protect our intellectual property, 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. Our ability to use the patents and patent applications licensed to us to protect our business will also depend on our ability to comply with the terms of the applicable licenses and other agreements. The laws of some foreign countries do not protect our proprietary rights to the same extent as the laws of the U.S., and we may encounter significant problems in protecting our proprietary rights in these countries.

The patent positions of biotechnology and pharmaceutical companies, including our patent position, involve complex legal and factual questions, and, therefore, validity and enforceability cannot be predicted with certainty. Patents may be challenged, deemed unenforceable, invalidated or circumvented. We will be able to protect our proprietary rights from unauthorized use by third parties only to the extent that our proprietary technologies, product candidates and any future products are covered by valid and enforceable patents or are effectively maintained as trade secrets.

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

- we will be able to successfully commercialize our product before some or all of our relevant patents expire, or in countries where we do not have patent protection;
- we or our licensors were the first to make the inventions covered by each of our pending patent applications;
- we or our licensors were the first to file patent applications for these inventions;
- others will not independently develop similar or alternative technologies or duplicate any of our technologies;
- any of our pending patent applications or those we have licensed will result in issued patents;
- any of our patents or those we have licensed will be valid or enforceable;
- any patents issued to us or our licensors and collaborators will provide a basis for any additional 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 patentable; or
- the patents of others will not have an adverse effect on our business.

If we do not obtain protection under the Hatch-Waxman Amendments and similar foreign legislation by extending the patent terms and obtaining data exclusivity for our product or product candidates, our business may be materially harmed.

The Hatch-Waxman Amendments permit a patent restoration term of up to five years as compensation for patent term lost during product development and the FDA regulatory review process. Our U.S. composition of matter patent for JUXTAPID is scheduled to expire in 2015, and we have filed an application for patent term extension for this patent. We also plan to apply for restorations or extensions of the term of certain patents outside the U.S. in those countries where such a mechanism is available. However, we may not be granted an extension because of, for example, failing to apply within applicable deadlines, failing to apply prior to expiration of relevant patents or otherwise failing to satisfy applicable requirements. Moreover, the applicable time period or the scope of patent protection afforded could be less than we request. If we are unable to obtain patent term extension or restoration or the term of any such extension is less than we request, our competitors may obtain approval of competing products following our patent expiration, and our revenue could be reduced, possibly materially.

In addition, the FDA has classified JUXTAPID as a new chemical entity in the U.S. and it is therefore eligible for data exclusivity under the Hatch-Waxman Amendments. A drug can be classified as a new chemical entity if the FDA has not previously approved any other new drug containing the same active moiety. Under sections 505(c)(3)(E)(ii) and 505(j)(5)(F)(ii) of the FDCA, as amended, a new chemical entity that is granted marketing approval may, even in the absence of patent protection, be eligible for five years of data exclusivity in the U.S. following marketing approval.

This data exclusivity precludes submission of 505(b)(2) applications or abbreviated new drug applications submitted by another company that references the new chemical entity application for four years if certain patents covering the new chemical entity or its method of use expire or are challenged by a generic applicant. In the EU, new chemical entities qualify for eight years of data exclusivity upon marketing authorization and an additional two years of market exclusivity. This data exclusivity, if granted, prevents regulatory authorities in the EU from assessing a generic application for eight years, after which generic marketing authorization can be submitted, but a generic may not be marketed for two years. If we are not able to gain or exploit the period of data exclusivity, we may face significant competitive threats to our commercialization of these compounds from other manufacturers, including the manufacturers of generic alternatives. Further, even if our compounds are considered to be new chemical entities and we are able to gain the prescribed period of data exclusivity, another company nevertheless could also market another version of the drug if such company can complete a full NDA with a complete human clinical trial process and obtain marketing approval of its product.

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

We may 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 and other advisors to protect our trade secrets and other proprietary information. These agreements may not effectively prevent disclosure of confidential information, and may not provide an adequate remedy in the event of unauthorized disclosure of confidential information. In addition, others may independently discover our trade secrets and proprietary information. For example, the FDA, as part of its Transparency Initiative, currently is considering whether to make additional information publicly available on a routine basis, including information that we may consider to be trade secrets or other proprietary information, and it is not clear at the present time how the FDA's disclosure policies may change in the future, if at all. Costly and time-consuming litigation could be necessary to enforce and determine the scope of our proprietary rights, and failure to obtain or maintain trade secret protection could adversely affect our competitive business position.

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 our product and any product candidates.

Our success will depend in part on our ability to operate without infringing the proprietary rights of third parties. There could be issued patents of which we are not aware that our products or product candidates infringe. There also could be patents that we believe we do not infringe, but that we may ultimately be found to infringe. Moreover, patent applications are in some cases maintained in secrecy until patents are issued. The publication of discoveries in the scientific or patent literature frequently occurs substantially later than the date on which the underlying discoveries were made and patent applications were filed. Because patents can take many years to issue, there may be currently pending applications of which we are unaware that may later result in issued patents that our products or product candidates infringe. For example, pending applications may exist that provide support or can be amended to provide support for a claim that results in an issued patent that our product infringes.

The pharmaceutical industry is characterized by extensive litigation regarding patents and other intellectual property rights. Other parties may obtain patents in the future and allege that our products or product candidates or the use of our technologies infringes these patent claims or that we are employing their proprietary technology without authorization. Likewise, third parties may challenge or infringe upon our existing or future patents.

Proceedings involving our patents or patent applications or those of others could result in adverse decisions regarding:

- the patentability of our inventions relating to our product or any product candidates; and
- the enforceability, validity or scope of protection offered by our patents relating to our product or any product candidates.

Even if we are successful in these proceedings, we may incur substantial costs and divert management's 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. Patent litigation is costly and time consuming. We may not have sufficient resources to bring these actions to a successful conclusion.

In addition, if we do not obtain a license, develop or obtain non-infringing technology, fail to defend an infringement action successfully or have infringed patents declared invalid, we may:

- incur substantial monetary damages;
- encounter significant delays in bringing our product candidates to market; and
- be precluded from manufacturing or selling our product candidates.

In such event, our business could be adversely affected, possibly materially.

If we fail to comply with our obligations in our license agreements for our product candidates, we could lose license rights that are important to our business.

Our existing license agreements impose, and we expect any future license agreements that we enter into will impose, various diligence, milestone payment, royalty, insurance and other obligations on us.

In addition, our license agreement with UPenn limits the field of use for JUXTAPID as a monotherapy or in combination with other dyslipidemic therapies for treatment of patients with HoFH, or for the treatment of patients with severe hypercholesterolemia unable to come within 15% of the NCEP LDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe combined hyperlipidemia unable to come within 15% of the NCEP non-HDL-C goal on maximal tolerated oral therapy, as determined by the patient's prescribing physician, or with severe hypertriglyceridemia unable to reduce TG levels to less than 1,000 mg/dL on maximal tolerated therapy. If we fail to comply with the obligations and restrictions under our license agreements, including the limited field of use under our license agreement with UPenn, the applicable licensor may have the right to terminate the license, in which case we might not be able to market any product that is covered by the licensed patents. Any breach or termination of our license agreement with UPenn would have a particularly significant adverse effect on our business because of our reliance on the commercial success of JUXTAPID. Although we intend to comply with the restrictions on field of use in our license agreement with UPenn by seeking product labels for JUXTAPID that are consistent with the license field, we may still be subject to the risk of breaching the license agreement if we are deemed to be promoting or marketing JUXTAPID for an indication not covered by any product label that we are able to obtain. In addition, because this restriction on the field of use limits the indications for which we can develop JUXTAPID, the commercial potential of JUXTAPID may not be as great as without this restriction.

Risks Related to Our Dependence on Third Parties

If we are unable to establish and maintain effective sales, marketing and distribution capabilities, or to enter into agreements with third parties to do so, we will be unable to successfully commercialize JUXTAPID.

We are marketing and selling JUXTAPID for HoFH directly in the U.S. using our own marketing and sales resources. If lomitapide is approved outside the U.S., we plan to market and sell lomitapide directly, using our own marketing and sales resources in certain key countries of Europe and in several other countries where it makes business sense to do so. We plan to use third parties to provide warehousing, shipping and other distribution services on our behalf in those countries. We may selectively seek to establish distribution and similar forms of arrangements to reach patients with HoFH in geographies that we do not believe we can cost-effectively address with our own sales and marketing capabilities. If we are unable to establish the capabilities to sell, market and distribute JUXTAPID, either through our own capabilities or by entering into arrangements with others, or if we are unable to enter into distribution agreements in those countries we do not believe we can cost-effectively address with our own sales and marketing capabilities, we may not be able to successfully sell JUXTAPID. We cannot guarantee that we will be able to establish and maintain our own capabilities or to enter into and maintain any distribution agreements with third-parties on acceptable terms, if at all. Additionally, we currently have a contract with a single specialty pharmacy distributor in the U.S. Any performance failure on the part of our existing distributor could impair our marketing and sales of JUXTAPID. Furthermore, our expenses associated with building up and maintaining the sales force and distribution capabilities around the world may be disproportionate compared to the revenues we may be able to generate on sales of JUXTAPID. We cannot guarantee that we will be successful in commercializing JUXTAPID.

We rely on third parties to conduct our clinical trials and to perform related services, and those third parties may not perform satisfactorily, including failing to meet established deadlines for the completion of such clinical trials. We may become involved in commercial disputes with these parties.

We do not have the ability to independently conduct clinical trials, and we rely on third parties such as CROs, medical institutions, academic institutions, and clinical investigators to perform this function. Our reliance on these third parties for clinical development activities reduces our control over these activities. However, if we sponsor clinical trials, we are responsible for ensuring that each of our clinical trials is conducted in accordance with the general investigational plan and protocols for the trial, even if we use CROs. Moreover, the FDA requires us to comply with standards, commonly referred to as GCPs, for conducting, recording, and reporting the results of clinical trials to assure that data and reported results are credible and accurate and that the rights, integrity and confidentiality of trial participants are protected. Our reliance on third parties that we do not control does not relieve us of these responsibilities and requirements. Furthermore, these third parties may also have relationships with other entities, some of which may be our competitors. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they provide is compromised due to the failure to adhere to regulatory requirements or our clinical trial protocols, or for other reasons, our development programs may be extended, delayed or terminated, additional marketing approvals for JUXTAPID or any other product candidate may be delayed or denied in the targeted indication, and we may be delayed or precluded in our efforts to successfully commercialize JUXTAPID or any other product candidate for targeted indications.

In addition, we may from time to time become involved in commercial disputes with these third parties, for example regarding the quality of the services provided by these third parties or our ultimate liability to pay for services they purported to provide on our behalf, or the value of such services. Due to our reliance on third-party service providers, we may experience commercial disputes such as this in the future. In some cases, we may be required to pay for work that was not performed to our specifications or not utilized by us, and these obligations may be material.

We do not have drug research or discovery capabilities, and will need to acquire or license existing drug compounds from third parties to expand our product candidate pipeline.

JUXTAPID has been licensed to us by UPenn. We currently have no drug research or discovery capabilities. Accordingly, if we are to expand our product candidate pipeline, we will need to acquire or license existing compounds from third parties. In addition, our right to use JUXTAPID is limited to specified patient populations, such as patients with HoFH, severe hypercholesterolemia or severe hypertriglyceridemia. Accordingly, if we wished to expand the development of JUXTAPID to address other indications, we would need to expand our license agreement with UPenn and potentially acquire rights from BMS. We will face significant competition in seeking to acquire or license promising drug compounds. Many of our competitors for such promising compounds may have significantly greater financial resources and more extensive experience in preclinical testing and clinical trials, obtaining regulatory approvals and manufacturing and marketing pharmaceutical products, and thus, may be a more attractive option to a potential licensor than us. If we are unable to acquire or license additional promising drug compounds, we will not be able to expand our product candidate pipeline.

Risks Related to Employee Matters and Managing Growth

Our future success depends on our ability to hire and retain our key executives and to attract, retain, and motivate qualified personnel.

We are highly dependent on Marc Beer, our Chief Executive Officer, and the other principal members of our executive, commercial, medical, and development teams. We have entered into employment agreements with certain members of our executive, commercial, medical and development teams, but any employee may terminate his or her employment with us at any time. We do not maintain "key man" life insurance for any of our employees. The loss of the services of any of these persons might impede the achievement of our development and commercialization objectives.

We expect to continue hiring qualified personnel. Recruiting and retaining qualified personnel will 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.

In addition, as a result of becoming a public company, we need to continue to establish and maintain effective disclosure and financial controls and make changes in our corporate governance practices. Failure to maintain adequate controls could impact the quality and integrity of our financial statements and cause us reputational harm.

In addition, we rely on consultants and advisors, including scientific, manufacturing, clinical, regulatory, pharmacovigilance and sales and marketing advisors, to assist us in formulating our development, manufacturing 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.

We will need to grow our organization, and we may encounter difficulties in managing this growth, which could disrupt our operations.

We currently have approximately 110 employees, and we expect to experience significant growth in the number of our employees and the scope of our operations. To manage our anticipated future growth, we must continue to implement and improve our managerial, operational and financial systems, expand our facilities and continue to recruit and train additional qualified personnel. Our management may need to divert a disproportionate amount of its attention away from our day-to-day activities, and devote a substantial amount of time to managing these growth 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, which may result in weaknesses in our infrastructure, give rise to operational mistakes, loss of business opportunities, loss of employees and reduced productivity among remaining employees. The physical expansion of our operations may lead to significant costs, and may divert financial resources from other projects. If our management is unable to effectively manage our expected growth, our expenses may increase more than expected, 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 JUXTAPID successfully, and to compete effectively will depend, in part, on our ability to effectively manage any future growth.

Risks Related to Our Financial Position and Capital Requirements

We have incurred significant operating losses since our inception, and anticipate that we will incur continued losses for the near future.

We have a limited operating history. To date, we have primarily focused on developing our lead compound, JUXTAPID. We have funded our operations to date primarily through proceeds from the private placement of convertible preferred stock, convertible debt, venture debt, bank debt, the proceeds from our initial public offering and the proceeds from our June 2011, June 2012 and January 2013 public offerings. We have incurred losses in each year since our inception in February 2005. As of December 31, 2012, we had an accumulated deficit of approximately $192.7 million. Substantially all of our operating losses resulted from costs incurred in connection with our development programs and from general and administrative costs associated with our operations. The losses we have incurred to date, 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 expenses to increase in the near-term as a result of spending on commercial launch of JUXTAPID in the U.S., and possible distribution of lomitapide in other countries as part of named patient supply or compassionate use or on a commercial basis, if approved; completion of our drug product manufacturing validation campaign; hiring of additional key personnel in the U.S., Europe and other countries; plans to conduct a clinical development program to support an

application for marketing approval of JUXTAPID in Japan in adult patients with HoFH; the initiation of a juvenile animal toxicology study, and clinical study of JUXTAPID in the treatment of pediatric patients with HoFH; the conduct of our observational cohort study as part of our post-marketing commitments to the FDA; and other possible clinical development activities. We expect to incur significant sales, marketing, and outsourced manufacturing expenses, as well as continued research and development expenses. In addition, we have incurred, and expect to continue to incur, additional costs associated with operating as a public company. As a result, we expect to continue to incur significant operating losses at least in 2013 and 2014 and potentially in subsequent years.

Because of the numerous risks and uncertainties associated with developing and commercializing pharmaceutical products, we are unable to predict with certainty the extent of any future losses or when we will become profitable, if at all.

We have not generated any significant revenue from JUXTAPID or any other product candidate and may never be profitable.

Our ability to become profitable depends upon our ability to generate revenue. As of December 31, 2012, we had not generated any revenue from any product, including JUXTAPID. Our ability to generate significant revenue depends on a number of factors, including our ability to:

- successfully launch JUXTAPID in the U.S.;
- successfully launch JUXTAPID in the EU, and other international markets, if approved;
- obtain market acceptance by patients, physicians and payers for JUXTAPID as a treatment for HoFH;
- obtain reimbursement and pricing for JUXTAPID sufficient to allow us to sell JUXTAPID on a competitive and profitable basis; and
- have sufficient commercial quantities of JUXTAPID manufactured.

JUXTAPID may not gain market acceptance or achieve commercial success. In addition, we anticipate incurring significant costs associated with commercializing JUXTAPID. We may not achieve profitability soon after generating product revenue, if ever. If we are unable to generate significant product revenue, we will not become profitable, and may be unable to continue operations without continued funding.

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

We may, in the future, need to raise additional capital to fund our operations, and to further commercialize and develop JUXTAPID. Our future capital requirements may be substantial and will depend on many factors including:

- the level of physician, patient and payer acceptance of JUXTAPID, and the success of our commercialization efforts;
- the decisions of the EMA with respect to our applications for marketing approval of JUXTAPID for the treatment of adult patients with HoFH in the EU; the costs of activities related to the EU regulatory approval process; the timing of approvals in the EU, if received; and the nature and timing of pricing approvals in the various countries in the EU;
- the decisions of various countries outside the U.S. and EU with respect to approval of lomitapide, and reimbursement and pricing decisions in such countries, if approved;
- the timing and cost of the planned juvenile animal toxicology study, and an anticipated clinical trial to evaluate JUXTAPID for treatment of pediatric patients with HoFH;
- the cost of establishing and maintaining the sales and marketing capabilities necessary for commercial launch of JUXTAPID in HoFH in the U.S. and in the EU and certain other key international markets, if approved;

- the timing and cost of our planned clinical development program of JUXTAPID in HoFH in Japanese patients;
- the cost of filing, prosecuting and enforcing patent claims;
- the costs of our manufacturing-related activities;
- the costs associated with commercializing JUXTAPID;
- the levels, timing and collection of revenue received from sales of approved products in the future;
- the cost of our observational cohort study as part of our post marketing commitment to the FDA;
- the timing and cost of other clinical development activities; and
- the timing and costs of future business development opportunities.

In November 2011, we filed a shelf registration statement on Form S-3 with the SEC, which became effective in December 2011. This shelf registration statement permitted us to offer, from time to time, any combination of common stock, preferred stock, debt securities and warrants of up to an aggregate of $125 million. In June 2012, we completed an underwritten public offering of 3,400,000 shares of common stock at a price to the public of $14.75 per share pursuant to our Form S-3 registration statement. The net proceeds to us from this offering were approximately $47.0 million, after deducting underwriting discounts, commissions, and other offering expenses. In July 2012, the underwriters exercised their option to purchase an additional 393,085 shares of common stock. The net proceeds to us from the issuance and sale of the additional shares were approximately $5.6 million, after deducting underwriting discounts, commissions, and other offering expenses. In January 2013, after increasing the amount of the shelf registration through an amendment to the Form S-3, we sold 3,110,449 shares of our common stock in an underwritten public offering at a price to the public of $26.64 per share. The net proceeds to us from this offering were approximately $78.3 million, after deducting underwriting discounts and commissions. As a result of the June 2012 and January 2013 public offerings, we have fully utilized the Form S-3 shelf registration statement filed in November 2011. On February 15, 2013, we filed an automatic shelf registration statement on Form S-3 ASR with the SEC. This shelf registration statement permits us to offer, from time to time, an unspecified amount of any combination of common stock, preferred stock, debt securities and warrants.

In March 2012, we entered into a Loan and Security Agreement with Silicon Valley Bank, pursuant to which Silicon Valley Bank made a term loan to us in the principal amount of $10.0 million. The Loan and Security Agreement provides for interest-only payments through February 28, 2013, with per annum interest of 6.75% and a final payment of $0.2 million.

We used the proceeds of the term loan to fully repay our existing loan from Hercules Technology II, L.P. and Hercules Technology III, L.P. The Loan and Security Agreement provides that we will repay the principal balance of the term loan in 36 monthly installments starting on March 1, 2013, and continuing through February 1, 2016. The remaining term loan principal balance and all accrued but unpaid interest will be due and payable on February 1, 2016. We may prepay all or any part of the outstanding term loan subject to a prepayment premium (defined in the Loan and Security Agreement) at our option. In connection with the Loan and Security Agreement, we granted Silicon Valley Bank a security interest in all of our personal property now owned or hereafter acquired, excluding intellectual property (and a negative pledge on intellectual property). The Loan and Security Agreement also provides for standard indemnification of Silicon Valley Bank and contains representations, warranties and certain covenants (including the agreement by us to maintain a specified level of liquidity). In July 2012, we entered into an arrangement with Silicon Valley Bank under the Loan and Security Agreement, pursuant to which we received a line of credit of up to $0.8 million to finance, subject to the terms of the Loan and Security Agreement, the purchase of certain types of equipment acquired by us during the two years ended December 31, 2012. As of December 31, 2012, we have financed approximately $0.6 million under this arrangement and the remainder of the line of credit expired unused.

We may pursue opportunities to obtain additional external financing in the future through debt and equity financing, lease arrangements related to facilities and capital equipment, collaborative research and development agreements, and license agreements.

We anticipate that our existing cash and cash equivalents will be sufficient to enable us to maintain our currently planned operations, including our continued product candidate development. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate.

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 we are unable to obtain additional financing, we may be required to reduce the scope of our planned development, sales and marketing efforts, which could harm our business, financial condition and operating results. The source, timing and availability of any future financing will depend principally upon equity and debt market conditions, interest rates and, more specifically, on the extent of our commercial success and the results of our future development efforts. There can be no assurance that external funds will be available on favorable terms, if at all.

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 and collaborations and strategic and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interest of our existing stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our stockholders. Debt financing, if available, would result in increased fixed payment obligations and may involve agreements that include covenants limiting or restricting our ability to take specific actions such as incurring debt, making capital expenditures or declaring dividends. If we raise additional funds through collaboration, strategic alliance and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams or product candidates, or grant licenses on terms that are not favorable to us.

Our limited operating history makes it difficult to evaluate our business and prospects.

We were incorporated in February 2005. Our operations to date have been limited to organizing and staffing our company and conducting product development activities and commercial-build and initial U.S. launch activities, primarily for JUXTAPID. Consequently, any predictions about our future performance may not be as accurate as they could be if we had a longer operating history and more experience in generating revenue. In addition, as a relatively young business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors.

Risks Related to our Common Stock

We have limited history generating product revenue, and may, in the future, need to raise capital to operate our business.

As of December 31, 2012, we had generated no product revenue. The FDA approved our first product, JUXTAPID, on December 21, 2012, and we launched the product in late January 2013. The EMA and other regulatory authorities outside the U.S. have not yet approved JUXTAPID, and may not approve JUXTAPID. Our ability to generate significant product revenue in the foreseeable future, and the amount of any such revenue, depend on a number of factors, including our ability to:

- effectively market, sell and distribute JUXTAPID in the U.S.;
- obtain approval of lomitapide in the EU and other key international markets as a treatment for patients with HoFH, and the timing and scope of such approval and the resulting label;
- successfully launch lomitapide in the EU and certain other key international markets, if approved in such jurisdictions;

- obtain market acceptance by patients and physicians for JUXTAPID as a treatment for HoFH;
- effectively estimate the size of the total addressable market;
- obtain named patient sales of JUXTAPID in countries where such sales can occur as a result of the FDA approval of JUXTAPID; and
- obtain reimbursement and pricing for JUXTAPID sufficient to allow us to sell JUXTAPID on a competitive and profitable basis.

We may in the future require additional financing. If we do not succeed in raising additional funds on acceptable terms, if needed, we may be required to alter or scale back our planned activities. In addition, we could be forced to discontinue product development, forego attractive business opportunities or curtail operations. Any additional sources of financing could involve the issuance of our equity securities, which would have a dilutive effect on our stockholders. No assurance can be given that additional financing will be available to us when needed on acceptable terms, or at all.

The market price of our common stock has been, and may continue to be, highly volatile.

Our stock price is volatile, and from October 22, 2010, the first day of trading of our common stock, to December 31, 2012, the trading prices of our stock have ranged from $9.00 to $26.73 per share. This is in part because there has been a public market for our common stock only since our initial public offering in October 2010, and our stock could be subject to wide fluctuations in price in response to various factors, many of which are beyond our control, including the following:

- the short-term or long-term success or failure of our commercialization of JUXTAPID in the U.S;
- the response of the EMA to our MAA for approval of lomitapide in the treatment of HoFH;
- the short-term or long-term success or failure of our commercialization of JUXTAPID in the EU and other key countries, if ultimately approved in such jurisdictions;
- the initiation and results of our planned further clinical trials of JUXTAPID;
- fluctuations in stock market prices and trading volumes of similar companies;
- general market conditions and overall fluctuations in U.S. equity markets;
- low trading volume;
- international financial market conditions, including the on-going sovereign debt crisis in the EU;
- variations in our quarterly operating results;
- changes in our financial guidance or securities analysts' estimates of our financial performance;
- announcements of new products, services or technologies, commercial relationships, acquisitions or other events by us or our competitors;
- changes in accounting principles;
- issuance by us of new securities, or sales of large blocks of our common stock, including sales by our executive officers, directors and significant stockholders;
- additions or departures of key personnel;
- success or failure of products within our therapeutic area of focus;
- discussion of us or our stock price by the financial press and in online investor communities;
- our relationships with and the conduct of third parties on which we depend; and
- other risks and uncertainties described in these risk factors.

In addition, the stock market in general, and the market for small pharmaceutical and biotechnology companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. In the past, following periods of volatility in the market, securities class-action litigation has often been instituted against companies. Such litigation, if instituted against us, could result in substantial costs and diversion of management's attention and resources, which could materially and adversely affect our business and financial condition.

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

Provisions in our certificate of incorporation and by-laws 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 acquirers to negotiate with our board of directors, they would apply even if an offer rejected by our board of directors was 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, a stockholder's ability to achieve a return on 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 for the foreseeable future.

We currently anticipate that we will retain any future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Therefore, the success of an investment in shares of our common stock will depend upon any future appreciation in the value of such shares. There is no guarantee that shares of our common stock will appreciate in value or even maintain the price at which our stockholders have purchased their shares.

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.

If our existing stockholders sell, or if the market believes our existing stockholders will sell, substantial amounts of our common stock in the public market, the trading price of our common stock could decline significantly.

As of December 31, 2012, there were:

- 4,751,842 shares issuable upon the exercise of stock options outstanding under our 2006 Stock Option and Award Plan, the 2010 Stock Option and Incentive Plan and the 2012 Inducement Stock Option Plan;
- 56,905 shares of restricted common stock subject to vesting;

- 489,270 shares available for future issuance under the 2010 Stock Option and Incentive Plan; and
- 781,400 shares available for issuance under our Inducement Stock Option Award Plan, a plan that is to be used exclusively for the grant of stock options to individuals who were not previously an employee or a non-employee director (or following a bona fide period of non-employment with us), as an inducement material to the individual's entry into employment, other than as an executive officer, with us within the meaning of Rule 5635(c)(4) of the NASDAQ Listing Rules.

Under the 2010 Plan, the shares reserved for issuance under the plan are automatically increased on an annual basis in accordance with a pre-determined formula. As a result, on January 1, 2012 and January 1, 2013, an additional 848,012 and 1,019,590 shares, respectively, were added to the aggregate number of shares reserved for future issuance under the 2010 Plan under the annual automatic share increase provision of the plan.

If additional shares are sold, or if it is perceived that they will be sold, in the public market, the price of our common stock could decline substantially.

We have registered approximately 8,000,000 shares of the common stock described above that are subject to outstanding stock options and reserved for issuance under our equity plans. These shares can be freely sold in the public market upon issuance, subject to vesting restrictions.

Item 1B. Unresolved Staff Comments.

Not applicable.

Item 2. Properties.

Our principal executive offices, which are located in Cambridge, Massachusetts, consist of 15,148 square feet of office space under a lease that expires in December of 2015. We believe that additional office space will be available on commercially reasonable terms as needed.

Item 3. Legal Proceedings.

We are currently not party to any material legal proceedings, although from time to time we may become involved in disputes in connection with the operation of our business.

Item 4. Mine Safety Disclosures

Not applicable.

PART II

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

Our common stock is quoted on the NASDAQ Global Select Market under the symbol "AEGR". The following table sets forth the high and low sales prices for our common stock in each of 2011 and 2012, as quoted on the NASDAQ Global Market. Our stock was transferred to the NASDAQ Global Select Market tier at NASDAQ effective as of January 2, 2013.

	Common Stock Price			
	2012		2011	
	High	Low	High	Low
First Quarter	$17.72	$13.01	$18.00	$11.54
Second Quarter	$17.20	$11.75	$25.92	$14.62
Third Quarter	$16.08	$12.87	$16.15	$11.80
Fourth Quarter	$26.73	$13.50	$17.20	$12.00

As of March 8, 2013, there were 4 holders of record of our common stock. We have never paid any cash dividends on our common stock and we do not anticipate paying such cash dividends in the foreseeable future. We currently anticipate that we will retain all future earnings, if any, for use in the development of our business.

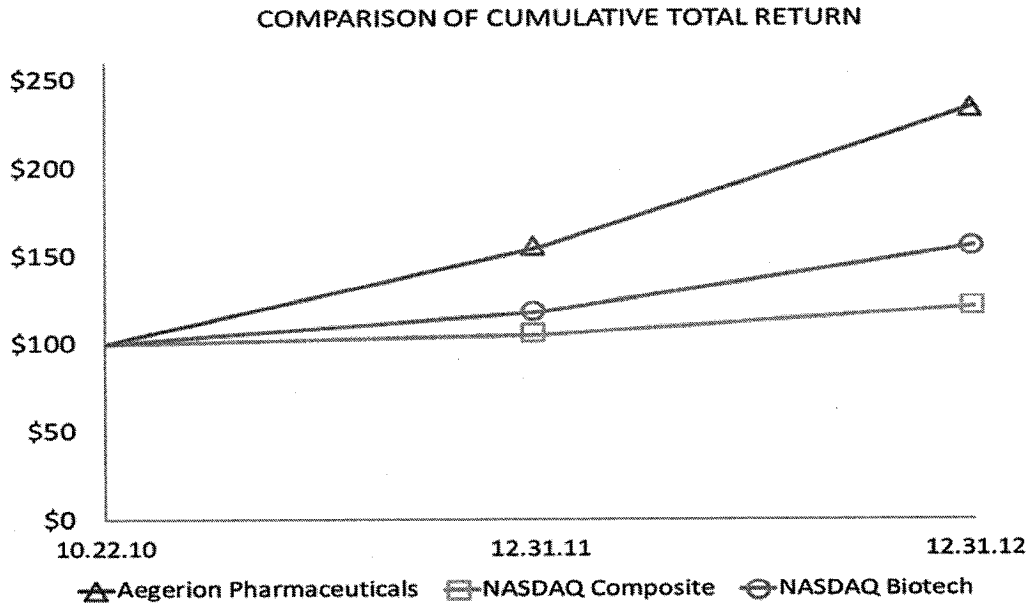
Equity Compensation Plan Information as of December 31, 2012

Information regarding our equity compensation plans is included in Item 12 of Part III of this Annual Report on Form 10-K and incorporated in this Item 5 by reference.

Performance Graph

The following performance graph shows the total shareholder return of an investment of $100 cash on October 22, 2010, the date our common stock first started trading on the NASDAQ Global Market, for (i) our common stock, (ii) the NASDAQ Composite Index (U.S.) and (iii) the NASDAQ Biotechnology Index as of December 31, 2012. Pursuant to applicable SEC rules, all values assume reinvestment of the full amount of all dividends, however no dividends have been declared on our common stock to date. The stockholder return shown on the graph below is not necessarily indicative of future performance, and we do not make or endorse any predictions as to future stockholder returns.

COMPARISON OF CUMULATIVE TOTAL RETURN*
Among Aegerion Pharmaceuticals, Inc., the NASDAQ Composite Index and the NASDAQ Biotechnology Index



* $100 invested on October 22, 2010 in stock or September 30, 2010 in index, including reinvestment of dividends, through fiscal year ending December 31, 2012.

Recent Sales of Unregistered Securities

Not applicable.

Use of Proceeds from Registered Securities

Not applicable.

Item 6. Selected Financial Data.

The following selected financial data are derived from the financial statements of Aegerion Pharmaceuticals, Inc., which have been audited by Ernst & Young LLP, independent registered public accounting firm. The data should be read in conjunction with the financial statements, related notes and other financial information included herein. The Company has reclassified certain prior period amounts to conform to the current period presentation. In 2012, the Company began allocating certain overhead costs across its functional areas and, as a result, reclassified certain amounts in prior periods from selling, general and administrative expenses to research and development expenses.

	Years Ended December 31,				
	2012	2011	2010	2009	2008
	(in thousands, except per share amounts)				
Statement of Operations Data:					
Costs and expenses:					
Research and development	$ 25,164	$ 24,433	$ 7,629	$ 7,041	$ 17,712
Selling, general and administrative	34,077	13,966	5,921	3,075	5,185
Restructuring costs	1,366	912	—	—	—
Total costs and expenses	60,607	39,311	13,550	10,116	22,897
Loss from operations	(60,607)	(39,311)	(13,550)	(10,116)	(22,897)
Interest expense	(937)	(1,114)	(2,404)	(2,083)	(1,127)
Interest income	162	209	109	177	533
Change in fair value of warrant liability	—	—	(416)	(174)	91
Other than temporary impairment on securities	—	—	(30)	—	(1,665)
Other (expense)/income, net	(883)	748	244	—	31
Loss before income taxes	(62,265)	(39,468)	(16,047)	(12,196)	(25,034)
Benefit from income taxes	—	—	1,793	—	—
Net loss	(62,265)	(39,468)	(14,254)	(12,196)	(25,034)
Less: accretion of preferred stock dividends and other deemed dividends	—	—	(8,751)	(3,287)	(6,242)
Net loss attributable to common stockholders	$(62,265)	$(39,468)	$(23,005)	$(15,483)	$(31,276)
Net loss attributable to common stockholders per share—basic and diluted	$ (2.64)	$ (2.03)	$ (5.07)	$ (9.35)	$ (20.92)
Weighted-average shares outstanding—basic and diluted	23,563	19,409	4,537	1,657	1,495

	As of December 31,				
	2012	2011	2010	2009	2008
			(in thousands)		
Balance Sheet Data:					
Cash, cash equivalents and marketable securities	$ 82,177	$ 73,163	$ 45,226	$ 2,074	$ 9,569
Total assets	85,089	75,568	45,747	2,650	10,252
Debt financing and convertible notes	10,611	10,000	—	20,096	16,098
Accumulated deficit	(192,722)	(130,457)	(90,989)	(70,857)	(58,664)
Total stockholders' equity (deficiency)	$ 60,401	$ 57,201	$ 41,078	$(70,127)	$(55,621)

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

You should read the following discussion and analysis of our financial condition and results of operations together with our financial statements and related notes. In addition to historical information, some of the information in this discussion and analysis contains forward-looking statements reflecting our current expectations and involves risk and uncertainties. For example, statements regarding our plans and strategy for our business, our expectations with respect to future financial performance, expense categories and levels, cash needs and liquidity sources are forward-looking statements. Our actual results and the timing of events could differ materially from those discussed in our forward-looking statements as a result of many factors, including those set forth under the "Risk Factors" in Part I, Item 1A of this Form 10-K.

Overview

We are a biopharmaceutical company dedicated to the development and commercialization of novel, life-altering therapies for patients with debilitating, often fatal, rare diseases.

Our first product, JUXTAPID™ (lomitapide) capsules, also referred to as lomitapide ("JUXTAPID"), received marketing approval from the U.S. FDA on December 21, 2012, as an adjunct to a low-fat diet and other lipid-lowering treatments, including LDL apheresis where available, to reduce LDL-C, TC, apo B and non-HDL-C in patients with HoFH. We launched JUXTAPID in the U.S. in late January 2013. In the first quarter of 2012, we submitted a MAA to the EMA requesting approval to market lomitapide as an adjunct to a low-fat diet and other lipid-lowering therapies, with or without apheresis, to reduce LDL-C, TC, apo B and TG in adults with HoFH. In March 2012, the EMA accepted the MAA for review with a review start date of March 21, 2012.

We expect that our near-term efforts will be focused on:

- commercializing JUXTAPID as a treatment for HoFH in the U.S.;
- gaining regulatory approval of lomitapide for adult patients with HoFH in the EU and in other international markets, and launching lomitapide in those countries in which we receive marketing approval;
- supporting and facilitating expanded access to JUXTAPID in countries where named patient sales supply or compassionate use can occur as a result of the FDA approval of JUXTAPID;
- clinical development activities to support a potential marketing authorization application for lomitapide in HoFH in Japan; and
- activities in support of our planned clinical study of lomitapide in pediatric HoFH patients.

We also expect to build our business in the future by acquiring rights to one or more product candidates targeted at life-threatening or substantially debilitating rare diseases that leverage our infrastructure and expertise.

As of December 31, 2012, we had not generated any revenue from the sale of any product. In the near-term, our ability to generate revenues is entirely dependent upon sales of JUXTAPID in the U.S. and in countries where JUXTAPID is available for sale on a named patient sale basis as a result of the approval of JUXTAPID in the U.S. As of December 31, 2012, we had an accumulated deficit of approximately $192.7 million and approximately $82.2 million in cash, cash equivalents and marketable securities. In January 2013, we sold 3,110,449 shares of our common stock in an underwritten public offering at a price to the public of $26.64 per share. The net proceeds to us from this offering were approximately $78.3 million after deducting underwriting discounts and commissions.

Financial Overview

Revenue

As of December 31, 2012, we had not generated any revenue from sales of any product, including JUXTAPID.

Research and Development Expenses

Since our inception, our research and development activities have primarily focused on the clinical development of JUXTAPID and regulatory activities directed at gaining approval of JUXTAPID in HoFH. We recognize both internal and external research and development expenses as they are incurred. Our research and development expenses have consisted primarily of:

- salaries and related expenses for personnel;
- fees paid to contract research organizations ("CROs"), in conjunction with independently monitoring our clinical trials and acquiring and evaluating data in conjunction with our clinical trials, including all related fees, such as for investigator grants, patient screening, laboratory work and statistical compilation and analysis;
- costs related to production of clinical materials, process validation and development efforts to support regulatory approval, including fees paid to contract manufacturers;
- costs related to upfront and milestone payments under in-licensing agreements;
- costs related to compliance with regulatory requirements in the U.S., EU and other foreign jurisdictions;
- consulting fees paid to third parties; and
- costs related to stock-based compensation granted to personnel in development functions.

We expense research and development costs as incurred. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made. Our research and development expenditures are subject to numerous uncertainties in timing and cost to completion. Our planned research and development activities, including those related to completing the process validation of our drug product, the conduct of non-clinical studies directed toward the possible expansion of the JUXTAPID label to include pediatric patients and clinical studies directed towards a potential filing for regulatory approval of JUXTAPID in Japan, may take several years or more to complete. The length of time generally varies according to the type, complexity, novelty and intended use of such a project.

Although we have received marketing approval for JUXTAPID from the FDA, we have not yet received marketing approval from the EMA, or any other foreign regulatory authority. Obtaining marketing approval is an extensive, lengthy, expensive and uncertain process, and the EMA or any other foreign regulatory authority, may delay, limit or deny approval of JUXTAPID for many reasons.

Our expenses related to development activities, including manufacturing and conducting clinical trials, are based on estimates of the services received and efforts expended pursuant to contracts with contract manufacturing organizations and with multiple research institutions and CROs that conduct and manage clinical trials on our behalf. The financial terms of these agreements are subject to negotiation and vary from contract to contract and may result in uneven payment flows. Generally, these agreements set forth the scope of work to be performed at a fixed fee or unit price. Payments under the clinical contracts depend on factors such as the successful enrollment of patients or the completion of clinical trial milestones. 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.

As a result of the uncertainties discussed above, we are unable to determine with certainty the duration and completion costs of our development projects.

Selling, General and Administrative Expenses

Selling, general and administrative expenses consist primarily of compensation for employees in executive and operational functions, including finance, human resources, information technology, sales and marketing and legal. Other significant costs include costs related to stock-based compensation related to options granted to personnel in executive and operational functions, and professional fees for accounting and legal services, including expenses associated with obtaining and maintaining patents.

Interest Income and Interest Expense

Interest income consists of interest earned on our cash, cash equivalents and marketable securities. Interest expense consists primarily of cash and non-cash interest costs related to our outstanding debt. Non-cash interest expense consists of the amortization of capitalized costs incurred in connection with the issuance of debt. We amortize these costs over the life of our debt agreements as interest expense in our statements of operations.

Net Operating Losses and Tax Carryforwards-

As of December 31, 2012, we had federal and state net operating loss carryforwards of approximately $121.5 million and $55.4 million, respectively. We also had federal and state research and development tax credit carryforwards of approximately $22.2 million and $0.8 million available to offset future taxable income. These federal net operating loss and federal tax credit carryforwards will begin to expire at various dates beginning in 2025, if not utilized. The state net operating loss and tax credit carryforwards will expire at various dates starting in 2014, if not utilized. The Tax Reform Act of 1986 provides for a limitation on the annual use of net operating loss and research and development tax credit carryforwards following certain ownership changes that could limit our ability to utilize these carryforwards. We have not completed a study to assess whether an ownership change has occurred, or whether there have been multiple ownership changes since our inception. Accordingly, we expect our ability to utilize the aforementioned carryforwards may be limited. Additionally, U.S. tax laws limit the time during which these carryforwards may be utilized against future taxes. As a result, we may not be able to take full advantage of these carryforwards for federal and state tax purposes.

Critical Accounting Policies and Estimates

Our management's discussion and analysis of our financial condition and results of operations are based on our financial statements, which have been prepared in accordance with U.S. generally accepted accounting principles. The preparation of these financial statements requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, revenue and expenses. On an ongoing basis, we evaluate these estimates and judgments, including those described below. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. These estimates and assumptions form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. Actual results and experiences may differ materially from these estimates.

While our significant accounting policies are more fully described in Note 2 to our financial statements appearing in Item 8 of this Form 10-K, we believe that the following accounting policies are the most critical to aid you in fully understanding and evaluating our reported financial results, and affect the more significant judgments and estimates that we use in the preparation of our financial statements.

Accrued Expenses

As part of the process of preparing financial statements, we are required to estimate accrued expenses. This process involves reviewing open contracts and purchase orders, communicating with our applicable personnel as well as applicable vendor personnel to identify services that have been performed on our behalf and estimating

the level of service performed and the associated cost incurred for the service when we have not yet been invoiced or otherwise notified of actual cost. The majority of our service providers invoice us monthly in arrears for services performed. We make estimates of our accrued expenses as of each balance sheet date in our financial statements based on facts and circumstances known to us at that time. We periodically confirm the accuracy of our estimates with the service providers, and make adjustments if necessary. Examples of estimated accrued expenses include:

- salary and related employee compensation costs;
- fees paid to consultants helping us to launch JUXTAPID as a treatment for HoFH in the U.S.
- fees paid to contract manufacturers in connection with the production of JUXTAPID, including fill/finish capabilities and for the manufacture of our validation runs;
- fees paid to contract research organizations and investigative sites in connection with clinical studies; and
- professional service fees.

Valuation of Financial Instruments

We regularly invest excess operating cash in deposits with major financial institutions; money market funds; notes issued by the U.S. government and U.S. and non U.S. corporations; as well as fixed income investments and U.S. bond funds, both of which can be readily purchased and sold using established markets. The carrying amounts of these marketable securities are generally considered to be representative of their respective fair values based upon pricing of securities with similar investment characteristics and holdings. We believe that the market risk arising from our holdings of these financial instruments is mitigated as many of these securities are government backed or of high credit quality.

Stock-Based Compensation

We measure the fair value of stock options and other stock-based compensation issued to employees and directors on the date of grant. The fair value of equity instruments issued to non-employees are remeasured as the award vests. For service type awards, compensation expense is recognized using the straight line method over the requisite service period, which is typically the vesting period. For awards that vest or begin vesting upon achievement of a performance condition, we begin recognizing compensation expense when achievement of the performance condition is deemed probable and recognize compensation expense using an accelerated attribution method over the implicit service period.

For equity awards that have previously been modified, any incremental increase in the fair value over the original award has been recorded as compensation expense on the date of the modification for vested awards or over the remaining service (vesting) period for unvested awards. The incremental compensation cost is the excess of the fair value based measure of the modified award on the date of modification over the fair value based measure of the original award immediately before the modification. We recorded stock-based compensation expense in our statement of operations as follows:

	Years Ended December 31,		
	2012	2011	2010
		(in thousands)	
Selling, general and administrative	$ 8,335	$4,635	$1,501
Research and development	2,306	1,437	337
Restructuring costs	1,094	580	—
Total	$11,735	$6,652	$1,838

We calculate the estimated fair value of stock-based compensation awards using the Black-Scholes option-pricing model. The Black-Scholes option-pricing model requires the input of subjective assumptions, including stock price volatility and the expected life of stock options. As a recent public company, we do not have sufficient history to estimate the volatility of our common stock price or the expected life of our options. We calculate expected volatility based on reported data for selected reasonably similar publicly traded companies, or guideline peer group, for which the historical information is available. We will continue to use the guideline peer group volatility information until the historical volatility of our common stock is relevant to measure expected volatility for future option grants.

The assumed dividend yield is based on our expectation of not paying dividends in the foreseeable future. We determine the average expected life of stock options according to the "simplified method" as described in Staff Accounting Bulletin 110, which is the mid-point between the vesting date and the end of the contractual term. We determine the risk-free interest rate by reference to implied yields available from five-year and seven-year U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant. We estimate forfeitures based on our historical analysis of actual stock option forfeitures. The weighted-average assumptions used in the Black-Scholes option-pricing model are as follows:

	Years Ended December 31,		
	2012	2011	2010
Risk-free interest rate	1.09%	1.80%	1.14%
Dividend yield	—	—	—
Weighted-average expected life of options (years)	6.13	6.51	6.26
Volatility	85.03%	83.88%	150.00%

There is a high degree of subjectivity involved when using option-pricing models to estimate stock-based compensation. There is currently no market-based mechanism or other practical application to verify the reliability and accuracy of the estimates stemming from these valuation models, nor is there a means to compare and adjust the estimates to actual values. Although the fair value of employee stock-based awards is determined using an option-pricing model, the calculated value may not be indicative of the fair value observed in a market transaction between a willing buyer and willing seller. If factors change and we employ different assumptions when valuing our options, the compensation expense that we record in the future may differ significantly from what we have historically reported.

Results of Operations

Comparison of the Years Ended December 31, 2012 and 2011

The following table summarizes the results of our operations for each of the years ended December 31, 2012 and 2011, together with the changes in those items in dollars and as a percentage:

	Years Ended December 31,			
	2012	2011	Change	%
		(in thousands)		
Costs and expenses:				
Research and development	$ 25,164	$ 24,433	$ 731	3%
Selling, general and administrative	34,077	13,966	20,111	144%
Restructuring costs	1,366	912	454	50%
Total costs and expenses	60,607	39,311	21,296	54%
Loss from operations	(60,607)	(39,311)	(21,296)	54%
Interest expense	(937)	(1,114)	177	(16)%
Interest income	162	209	(47)	(22)%
Other (expense)/income, net	(883)	748	(1,631)	(218)%
Net loss	$(62,265)	$(39,468)	$ 22,797	(58)%

Revenue

We did not recognize any revenue for the years ended December 31, 2012 or 2011, respectively.

Research and Development Expenses

Research and development expenses were $25.2 million for the year ended December 31, 2012, compared to $24.4 million for the year ended December 31, 2011. The $0.8 million increase was primarily attributable to increases of $3.6 million in manufacturing validation expenses, $3.1 million in salary and other employee–related compensation costs, $2.4 million in outside service expenses, and $0.9 million in stock-based compensation expense, offset in part by decreases of $7.8 million in pre-clinical and clinical trial expenses and $1.7 million in consulting expenses. The increase in manufacturing validation expenses was due to completion of our drug substance validation campaign in 2012. The increases in salary and related employee- related compensation costs and stock-based compensation expense were primarily due to an increase in research and development headcount and an annualization of costs for those employees hired in 2011. The increases in outside service expenses were due to resources devoted to regulatory work and related activities in connection with our NDA and other lomitapide regulatory filings. The decrease in both clinical trial costs and consulting expenses is due to the substantial completion of our pivotal clinical trial for JUXTAPID in 2011.

We expect research and development costs will increase in 2013 as compared to 2012 as a result of increases in headcount, primarily in our medical affairs function; our planned clinical development activities to support a marketing authorization application for JUXTAPID in HoFH in Japan; activities in support of our planned clinical study of JUXTAPID in pediatric HoFH patients; future international regulatory filings for JUXTAPID and other possible clinical development activities and post marketing commitments. Due to the numerous risks and uncertainties associated with the timing and costs to conduct clinical trials and related activities, we cannot determine these future expenses with certainty and the actual amounts may vary significantly from our forecasts. Generally, inventory may be capitalized if it is probable that future revenue will be generated from the sale of the inventory and that these revenues will exceed the cost of the inventory. Since the approval of JUXTAPID in the U.S. in December 2012 and subsequent product launch in January 2013 the Company evaluated whether a portion of its costs incurred for manufacturing validation runs may be capitalized as inventory. In 2012, we expensed the costs of our manufacturing validation runs due to the high risk inherent in drug development and uncertainty as to whether lomitapide would be approved and ultimately saleable.

Selling General and Administrative Expenses

Selling, general and administrative expenses were $34.1 million for the year ended December 31, 2012, as compared to $14.0 million for the year ended December 31, 2011. The $20.1 million increase was primarily due to an $11.2 million increase in salary and employee-related compensation costs, a $3.7 million increase in stock-based compensation expense and a $4.4 million increase in outsourced services and consulting expenses, which includes pre-launch sales and marketing expenditures, and legal, accounting and auditing fees. These increases were primarily due to the build-out of the sales, marketing and general and administrative functions and related headcount as well as costs incurred to prepare for the commercial launch of JUXTAPID.

We expect that our selling, general and administrative expenses will increase in 2013 due to the annualization of costs for those sales and marketing employees hired throughout 2012, continued activities related to the commercialization of JUXTAPID in the U.S., and the building of our commercial infrastructure in countries outside the U.S. in anticipation of both named patient sales efforts and of a possible future launch in those countries. These increases will likely include increased costs for additional sales and marketing personnel, including sales representatives and reimbursement case managers, as well as additional costs associated with the establishment of our international distribution channel for JUXTAPID.

Restructuring Costs

In the fourth quarter of 2011, we consolidated facilities and related administrative functions into our Cambridge headquarters. As a result, we closed our Bedminster, New Jersey office, effective December 31, 2011, and reduced headcount by five positions. Restructuring costs were $1.4 million and $0.9 million for the years ended December 31, 2012 and 2011, respectively. Included in the restructuring charges are $0.3 million and $0.2 million of employee severance and outplacement services costs for five employees, primarily in general and administrative positions, for the years ended December 31, 2012 and 2011, respectively.

In addition, we accelerated the vesting of 137,136 total stock options granted in 2010, 2009 and 2008 to the former New Jersey employees upon the termination of their employment. As such, we recognized expense related to those stock options based on the fair value of the stock options at the date of the modification of each award. We expensed the value of the modification over the remaining service periods for each of the employees. We recognized approximately $1.1 million and $0.6 million of stock compensation expense related to these modifications for the years ended December 31, 2012 and 2011, respectively.

In January 2012, we entered into a sublease agreement for the Bedminster, New Jersey facility for the remaining term of the lease. In determining the ongoing facilities charge, we considered our sublease arrangement for the facility, including sublease terms and the sublease rates. We will record ongoing restructuring charges of approximately $35,000 related to the remaining lease obligation through July 2018.

Interest Expense

Interest expense was $0.9 million and $1.1 million for the years ended December 31, 2012 and 2011, respectively. The $0.2 million decrease was primarily due to the reduced interest rate associated with the Silicon Valley Bank ("Silicon Valley Bank") term loan compared to our loan with Hercules Technology, II, L.P. and Hercules III, L.P. (collectively, "the Hercules Funds") that was repaid in the first quarter of 2012.

Interest Income

Interest income was $0.2 million for both of the years ended December 31, 2012 and 2011, respectively.

Other (Expense)/Income

For the year ended December 31, 2012 other expense was $0.9 million primarily attributable to charges incurred in relation to the early repayment of our $10.0 million loan to the Hercules Funds. For the year ended December 31, 2011, other income was $0.7 million, primarily attributable to a gain on sale of our long-term investment in an auction rate security and an auction rate security converted to preferred stock.

Comparison of the Years Ended December 31, 2011 and 2010

	Years Ended December 31,			
	2011	2010	Change	%
	(in thousands)			
Costs and expenses:				
Research and development	$ 24,433	$ 7,629	$ 16,804	220%
Selling, general and administrative	13,966	5,921	8,045	136%
Restructuring costs	912	—	912	100%
Total costs and expenses	39,311	13,550	25,761	190%
Loss from operations	(39,311)	(13,550)	(25,761)	190%
Interest expense	(1,114)	(2,404)	1,290	(54)%
Interest income	209	109	100	92%
Change in fair value of warrant liability	—	(416)	416	(100)%
Other income, net	748	214	534	250%
Loss before income taxes	(39,468)	(16,047)	23,421	(146)%
Benefit from income taxes	—	1,793	(1,793)	(100)%
Net loss	$(39,468)	$(14,254)	25,214	(177)%

Revenue

We did not recognize any revenue for the years ended December 31, 2011 and 2010, respectively.

Research and Development Expenses

Research and development expenses were $24.4 million for the year ended December 31, 2011 compared with $7.6 million for the year ended December 31, 2010. The $16.8 million increase for the year ended December 31, 2011 was primarily due to increased expenses related to our lomitapide development program, including increases of $5.0 million in clinical trial costs, $3.1 million in clinical consultant expenses, $2.8 million in employee salary and related compensation costs, $2.3 million in process development manufacturing and $1.3 million in pre-clinical expenses. In addition, stock-based compensation expense increased $1.1 million. These increases were due to increased headcount, the advancement of the clinical development program for lomitapide as well as the advancement of the regulatory work and related preparations for the filing of our NDA and MAA for lomitapide.

Selling, General and Administrative Expenses

Selling, general and administrative expenses were $14.0 million for the year ended December 31, 2011, compared with $5.9 million for the year ended December 31, 2010. The $8.0 million increase was primarily attributable to increases of $3.1 million in stock-based compensation expense, $2.5 million in professional services expenses, $2.2 million in compensation costs, and $0.8 million for facility-related expenses. These increases are mainly due to building out our management team and related stock-based compensation costs, as well as the costs associated with operating as a public company.

Restructuring Costs

Restructuring costs of $0.9 million in 2011 were related to closure of our Bedminster, New Jersey facility. Included in the restructuring charges are $0.2 million of employee severance and outplacement services costs for five employees, primarily in general and administrative positions, $0.1 million representing the net present value of the remaining lease obligation for our Bedminster, New Jersey facility and a net write off of $0.1 million of fixed assets, primarily computer equipment and leasehold improvements, that were no longer in use after vacating the facility. We also recorded $0.6 million of non-cash restructuring charges related to the modification of stock options for former employees in 2011.

Interest Expense

Interest expense was $1.1 million for the year ended December 31, 2011, compared with $2.4 million for the year ended December 31, 2010. The $1.3 million decrease was primarily due to the write-off of non-cash deferred financing fees associated with the conversion of our preferred stock and convertible notes into shares of common stock at the closing of our initial public offering in 2010.

Interest Income

Interest income was $0.2 million for the year ended December 31, 2011, compared with $0.1 million for the year ended December 31, 2010. The $0.1 million increase was due to higher cash, cash equivalent and marketable securities balances subsequent to our public offerings in late 2010 and 2011.

Change in Fair Value of Warrant Liability

We recorded $0.4 million of other expense during the year ended December 31, 2010 due to the revaluation of our preferred stock warrant to fair value prior to its conversion to a warrant to purchase common stock upon the closing of our IPO.

Other Income

Other income was $0.7 million for the year ended December 31, 2011, primarily attributable to the gain on sales of our long-term investment in an auction rate security and an auction rate security converted to preferred stock. Other income for the year ended December 31, 2010 was $0.2 million representing the receipt of funds from the U.S. Treasury Department in 2010 for the Qualified Therapeutics Discovery Projects Grant Program.

Benefit From Income Taxes

We recorded a $1.8 million income tax benefit during the year ended December 31, 2010, representing proceeds from the sale of New Jersey state net operating losses to a third party during the first half of 2010.

Liquidity and Capital Resources

Since our inception in 2005, we have funded our operations primarily through proceeds from the private placement of convertible preferred stock, convertible debt, venture debt, bank debt and the proceeds from our public issuances of common stock. As of December 31, 2012, we had not generated any revenues. We have incurred losses and generated negative cash flows from operations since inception. As of December 31, 2012, our cash, cash equivalents and marketable securities totaled $82.2 million.

On March 28, 2012, we entered into a Loan and Security Agreement with Silicon Valley Bank (as amended, the "Loan and Security Agreement") pursuant to which Silicon Valley Bank made a term loan to us in the principal amount of $10.0 million. The Loan and Security Agreement provides for interest-only payments through February 28, 2013, with per annum interest of 6.75% and a final payment of $0.2 million. The proceeds of the term loan were used to repay our existing loan from the Hercules Funds. The Loan and Security Agreement provides that we shall repay the principal balance of the term loan in 36 equal monthly installments starting on March 1, 2013 and continuing through February 1, 2016. The remaining term loan principal balance and all accrued but unpaid interest will be due and payable on February 1, 2016. At our option, we may prepay all or any part of the outstanding term loan subject to a prepayment premium (defined in the Loan and Security Agreement).

In July 2012, we entered into an arrangement with Silicon Valley Bank, pursuant to which we received a line of credit of up to $0.8 million to finance, subject to the terms of the Loan and Security Agreement, as amended, the purchase of certain types of equipment acquired by us during the two years ended December 31, 2012. As of December 31, 2012, we financed approximately $0.6 million under this line of credit and the remainder of the line of credit expired unused. Pursuant to the agreement, monthly principal payments begin in January 2013 and continue through December, 2015.

In June 2012, we sold 3,400,000 shares of common stock at a public offering price of $14.75 per share, resulting in proceeds to us of approximately $47.0 million, net of underwriting discounts, commissions and other offering expenses. In July 2012, the underwriters exercised their overallotment option to purchase an additional 393,085 shares at a public offering price of $14.75 per share, resulting in proceeds to us of approximately $5.6 million, net of underwriting discounts, commissions and other offering expenses.

In January 2013, we sold 3,110,449 shares of our common stock in an underwritten public offering at a price to the public of $26.64 per share. The net proceeds to us from this offering were approximately $78.3 million, after deducting underwriting discounts and commissions.

Cash Flows

The following table sets forth the major sources and uses of cash for the years ended December 31, 2012, 2011 and 2010:

	Years Ended December 31,		
	2012	2011	2010
		(in thousands)	
Net cash provided by (used in):			
Operating activities	$(42,557)	$(30,509)	$ (8,499)
Investing activities	(28)	(46,534)	(12)
Financing activities	53,380	59,335	51,183
Effect of exchange rates on cash	8	(25)	—
Net increase(decrease) in cash and cash equivalents	$ 10,803	$(17,733)	$42,672

The primary use of cash used in operating activities for all periods was to fund our net operating losses, primarily related to clinical development of JUXTAPID and our efforts to obtain marketing approval, as well as the build-out of our global sales, marketing, and general and administrative infrastructure.

Cash used in investing activities in 2012 was primarily from purchases of marketable securities of $54.4 million, offset by the maturities of marketable securities of $52.9 million. Cash used in investing activities in 2011 was primarily for purchases of marketable securities of $62.8 million. Cash used in investing activities in 2010 was primarily for purchases of property, plant and equipment.

The cash provided by financing activities for 2012 primarily consisted of $52.6 million of net proceeds from the 2012 offering of common stock, $10.6 million of debt proceeds received from Silicon Valley Bank and $1.1 million of proceeds from the exercise of stock options. The increase was offset by repayment of our term loan of $10.0 million and a debt extinguishment fee of $0.9 million paid to Hercules Funds.

The cash provided by financing activities for 2011 primarily consisted of $49.2 million of net proceeds from the 2011 offering of common stock and $10.0 million of debt proceeds from the Hercules Funds, offset by $0.2 million in deferred financing fees, and $0.4 million of proceeds from the exercise of stock options. The cash provided by financing activities for 2010 consisted mainly of $50.8 million of net proceeds from our IPO and the issuance of $7.5 million of convertible notes, partially offset by $5.5 million of principal repayments under a previous Hercules loan agreement and $1.1 million of deferred financing fees related to the initial public offering.

Contractual Obligations and Commitments

The following table summarizes our contractual obligations and commitments as of December 31, 2012 (in thousands):

	Payments Due By Period				
	Total	2013	2014 and 2015	2016 and 2017	2018 and Thereafter
Long term debt (including interest)	$11,760	$3,643	$7,560	$557	$—
Operating lease obligations	3,118	871	1,830	321	96
Total[1]	$14,878	$4,514	$9,390	$878	$ 96

(1) This table does not include (a) any milestone payments which may become payable to third parties under license agreements as the timing and likelihood of such payments are not known, (b) any royalty payments to third parties as the amounts, timing and likelihood of such payments are not known and (c) contracts that are entered into in the ordinary course of business which are not material in the aggregate in any period presented above.

We currently purchase supplies of drug substance and drug product from our contract manufacturers under the applicable commercial supply agreement. As of December 31, 2012, we had approximately $3.4 million committed for contract manufacturing costs. We expect these amounts will be paid during 2013.

In March 2012, we filed an NDA for JUXTAPID in the U.S. as a treatment for HoFH, and paid UPenn a $50,000 milestone payment under the license agreement. In December 2012, we received marketing approval for JUXTAPID in the U.S. as a treatment for HoFH, and accrued $0.1 million, at December 31, 2012, for payment of the final HoFH-related milestone under the license agreement. Fifty percent of these milestone payments are creditable against future royalties on JUXTAPID sales, as defined in the license agreement, owed to UPenn.

Under our license agreement with UPenn, we will also be required to make development milestone payments of up to an aggregate amount of $2.6 million if we decide to develop lomitapide for indications within the licensed field other than HoFH. All such development milestone payments for these other indications are payable only once, no matter how many licensed products for these other indications are developed. In addition, we are required to make specified royalty payments on net sales of products covered by the license (subject to a variety of customary reductions), and share with UPenn specified percentages of sublicensing royalties and other consideration that we receive under any sublicenses that we may grant. Lomitapide is a licensed product under the license agreement with UPenn.

Future Funding Requirements

In the future, we may need to raise additional capital to fund our operations. Our future capital requirements may be substantial, and will depend on many factors, including:

- the level of physician, patient and payer acceptance of JUXTAPID, and the success of our commercialization efforts;
- the decisions of the EMA with respect to our applications for marketing approval of JUXTAPID for the treatment of adult patients with HoFH in the EU; the costs of activities related to the EU regulatory approval process; the timing of approvals in the EU if received; and the nature and timing of pricing approvals of the various countries in the EU;
- the decisions of various countries outside the U.S. and EU with respect to approval of lomitapide, and reimbursement and pricing decisions in such countries, if approved;

- the timing and cost of the planned juvenile animal toxicology study, and an anticipated clinical trial to evaluate JUXTAPID for treatment of pediatric patients with HoFH;
- the cost of establishing and maintaining the sales and marketing capabilities necessary for commercial launch of JUXTAPID in HoFH in the U.S. and in the EU and certain other key international markets, if approved;
- the timing and cost of our planned clinical development program of JUXTAPID in HoFH in Japanese patients;
- the cost of filing, prosecuting and enforcing patent claims;
- the costs of our manufacturing-related activities;
- the costs associated with commercializing JUXTAPID;
- the levels, timing and collection of revenue received from sales of approved products in the future;
- the cost of our observational cohort study as part of our post-marketing commitments to the FDA;
- the timing and cost of other clinical development activities; and
- the timing and costs of future business development opportunities.

In June 2012, we completed an underwritten public offering of 3,400,000 shares of common stock at a price to the public of $14.75 pursuant to a Form S-3 registration statement. The net proceeds to us from this offering were approximately $47.0 million, after deducting underwriting discounts, commissions and other offering expenses. In July 2012, the underwriters exercised their overallotment option to purchase an additional 393,085 shares of common stock at a price of $14.75 per share. The net proceeds to us from the issuance and sale of the over-allotment shares were approximately $5.6 million, after deducting underwriting discounts, commissions and other offering expenses.

On January 14, 2013, we completed an underwritten public offering of 3,110,449 common shares at a price to the public of $26.64 per share pursuant to a Form S-3 registration statement. The net proceeds to us from this offering were approximately $78.3 million, after deducting underwriting discounts and commissions. We currently intend to use the net proceeds of these offerings to fund activities directed at commercial launch of JUXTAPID in the U.S.; pursuing approval of our MAA submission with the EMA for lomitapide, and, if it is approved, commercial activities in the EU; expansion of operations in certain countries to pursue regulatory approval of lomitapide and to conduct sales on a named-patient-sales basis, where permitted; advancement of the clinical development of JUXTAPID; and business development activities; with any remainder to fund working capital, capital expenditures and for other general corporate purposes.

On February 15, 2013, we filed an automatic shelf registration statement on Form S-3 ASR with the Securities and Exchange Commission ("SEC"), which became immediately effective. This shelf registration statement permits us to offer, from time to time, an unspecified amount of any combination of common stock, preferred stock, debt securities and warrants.

If we are unable to obtain additional financing, we may be required to reduce the scope of our planned activities which could harm our business, financial condition and operating results. The source, timing and availability of any future financing will depend principally upon equity and debt market conditions, interest rates and, more specifically, on the extent of our commercial success and our continued progress in our regulatory and development activities. There can be no assurance that external funds will be available on favorable terms, if at all. As of December 31, 2012, we had not generated any revenue. We expect our continuing operating losses to result in increases in cash used in operations at least through 2013 and potentially in subsequent years. We anticipate our existing cash, cash equivalents and marketable securities will be sufficient to enable us to maintain our currently planned operations, including our continued product development, until we are profitable.

Off-Balance Sheet Arrangements

We do not currently have, nor have we ever had, any relationships with unconsolidated entities or financial partnerships, such as entities often referred to as structured finance or special purpose entities, which would have been established for the purpose of facilitating off-balance sheet arrangements or other contractually narrow or limited purposes. In addition, we do not engage in trading activities involving non-exchange traded contracts. Therefore, we are not materially exposed to any financing, liquidity, market or credit risk that could arise if we had engaged in these relationships.

Item 7A. Quantitative and Qualitative Disclosures About Market Risk.

Our primary exposure to market risk is interest income sensitivity, which is affected by changes in the general level of U.S. interest rates. We do not have any derivative financial instruments.

Item 8. Financial Statements and Supplementary Data.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Shareholders of Aegerion Pharmaceuticals, Inc.

We have audited the accompanying consolidated balance sheets of Aegerion Pharmaceuticals, Inc. as of December 31, 2012 and 2011, and the related consolidated statements of operations, comprehensive loss, stockholders' equity (deficiency), and cash flows for each of the three years in the period ended December 31, 2012. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. An audit includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements. An audit also includes assessing the accounting principles used and significant estimates made by management, as well as evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the consolidated financial position of Aegerion Pharmaceuticals, Inc. at December 31, 2012 and 2011, and the consolidated results of its operations and its cash flows for each of the three years in the period ended December 31, 2012, in conformity with U.S. generally accepted accounting principles.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), Aegerion Pharmaceuticals, Inc.'s internal control over financial reporting as of December 31, 2012, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission and our report dated March 18, 2013 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

Metro Park, New Jersey
March 18, 2013

Aegerion Pharmaceuticals, Inc.

Consolidated Balance Sheets
(in thousands, except per share amounts)

	December 31, 2012	December 31, 2011
Assets		
Current assets:		
Cash and cash equivalents	$ 37,171	$ 26,368
Marketable securities	45,006	46,795
Prepaid expenses and other current assets	1,571	914
Total current assets	83,748	74,077
Restricted cash	105	105
Property and equipment, net	1,143	526
Other assets	93	860
Total assets	$ 85,089	$ 75,568
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable	$ 4,953	$ 2,798
Current portion of long-term debt	3,022	1,875
Accrued liabilities	8,951	4,727
Total current liabilities	16,926	9,400
Long-term debt, net current portion	7,589	8,125
Other liabilities	173	842
Total liabilities	24,688	18,367
Commitments and contingencies		
Stockholders' equity:		
Common stock, $0.001 par value, 125,000 shares authorized at December 31, 2012 and 2011; 25,593 and 21,304 shares issued at December 31, 2012 and 2011, respectively, and; 25,489 and 21,200 shares outstanding at December 31, 2012 and 2011, respectively	25	21
Treasury Stock, at cost; 104 shares at December 31, 2012 and 2011	—	—
Subscription receivable	(129)	—
Additional paid-in-capital	253,210	187,685
Accumulated deficit	(192,722)	(130,457)
Accumulated other comprehensive items	17	(48)
Total stockholders' equity	60,401	57,201
Total liabilities and stockholders' equity	$ 85,089	$ 75,568

See accompanying notes.

Aegerion Pharmaceuticals, Inc.

Consolidated Statements of Operations
(in thousands, except per share amounts)

	Years Ended December 31,		
	2012	2011	2010
Costs and expenses:			
Research and development	$ 25,164	$ 24,433	$ 7,629
Selling, general and administrative	34,077	13,966	5,921
Restructuring costs	1,366	912	—
Total costs and expenses	60,607	39,311	13,550
Loss from operations	(60,607)	(39,311)	(13,550)
Interest expense	(937)	(1,114)	(2,404)
Interest income	162	209	109
Change in fair value of warrant liability	—	—	(416)
Other (expense)/income, net	(883)	748	214
Loss before income taxes	(62,265)	(39,468)	(16,047)
Benefit from income taxes	—	—	1,793
Net loss	(62,265)	(39,468)	(14,254)
Less: accretion of preferred stock dividends and other deemed dividends	—	—	(8,751)
Net loss attributable to common stockholders	$(62,265)	$(39,468)	$(23,005)
Net loss attributable to common stockholders per common share—basic and diluted	$ (2.64)	$ (2.03)	$ (5.07)
Weighted-average shares outstanding—basic and diluted	23,563	19,409	4,537

See accompanying notes.

Aegerion Pharmaceuticals, Inc.

Consolidated Statements of Comprehensive Loss
(in thousands)

	Years Ended December 31,		
	2012	2011	2010
Net loss	$(62,265)	$(39,468)	$(14,254)
Other comprehensive income/(loss), net of tax			
Unrealized gains/(losses) on marketable securities			
Unrealized holding gains on available-for-sale investments	28	129	510
Less: Reclassification adjustments for gains included in net loss	—	(748)	—
Foreign currency translation	37	(20)	—
Other comprehensive income/(loss)	65	(639)	510
Comprehensive loss	$(62,200)	$(40,107)	$(13,744)

See accompanying notes.

Aegerion Pharmaceuticals, Inc.

Consolidated Statements of Stockholders' Equity (Deficiency)
(in thousands)

	Common Stock		Subscription Receivable	Additional Paid-In Capital	Treasury Stock		Accumulated Deficit	Accumulated Other Comprehensive Items	Total Stockholders' Equity/(Deficiency)
	Shares	Capital			Shares	Capital			
Balance at December 31, 2009	**1,812**	**$ 2**	**$ —**	**$ 649**	**104**	**$—**	**$ (70,857)**	**$ 81**	**$(70,125)**
Issuance of common stock	5,750	6	—	48,671	—	—	—	—	48,677
Stock options exercised	38	—	(91)	91	—	—	—	—	—
Conversion of convertible notes into common stock	3,093	3	—	23,508	—	—	—	—	23,511
Conversion of preferred stock into common stock	7,052	7	—	52,912	—	—	—	—	52,919
Reclass of warrant liability	—	—	—	983	—	—	—	—	983
Stock based compensation resulting from stock options granted to employees and board of directors	—	—	—	1,825	—	—	—	—	1,825
Stock based compensation resulting from stock options granted to nonemployees	—	—	—	13	—	—	—	—	13
Beneficial conversion-convertible notes	—	—	—	5,878	—	—	(5,878)	—	—
Accretion of preferred stock dividend	—	—	—	(2,873)	—	—	—	—	(2,873)
Accretion of preferred stock issuance costs	—	—	—	(107)	—	—	—	—	(107)
Net loss	—	—	—	—	—	—	(14,254)	—	(14,254)
Change in unrealized gain/(loss) on securities	—	—	—	—	—	—	—	510	510
Balance at December 31, 2010	17,745	18	(91)	131,550	104	—	(90,989)	591	41,079
Stock based compensation resulting from stock options granted to employees and board of directors	—	—	—	5,844	—	—	—	—	5,844
Stock based compensation resulting from stock options granted to nonemployees	—	—	—	228	—	—	—	—	228
Stock based compensation resulting from restructuring activities	—	—	—	580	—	—	—	—	580
Issuance of common stock	3,400	3	—	49,191	—	—	—	—	49,194
Stock options exercised	159	—	91	292	—	—	—	—	383
Net loss	—	—	—	—	—	—	(39,468)	—	(39,468)
Change in unrealized gain/(loss) on securities	—	—	—	—	—	—	—	(619)	(619)
Foreign currency translation	—	—	—	—	—	—	—	(20)	(20)
Balance at December 31, 2011	21,304	21	—	187,685	104	—	(130,457)	(48)	57,201
Stock based compensation resulting from stock options granted to employees and board of directors	—	—	—	10,246	—	—	—	—	10,246
Stock based compensation resulting from stock options granted to nonemployees	—	—	—	395	—	—	—	—	395
Stock based compensation resulting from restructuring activities	—	—	—	1,094	—	—	—	—	1,094
Issuance of common stock	3,793	4	—	52,515	—	—	—	—	52,519
Stock options exercised	364	—	(129)	1,275	—	—	—	—	1,146
Warrants exercised	66	—	—	—	—	—	—	—	—
Vesting of restricted stock awards	66	—	—	—	—	—	—	—	—
Net loss	—	—	—	—	—	—	(62,265)	—	(62,265)
Change in unrealized gain/(loss) on securities	—	—	—	—	—	—	—	28	28
Foreign currency translation	—	—	—	—	—	—	—	37	37
Balance at December 31, 2012	**25,593**	**$ 25**	**$(129)**	**$253,210**	**104**	**$—**	**$(192,722)**	**$ 17**	**$ 60,401**

See accompanying notes.

Aegerion Pharmaceuticals, Inc.

Consolidated Statements of Cash Flows
(in thousands)

	Year Ended December 31,		
	2012	2011	2010
Operating activities			
Net loss	$(62,265)	$(39,468)	$(14,254)
Adjustments to reconcile net loss to net cash used in operating activities:			
Depreciation	197	45	10
Amortization of premium on marketable securities	1,032	362	—
Stock-based compensation	10,641	6,072	1,838
Noncash interest expense	153	230	1,919
Deferred rent expense	34	107	—
Noncash restructuring costs	1,094	616	—
Mark to market of warrant liability	—	—	416
Impairment loss on investments in securities and other non-cash (gains)/losses	53	(748)	30
Loss on extinguishment of debt	766	—	—
Changes in operating assets and liabilities:			
Restricted cash	—	(105)	—
Prepaid expenses and other current assets	(640)	(414)	(466)
Other long-term assets	—	(67)	—
Accounts payable	2,150	(347)	1,730
Accrued liabilities	4,217	3,208	278
Other liabilities	11	—	—
Net cash used in operating activities	(42,557)	(30,509)	(8,499)
Investing activities			
Purchases of property and equipment	(815)	(631)	(12)
Purchases of marketable securities	(54,424)	(62,812)	—
Maturities of marketable securities	52,946	11,050	—
Sales and redemptions of marketable securities	2,265	5,859	—
Net cash used in investing activities	(28)	(46,534)	(12)
Financing activities			
Debt extinguishment fee	(875)	—	(525)
Proceeds from issuances of convertible notes	—	—	7,500
Proceeds from issuances of notes payable	10,611	10,000	—
Payment of notes payable	(10,000)	—	(5,481)
Proceeds from exercise of stock options	1,146	383	—
Proceeds from issuances of common stock, net of offering expenses	52,573	49,194	50,801
Deferred financing fees	(75)	(242)	(1,112)
Net cash provided by financing activities	53,380	59,335	51,183
Exchange rate effect on cash	8	(25)	—
Net increase (decrease) in cash and cash equivalents	10,803	(17,733)	42,672
Cash and cash equivalents, beginning of period	26,368	44,101	1,429
Cash and cash equivalents, end of period	$ 37,171	$ 26,368	$ 44,101
Supplemental disclosures of cash flow information			
Deferred charge due on maturity of Hercules term loan	$ —	$ 775	$ —
Accretion of preferred stock dividends and issuance costs	$ —	$ —	$ 2,979
Cash paid for interest	$ 815	$ 794	$ 485
Warrant liability reclass to additional paid in capital	$ —	$ —	$ 983
Deemed dividend—convertible notes beneficial conversion option	$ —	$ —	$ 5,878

1. Organization

Aegerion Pharmaceuticals, Inc. (the "Company" or "Aegerion") is a biopharmaceutical company dedicated to the development and commercialization of novel, life-altering therapies for patients with debilitating, often fatal, rare diseases.

The Company's first product, JUXTAPID™ (lomitapide) capsules, also referred to as lomitapide, ("JUXTAPID") received marketing approval from the U.S. Food and Drug Administration ("FDA") on December 21, 2012. The Company launched JUXTAPID in the U.S. in late January 2013. In the first quarter of 2012, the Company submitted a Marketing Authorization Application ("MAA") to the European Medicines Agency ("EMA") requesting approval to market lomitapide. Since inception, the Company had been in its development stage as defined by FASB Accounting Standards ("ASC"), 915, *Development Stage Entities.* With the marketing approval of JUXTAPID and the commencement of principal operations, the Company is no longer considered to be in the development stage. In the near-term, the Company's ability to generate revenues is entirely dependent upon sales of JUXTAPID in the U.S. and in countries where JUXTAPID is available for sale on a named patient basis as a result of the approval of JUXTAPID in the U.S. Based on the Company's current operating plan, the Company believes that its existing cash, cash equivalents and marketable securities provides for sufficient resources to fund its currently planned operations, including the Company's continued product development, for at least the next 12 months.

2. Summary of Significant Accounting Principles

Basis of Presentation and Principles of Consolidation

The accompanying consolidated financial statements have been prepared in accordance with U.S. generally accepted accounting principles ("GAAP") and include all adjustments necessary for the fair presentation of the Company's financial position for the periods presented.

The accompanying consolidated financial statements include the accounts of Aegerion Pharmaceuticals, Inc. and its subsidiaries. All intercompany balances and transactions have been eliminated in consolidation.

The Company has reclassified certain prior period amounts to conform to the current period presentation. In 2012, the Company began allocating certain overhead costs across its functional areas and as a result reclassified certain amounts in the prior year from selling, general and administrative expenses to research and development expenses. The total amount reclassified from selling, general and administrative costs to research and development was approximately $0.7 million in 2011.

Use of Estimates

The preparation of financial statements in conformity with GAAP requires management to make estimates and assumptions that affect the amounts reported in the financial statements and accompanying notes. Actual results could differ from those estimates.

Cash and Cash Equivalents

Cash and cash equivalents consist of highly liquid instruments purchased with an original maturity of three months or less at the date of purchase.

Restricted Cash

During the first quarter of 2011, the Company entered into a lease for new office space and relocated its principal executive offices to Cambridge, Massachusetts. As of December 31, 2012 and 2011, restricted cash $0.1 million represents the collateralized outstanding letter of credit associated with this lease. The funds are invested in a certificate of deposit. The letter of credit permits draws by the landlord to cure defaults under the lease by the Company.

Investments in Securities

At December 31, 2012 and 2011, the Company's investments primarily consisted of U.S. government agency securities, commercial paper, corporate debt and certificates of deposit. These investments are classified as available-for-sale. Interest earned on fixed income investments is included in interest income. The amortized cost of available-for-sale investments at December 31, 2012 is adjusted for amortization of premiums and accretion of discounts to maturity. Such amortization and accretion are included in interest income.

Unrealized gains and losses on these investments are reported within accumulated other comprehensive items as a separate component of stockholders' equity/(deficiency).

If a decline in the fair value of a marketable security below the Company's cost basis is determined to be other than temporary, such marketable security is written down to its estimated fair value as a new cost basis and the amount of the write-down is included in earnings as an impairment charge.

Property and Equipment

Property and equipment are stated at cost, less accumulated depreciation. Depreciation is provided over the estimated useful lives of the respective assets, which range from three to seven years, using the straight-line method.

Deferred Financing Costs

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

Long-Lived Assets

Long-lived assets to be held and used are reviewed for impairment when events or changes in circumstances indicate that the carrying amount of such assets may not be recoverable. Determination of recoverability is based on an estimate of undiscounted future cash flows resulting from the use of the asset and its eventual disposition. In the event that such cash flows are not expected to be sufficient to recover the carrying amount of the assets, the assets are written down to their estimated fair values. Long-lived assets to be disposed are reported at the lower of the carrying amount or fair value less cost to sell.

Research and Development Costs

Research and development expenses are charged to expense as incurred. Research and development expenses comprise costs incurred in performing research and development activities, including personnel-related costs, stock-based compensation, facilities-related overhead, clinical trial costs, manufacturing costs and other contracted services, license fees, and other external costs. Nonrefundable advance payments for goods and services that will be used in future research and development activities are expensed when the activity has been performed or when the goods have been received rather than when the payment is made in accordance with the provisions of ASC 730, *Research and Development* ("ASC 730").

Generally, inventory may be capitalized if it is probable that future revenue will be generated from the sale of the inventory and that these revenues will exceed the cost of the inventory. Since the approval of JUXTAPID and subsequent product launch in 2013, the Company evaluated whether a portion of its costs incurred for manufacturing validation runs may be capitalized as inventory. In 2012, the Company expensed the costs of manufacturing validation runs for lomitapide due to the high risk inherent in drug development and uncertainty as to whether lomitapide would be approved and ultimately saleable.

Concentration of Credit Risk

The Company's financial instruments that are exposed to credit risks consist primarily of cash and cash equivalents and available-for-sale investment securities. The Company maintains its cash and cash equivalents in bank accounts, which, at times, exceed federally insured limits. The Company has not experienced any credit losses in these accounts and does not believe it is exposed to any significant credit risk on these funds.

Income Taxes

The Company uses the liability method to account for income taxes, including the recognition of deferred tax assets and deferred tax liabilities for the anticipated future tax consequences attributable to differences between financial statements amounts and their respective tax bases. The Company reviews its deferred tax assets for recovery. A valuation allowance is established when the Company believes that it is more likely than not that its deferred tax assets will not be realized. Changes in valuation allowances from period to period are included in the Company's tax provision in the period of change.

The Company accounts for uncertain income taxes under the guidance prescribed by ASC 740-10, *Accounting for Uncertainty in Income Taxes*. Under this guidance, the Company may recognize the tax benefit from an uncertain tax position only if it is more likely than not to be sustained upon examination based on the technical merits of the position. The amount of the accrual for which an exposure exists is measured as the largest amount of benefit determined on a cumulative probability basis that the Company believes is more likely than not to be realized upon ultimate settlement of the position. In the event the Company recognizes any interest or penalties, related to uncertain tax positions, the accounting policy of the Company is to recognize the interest accrued and the penalties related to unrecognized tax benefits as a component of income tax expense. The Company did not incur any interest or penalties related to income tax during the years ended December 31, 2012 and 2011. The Company's income tax return reporting periods since December 31, 2009 are open to income tax audit examination by the federal and state tax authorities. In addition, because the Company has net operating loss carry-forwards, the Internal Revenue Service is permitted to audit earlier years and propose adjustments up to the amount of net operating loss generated in those years.

Stock-Based Compensation

The Company accounts for its stock-based compensation to employees in accordance with ASC 718, *Compensation-Stock Compensation* and to non-employees in accordance with ASC 505-50, *Equity Based Payments to Non-Employees.* The Company measures the fair value of stock options and other stock-based compensation issued to employees and directors on the date of grant using the Black Scholes option pricing model. The fair value of equity instruments issued to non-employees are remeasured as the instrument vests For service type awards, compensation expense is recognized using the straight line method over the requisite service period, which is typically the vesting period. For awards that vest or begin vesting upon achievement of a performance condition, the Company begins recognizing compensation expense when the achievement of the performance condition is deemed probable and recognizes compensation expense using an accelerated attribution method over the implicit service period. See Note 12 for further information about the Company's stock option plans.

The Company has from time to time modified the terms of its stock options to employees and directors. The Company accounts for the incremental increase in the fair value over the original award on the date of the

modification for vested awards or over the remaining service (vesting) period for unvested awards. The incremental compensation cost is the excess of the fair value based measure of the modified award on the date of modification over the fair value of the original award immediately before the modification.

The modifications made to the Company's equity awards in 2012 did not result in significant incremental compensation costs, either individually or in the aggregate.

Comprehensive Loss

Comprehensive loss combines net loss and other comprehensive items. Other comprehensive items represent certain amounts that are reported as components of shareholders' equity in the accompanying balance sheet, including currency translation adjustments and unrealized gains and losses on available-for-sale investments.

Accumulated other comprehensive items in the accompanying balance sheet consist of the following:

	December 31,	
	2012	2011
	(in thousands)	
Net unrealized loss on available-for-sale investments	$ —	$(28)
Cumulative translation adjustment	17	(20)
	$ 17	$(48)

Segment Information

The Company currently operates in one business segment focusing on the development and commercialization of its lead product, JUXTAPID. The Company is not organized by market and is managed and operated as one business. A single management team reports to the chief operating decision maker who comprehensively manages the entire business. The Company does not operate any separate lines of business or separate business entities with respect to its products. Accordingly, the Company does not accumulate discrete financial information with respect to separate service lines and does not have separately reportable segments. The Company's long-lived assets in regions other than the United States are immaterial.

3. Property and Equipment

Property and equipment consist of the following:

	December 31,	
	2012	2011
	(in thousands)	
Computer and office equipment	$1,150	$484
Office furniture and equipment	237	139
Leasehold improvements	32	—
	1,419	623
Less accumulated depreciation and amortization	(276)	(97)
Property and equipment, net	$1,143	$526

Depreciation expense was $0.2 million, $45,000 and $10,000 for the years ended December 31, 2012, 2011 and 2010, respectively.

4. Investments in Securities

The following is a summary of investments held by the Company as of December 31, 2012:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value at December 31, 2012
		(in thousands)		
Corporate debt securities	$30,515	$ 2	$ (8)	$30,509
U.S. government agency securities	8,498	7	—	8,505
Commercial paper	5,992	—	—	5,992
Total	$45,005	$ 9	$ (8)	$45,006

The following is a summary of investments held by the Company as of December 31, 2011:

	Amortized Cost	Gross Unrealized Gains	Gross Unrealized Losses	Fair Value at December 31, 2011
		(in thousands)		
Corporate debt securities	$21,403	$ 1	$ (27)	$21,377
U.S. government agency securities	13,825	2	(2)	13,825
Commercial paper	8,595	—	—	8,595
Certificates of deposit	3,000	—	(2)	2,998
Total	$46,823	$ 3	$ (31)	$46,795

5. Fair Value of Financial Instruments

The Company's financial instruments consist of cash and cash equivalents, marketable securities, accounts payable, accrued liabilities and debt payable. The carrying amount of cash equivalents, marketable securities, accounts payable, and accrued liabilities are generally considered to be representative of their respective fair values because of the short-term nature of those instruments at December 31, 2012 and 2011. The stated interest rate on the Company's debt payable is based on market rates for similar types of debt instruments. Accordingly, the carrying value of the Company's long term debt based on Level 2 inputs approximates fair value at December 31, 2012 and 2011.

Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants at the measurement date. ASC 820, *Fair Value Measurements and Disclosures*, establishes a fair value hierarchy for those instruments measured at fair value that distinguishes between fair value measurements based on market data (observable inputs) and those based on the Company's own assumptions (unobservable inputs). This hierarchy maximizes the use of observable inputs and minimizes the use of unobservable inputs. The three levels of inputs used to measure fair value are as follows:

- *Level 1*—Quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date. The Company's Level 1 assets consist of cash and money market investments.
- *Level 2*—Inputs other than quoted prices in active markets that are observable for the asset or liability, either directly or indirectly. The Company's Level 2 assets consist of corporate debt securities, U.S. government agency securities, commercial paper and certificates of deposit.
- *Level 3*—Inputs that are unobservable for the asset or liability.

As of December 31, 2012 and 2011, the Company held par value $44.3 million and $46.3 million of investments in marketable securities, respectively. The Company's cash equivalents are classified within Levels 1 and 2 of the fair value hierarchy. The Company's investments in marketable securities are classified within Level 2 of the fair value hierarchy. The fair value measurements of the Company's financial instruments at December 31, 2012 and 2011 are summarized in the tables below:

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2012
		(in thousands)		
Assets:				
Cash and cash equivalents	$31,793	$ 5,078	$—	$36,871
Corporate debt securities	—	30,509	—	30,509
U.S. government agency securities	—	8,505	—	8,505
Commercial paper	—	5,992	—	5,992
Certificates of deposit	—	300	—	300
Total assets at fair value	$31,793	$50,384	$—	$82,177

	Quoted Prices in Active Markets for Identical Assets (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)	Balance at December 31, 2011
		(in thousands)		
Assets:				
Cash and cash equivalents	$25,118	$ 1,250	$—	$26,368
Corporate debt securities	—	21,378	—	21,378
U.S. government agency securities	—	13,824	—	13,824
Commercial paper	—	8,595	—	8,595
Certificates of deposit	—	2,998	—	2,998
Total assets at fair value	$25,118	$48,045	$—	$73,163

As of December 31, 2012, the contractual maturity of all of the Company's marketable securities occur in one year or less.

In 2007, the Company invested a portion of its cash in investments consisting primarily of investment-grade, asset-backed, variable-rate debt obligations and auction rate securities ("ARS") as outlined in the Company's then current investment policy, which were classified as available-for-sale securities and were reported in the balance sheet at fair value. In December 2008, one of the Company's ARS was subject to a put option, which converted the security into non-cumulative redeemable perpetual preferred stock. In March 2010, the Company was notified that the issuer of the non-cumulative redeemable perpetual preferred stock would not pay dividends as a result of having reported an earned surplus deficit. The Company determined the decline in fair value of its non-cumulative redeemable perpetual preferred stock for the year ended December 31, 2010 to be other than temporary and recorded an impairment charge of $30,000. The Company sold the ARS during 2011 for total proceeds of $1.3 million. In conjunction with the sale, the Company recorded a gain of $0.8 million, which is recorded in other (expense)/ income, net in the accompanying consolidated statement of operations.

The changes in fair value of the Company's Level 3 financial instruments during the year ended December 31, 2011 were as follows:

	Auction rate securities
	(in thousands)
Balance at December 31, 2010	$ 945
Reversal of unrealized gain recorded in other comprehensive loss	(531)
Realized gain included in net loss	681
Proceeds from sale of investments	(1,095)
Balance at December 31, 2011	$ —

The estimated fair value of the ARS was derived through discounted cash flows, a Level 3 input. The Company's discounted cash flow analysis considered, among other things, the quality of the underlying collateral, the credit rating of the issuer, an estimate of when these securities were either expected to have a successful auction or otherwise return to par value, the expected interest income to be received over this period, and the estimated required rate of return for investors that may be willing to purchase such a security. The Company also considered third-party valuations, to the extent available, and similar securities priced in the marketplace when arriving at the estimated fair value.

At December 31, 2010, the Company's auction rate securities were reflected at 63% of par value. The estimated fair value of the Company's ARS that was converted to preferred stock was estimated by analyzing similar securities in the marketplace, a Level 2 input. The Company also considered the fair value of the underlying collateral and credit rating of the issuer. Based on observed settlements of similar securities, the preferred stock was recorded at 12% of par value at December 31, 2010. The Company sold its investment in the ARS during 2011.

Warrant Liability/Equity Instrument

Warrants

In 2009, Hercules Technology Growth Capital, Inc., or Hercules, held a warrant to purchase an aggregate of 387,238 shares of series A redeemable convertible preferred stock at an exercise price of $1.86 per share. In accordance with its terms, the instrument became an equity warrant to purchase 107,779 shares of common stock at an exercise price of $6.68 per share upon the closing of the Company's initial public offering ("IPO"). The estimated fair value of the Company's warrant was determined using a Black-Scholes option pricing model, a Level 3 input. The significant assumptions used in estimating the fair value of the Company's warrant liability as of October 27, 2010 included the exercise price of $6.68 per share, estimate for volatility of 150%, risk free interest rate of 2.3%, fair value of the common stock of $9.50 per share and the estimated life of the warrant of 6.4 years. The Company accounted for its warrant in accordance with ASC 480-10, *Distinguishing Liabilities from Equity,* which requires that a financial instrument, other than an outstanding share, that, at inception, is indexed to an obligation to repurchase the issuer's equity shares, regardless of the timing or likelihood of the redemption shall be classified as a liability. Any modification to the warrant liability was recorded in earnings during the period of the modification. On October 27, 2010, the date of the IPO, all of the shares of preferred stock were converted to shares of common stock. The Company revalued the warrant based upon the fair value of the common stock upon the closing of the IPO, and subsequently reclassified the warrant from liability to equity. The warrant was exercised in its entirety in the first quarter of 2012, resulting in the Company issuing 65,944 net shares of its common stock to Hercules.

6. Accrued Liabilities

Accrued liabilities consist of the following:

	December 31,	
	2012	2011
	(in thousands)	
Accrued employee compensation and related costs	$4,105	$1,180
Accrued research and development costs	2,239	2,317
Accrued professional fees	846	352
Accrued sales and marketing costs	694	—
Other accrued liabilities	1,067	878
Total	$8,951	$4,727

7. Commitments

Leases

The Company leased certain office facilities and office equipment under operating leases during the year ended December 31, 2012. The future minimum payments net of non-cancelable sublease payments for all non-cancelable operating leases as of December 31, 2012 are as follows, (in thousands):

	Lease Commitments	Sublease Income	Obligations Net of Sublease Payments
Year Ending December 31:			
2013	$ 871	(125)	746
2014	912	(128)	784
2015	918	(132)	786
2016	159	(135)	24
2017	162	(138)	24
Thereafter	96	(83)	13
Total	$3,118	$(741)	$2,377

Rent expense under operating leases was approximately $0.6 million, $0.6 million and $0.2 million for the years ended December 31, 2012, 2011 and 2010, respectively.

On November 24, 2010, the Company entered into a lease for office space in Bedminster, New Jersey. The lease provides for an initial base rent of $12,000 per month plus certain operating expenses and taxes beginning on April 1, 2011, and shall increase on an annual basis beginning in April 2012. As discussed in Note 16, the Company has closed this facility and, in January 2012, entered into an agreement to sublease this facility.

Effective January 1, 2011, the Company entered into a five year lease for office space for its headquarters in Cambridge, Massachusetts, and amended this lease in November 2011. The amended lease provides for an initial base rent of $47,000 per month, plus certain operating expenses and taxes, and the base rent shall increase on an annual basis beginning in January 2013. On September 24, 2012, the Company entered into a second amendment to lease additional square footage which provides for an increase in base rent beginning February 1, 2013.

Other Commitments

University of Pennsylvania Licensing Agreements

In May 2006, the Company entered into a license agreement with The Trustees of the University of Pennsylvania ("UPenn") pursuant to which it obtained an exclusive, worldwide license from UPenn to certain know-how and a range of patent rights applicable to lomitapide. In particular, the Company obtained a license to certain patent and patent applications owned by UPenn relating to the dosing of micrososmal triglyceride transfer protein inhibitors, including lomitapide, and certain patents and patent applications and know-how covering the composition of matter of lomitapide that were assigned to UPenn by Bristol-Myers Squibb Company ("BMS") in the field of monotherapy or in combination with other dyslipidemic therapies, which are therapies for the treatment of patients, with abnormally high or low levels of plasma cholesterol or triglycerides.

To the extent that rights under the BMS-UPenn assigned patents were not licensed to the Company under its license agreement with UPenn or were retained by UPenn for non-commercial educational and research purposes, those rights, other than with respect to lomitapide, were licensed by UPenn back to BMS on an exclusive basis pursuant to a technology donation agreement between UPenn and BMS. In the technology donation agreement, BMS agreed not to develop or commercialize any compound, including lomitapide, covered by the composition of matter patents included in the BMS-UPenn assigned patents in the field licensed to the Company exclusively by UPenn. Through the Company's license with UPenn, as provided in the technology donation agreement, it has the exclusive right with respect to the BMS-UPenn assigned patents regarding their enforcement and prosecution in the field licensed exclusively to the Company by UPenn.

The license from UPenn covers, among other things, the development and commercialization of lomitapide alone or in combination with other active ingredients in the licensed field. The license is subject to customary noncommercial rights retained by UPenn for non-commercial educational and research purposes. The Company may grant sublicenses under the license, subject to certain limitations.

The Company is obligated under this license agreement to use commercially reasonable efforts to develop, commercialize, market and sell at least one product covered by the licensed patent rights, such as lomitapide. Pursuant to this license agreement, the Company paid UPenn a one-time license initiation fee of $56,000, which was included in research and development expense in 2005. The Company will be required to make development milestone payments to UPenn of up to $0.2 million when a licensed product's indication is limited to homozygous familial hypercholesterolemia ("HoFH") or severe refractory hypercholesterolemia, and an aggregate of $2.6 million for all other indications within the licensed field. All such development milestone payments for these other indications are payable only once, no matter how many licensed products for these other indications are developed. In addition, the Company will be required to make specified royalty payments on net sales of products covered by the license (subject to a variety of customary reductions) and share with UPenn specified percentages of sublicensing royalties and other consideration that the Company receives under any sublicenses that it may grant.

This license agreement will remain in effect on a country-by-country basis until the expiration of the last-to-expire licensed patent right in the applicable country. The Company has the right to terminate this license agreement for UPenn's uncured material breach of the license agreement or for convenience upon 60 days' prior written notice to UPenn, subject to certain specific conditions and consequences. UPenn may terminate this license agreement for the Company's uncured material breach of the license agreement, its uncured failure to make payments to UPenn or if the Company is the subject of specified bankruptcy or liquidation events.

In March 2012, the Company filed a new drug application ("NDA") for lomitapide as a treatment for HoFH, and paid UPenn a $50,000 milestone payment under the license agreement. In December 2012, the Company received marketing approval for lomitapide in the U.S., as a treatment for HoFH, and the Company accrued the remaining related milestone amount, $0.1 million, as of December 31, 2012. Fifty percent of these milestone payments will offset future royalties on the sale of JUXTAPID.

Other commitments

The Company entered into manufacturing services agreements with two contract manufacturers to purchase supplies of drug substance and drug product under the applicable commercial supply agreement. As of December 31, 2012, the Company had approximately $3.4 million committed for contract manufacturing costs. The Company expects these amounts will be paid during 2013. The Company has no material commitments for capital expenditures.

8. Debt Financing

On March 28, 2012, the Company entered into a Loan and Security Agreement (as amended the "Loan and Security Agreement") with Silicon Valley Bank, pursuant to which Silicon Valley Bank made a term loan to the Company in the principal amount of $10.0 million. The Loan and Security Agreement provides for interest-only payments through February 28, 2013, with per annum interest of 6.75% and a final payment of $0.2 million. The proceeds of the term loan were used by the Company to repay the Company's then outstanding $10.0 million loan from Hercules Technology II, L.P. and Hercules Technology III, L.P. (collectively, "the Hercules Funds").

The Loan and Security Agreement provides that the Company shall repay the principal balance of the term loan in 36 equal monthly installments starting on March 1, 2013 and continuing through February 1, 2016. The remaining term loan principal balance and all accrued but unpaid interest will be due and payable on February 1, 2016. At its option, the Company may prepay all or any part of the outstanding term loan subject to a prepayment premium.

In connection with the Loan and Security Agreement, the Company granted Silicon Valley Bank a security interest in all of the Company's personal property now owned or hereafter acquired, excluding intellectual property, and a negative pledge on intellectual property. The Loan and Security Agreement also provides for standard indemnification of Silicon Valley Bank and contains representations, warranties and certain covenants of the Company, including the agreement by the Company to maintain a specified level of liquidity, below which the Company would be required to pledge up to $5.0 million to Silicon Valley Bank.

As a result of these transactions, in the first quarter of 2012, the Company recorded a loss on extinguishment of debt of $0.8 million, primarily representing the write off of related deferred financing costs, the acceleration of recognition of debt extinguishment fees and the prepayment premium payable to the Hercules Funds.

In July 2012, the Company amended the Loan and Security Agreement with Silicon Valley Bank, pursuant to which the Company received a line of credit of up to $0.8 million to finance the purchase of certain types of equipment acquired by the Company during the two years ended December 31, 2012 with per annum interest of 4.75%.

As of December 31, 2012, the Company has financed approximately $0.6 million under this arrangement, and the remaining line of credit balance expired unused. Pursuant to the agreement, monthly principal payments start in January 2013 and continue through December 2015.

Future minimum payments under the Company's debt agreements as of December 31, 2012, are as follows (in thousands):

Years Ending December 31,	
2013	$ 3,643
2014	3,960
2015	3,600
2016	557
	11,760
Less amounts representing interest	(1,149)
Less current portion	(3,022)
Long term debt, net current portion	$ 7,589

9. Convertible Notes and Beneficial Conversion Option

During 2010, 2009, and 2008, the Company entered into several loan agreements with various financial institutions and individuals ("Purchasers"), whereby the Purchasers agreed to purchase, an aggregate principal amount of $21.3 million of senior subordinated convertible notes ("Notes"), subordinate only to certain loans made to the Company by Hercules as specified in the subordination agreement among the Purchasers, the Company and Hercules Funds. The interest on these Notes was 8.0% per annum.

In connection with the Notes, the Company provided to the purchasers a beneficial conversion option, which provided that immediately upon the closing of sales of the Company's capital stock, in one transaction or series of related transactions, which sale or sales, including without limitation any initial public offering, that results in gross proceeds of at least $10.0 million, the Notes will automatically convert into such shares of newly issued capital stock. The purchasers were entitled to receive a number of shares determined by dividing the loan balance as of the conversion date by an amount equal to 85% of the price per share of the newly issued capital stock. In October 2010, the Company modified the conversion percentage to 80% of the share price. Upon the closing of the IPO, the outstanding principal and interest under these Notes were converted into 3,093,472 shares of common stock at a conversion price of $7.60, or 80% of the offering price. As a result, a beneficial conversion charge of approximately $5.9 million was recognized in additional paid-in-capital.

10. Net Loss Attributable to Common Stockholders Per Share

Basic net loss per common share is computed by dividing the net loss by the weighted-average number of common shares outstanding for the period, less weighted-average unvested common shares subject to repurchase. Diluted net loss per common share is computed by dividing the net loss applicable to common stockholders by the weighted-average number of unrestricted shares of common stock and dilutive common stock equivalents outstanding for the period, determined using the treasury-stock method and the as if-converted method.

The Company previously determined that its series A and B redeemable convertible preferred stock represented participating securities since both securities participated equally with common stock in dividends and unallocated income. The series A and B redeemable convertible preferred stock were converted to common stock on October 27, 2010 upon the closing of the IPO.

Net loss attributable to common stockholders for each period must be allocated to common stock and participating securities to the extent that the securities are required to share in the losses. The Company's series A and B redeemable convertible preferred stock do not have a contractual obligation to share in losses of the Company. As a result, basic net loss per common share is calculated by dividing net loss by the weighted-average shares of common stock outstanding during the period.

The following table presents the computation of basic and diluted net loss per share of common stock:

	Years Ended December 31,		
	2012	2011	2010
	(in thousands, except per share amounts)		
Numerator:			
Net loss	$(62,265)	$(39,468)	$(14,254)
Accretion of preferred stock and other deemed dividends	—	—	(8,751)
Net loss attributable to common stockholders	$(62,265)	$(39,468)	$(23,005)
Denominator:			
Weighted-average common shares outstanding—basic and diluted	23,563	19,409	4,537
Basic and diluted net loss per common share	$ (2.64)	$ (2.03)	$ (5.07)

The following table shows historical dilutive common share equivalents outstanding, which are not included in the above calculation, as the effect of their inclusion is anti-dilutive during each period.

	Years Ended December 31,		
	2012	2011	2010
	(in thousands)		
Unvested restricted stock	57	30	—
Options	4,752	2,965	1,716
Warrants	—	108	108
Total	4,809	3,103	1,824

11. Capital Structure

Preferred Stock

At December 31, 2012, the Company was authorized to issue 5,000,000 shares of $0.001 par value preferred stock. There were no shares issued and outstanding. Dividends on the preferred stock will be paid when, and if, declared by the Board of Directors.

Common Stock

At December 31, 2012, the Company was authorized to issue 125,000,000 shares of $0.001 par value common stock. Dividends on the common stock will be paid when, and if, declared by the Board of Directors. Each holder of common stock is entitled to vote on all matters and is entitled to one vote for each share held.

The Company will, at all times, reserve and keep available, out of its authorized but unissued shares of common stock, sufficient shares to affect the conversion of shares for stock options.

Public Offerings

In June 2012, the Company sold 3,400,000 shares of common stock at a public offering price of $14.75 per share, resulting in proceeds to the Company of approximately $47.0 million, net of underwriting discounts, commissions and other offering expenses.

In July 2012, the underwriters exercised their overallotment option to purchase an additional 393,085 shares at a public offering price of $14.75 per share, resulting in proceeds to the Company of approximately $5.6 million, net of underwriting discounts, commissions and other offering expenses.

In January 2013, the Company sold 3,110,449 shares of common stock at a public offering price of $26.64 per share, resulting in proceeds to the Company of approximately $78.3 million, net of underwriting discounts and commissions.

12. Stock Option Plans

The Company's 2010 Stock Option and Incentive Plan (the "2010 Option Plan") was approved by stockholders in October 2010. The 2010 Option Plan allows the Company to make grants of incentive stock options, non-qualified stock options, stock appreciation rights, restricted stock awards, restricted stock units, unrestricted stock awards, cash-based awards, performance share awards and dividend equivalent rights. All full-time and part-time officers, employees, non-employee directors and other key persons, including consultants and prospective employees, are eligible to participate in the 2010 Option Plan.

There are certain limits on the number of awards that may be granted under the 2010 Option Plan. For example, no more than 1,500,000 shares of common stock may be granted in the form of stock options or stock appreciation rights to any one individual during any one-calendar-year period. Annually on January 1, the maximum number of shares available for issuance under the 2010 Option Plan increases by 4% of the number of shares of Common Stock issued and outstanding on the immediately preceding December 31. At December 31, 2012, the Company has 489,270 shares of common stock available for issuance under its 2010 Option Plan.

In October 2012, the Board of Directors approved the reservation of the 1,000,000 shares of common stock to be used exclusively for the grant of non-qualified stock options to individuals who were not previously an employee or non-employee of the Company as an inducement new hire stock option award. These options will be granted under the Company's 2012 Inducement Stock Option Plan ("the 2012 Option Plan"). At December 31, 2012, the Company has 781,400 shares of common stock available for issuance under the 2012 Option Plan.

The 2010 Option Plan and 2012 Option Plan are administered by the Compensation Committee ("the administrator"). Subject to the terms of the plans and applicable laws and regulations, the administrator has full power and authority to select the participants to whom awards will be granted, to make any combination of awards to participants, to delegate authority to make awards, to accelerate the exercisability or vesting of any award and to determine the specific terms and conditions of each award.

The exercise price of stock options awarded under the 2010 and 2012 Option Plans may not be less than the fair value of the Company's common stock on the date of the option grant, and the term of each option may not exceed ten years from the date of grant. The administrator will determine at what time or times each option may be exercised and, subject to the provisions of the 2010 Option Plan and 2012 Option Plan, the period of time, if any, after retirement, death, disability or other termination of employment during which options may be exercised.

The Company's 2006 Stock Option and Grant Plan (the "2006 Plan") was approved by stockholders in June 2006. As of the October 2010 adoption of the 2010 Option Plan, the Company no longer granted options from this plan.

In the event of a merger, sale or dissolution, or a similar sale event, unless assumed or substituted, all stock options and restricted stock awards granted under all of the Company's stock option plans that are not exercisable immediately prior to the effective time of a sale event will automatically become fully exercisable, and non-forfeitable and awards with conditions and restrictions relating to the attainment of performance goals may become vested and non-forfeitable in connection with a sale event in the administrator's discretion. In addition, upon the effective time of any such sale event, the Company's stock option plans and all awards will terminate unless the parties to the transaction, in their discretion, provide for appropriate substitutions or assumptions of outstanding awards.

The Company recorded stock-based compensation expense in the Company's statements of operations as follows:

	Years Ended December 31,		
	2012	2011	2010
		(in thousands)	
Selling, general and administrative	$ 8,335	$4,635	$1,501
Research and development	2,306	1,437	337
Restructuring costs	1,094	580	—
Total	$11,735	$6,652	$1,838

No related tax benefits of the stock-based compensation costs have been recognized since the Company's inception. The Company recorded $0.8 million of stock-based compensation expense related to the achievement of certain performance conditions in the fourth quarter of 2012.

The Company uses the Black-Scholes option-pricing model to determine the estimated fair value for stock-based awards. The fair value of stock option awards is amortized on a straight-line basis over the requisite service period of the awards, which is generally the vesting period. Expected volatility was calculated based on reported data for selected reasonably similar publicly traded companies, or guideline peer group, for which historical information was available. The Company will continue to use the guideline peer group volatility information until the historical volatility of the Company's common stock is relevant to measure expected volatility for future option grants. The assumed dividend yield was based on the Company's expectation of not paying dividends in the foreseeable future. The average expected life was determined according to the "simplified method" as described in Staff Accounting Bulletin ("SAB") 110, which is the mid-point between the vesting date and the end of the contractual term. The risk-free interest rate is determined by reference to implied yields available from U.S. Treasury securities with a remaining term equal to the expected life assumed at the date of grant. Forfeitures are estimated based on the Company's historical analysis of actual stock option forfeitures. The weighted-average grant-date fair values of stock options granted during the years ended December 31, 2012, 2011 and 2010 were $11.67, $9.74 and $1.71 per share, respectively. The weighted-average assumptions used in the Black-Scholes option-pricing model are as follows:

	Years Ended December 31,		
	2012	2011	2010
Risk-free interest rate	1.09%	1.80%	1.14%
Dividend yield	—	—	—
Weighted-average expected life of options (years)	6.13	6.51	6.26
Volatility	85.03%	83.88%	150.00%

The following table summarizes stock option activity for the Company:

	Number of Outstanding Stock Options	Exercise Price Per Share	Weighted-Average Exercise Price Per Share
	(in thousands, except per share amounts)		
Balance at December 31, 2011	2,965	$ 1.54 – $19.25	$ 9.45
Granted	2,478	$13.39 – $22.35	
Exercised	(364)	$ 1.54 – $14.84	
Forfeited/cancelled	(327)	$ 1.54 – $17.64	
Balance at December 31, 2012	4,752	$ 1.54 – $22.35	$13.01

The Company had 3,580,461 and 2,172,262 shares of outstanding unvested stock options at a weighted average exercise price of $14.28 and $10.93 per share as of December 31, 2012 and 2011, respectively. The Company had 56,905 and 29,890 shares of unvested restricted common stock granted to nonemployees at December 31, 2012 and 2011, respectively. Total unrecognized stock-based compensation cost related to unvested stock options and restricted common stock as of December 31, 2012 was $32.2 million. This unrecognized cost is expected to be recognized over a weighted-average period of approximately 2.9 years. In addition, the Company has $8.0 million of unrecognized compensation expense related to unvested stock options that contain performance criteria.

The aggregate intrinsic value as of December 31, 2012 for options exercisable was $19.1 million. The total intrinsic value of all options outstanding as of December 31, 2012 was $58.8 million. The intrinsic value of the options exercised in 2012, 2011, and 2010 was $4.1 million, $2.0 million, and $0.5 million respectively. The total fair value of options vested during the years ended December 31, 2012, 2011 and 2010 was $9.5 million, $7.9 million and $1.8 million, respectively.

As of December 31, 2012, the number of options outstanding that are expected to vest over the requisite service period or the achievement of the performance condition is deemed probable, net of estimated future option forfeitures was 4,186,842 with a weighted-average exercise price of $12.64 per share, an aggregate intrinsic value of $53.3 million and a weighted-average remaining contractual life of 8.7 years.

The following table summarizes information about stock options outstanding at December 31, 2012:

	Outstanding			Exercisable		
	Number of Options	Weighted Average Exercise Price Per Share	Weighted Average Remaining Contractual Life (years)	Number of Options	Weighted Average Exercise Price Per Share	Weighted Average Remaining Contractual Life (years)
	(in thousands, except per share amounts)					
Exercise Price						
$1.54-9.50	950	$ 1.78	7.7	541	$ 1.77	7.6
$13.01-13.39	1,037	13.18	9.1	186	13.13	9.0
$14.41-15.26	915	14.83	9.0	183	14.89	8.6
$15.95-17.30	945	16.31	9.1	87	16.75	9.1
$17.64-22.35	905	19.30	8.7	174	17.77	8.4
	4,752	$13.01	8.7	1,171	$ 9.11	8.2

Stock Option Grants to Nonemployees

The Company granted 30,000 and 25,000 shares of nonqualified common stock options to non-employee consultants at December 31, 2012 and 2011, respectively. The Company valued these options using the Black-Scholes option-pricing model and recognizes expense related to these awards using the graded-vesting method. The unvested shares held by consultants have been and will be revalued using the Company's estimate of fair value at each reporting period through the remaining vesting period. The reassessment may result in additional charges to expense in the future. The Company recorded compensation expense related to non-employees of approximately $0.4 million, $0.2 million, and $13,000 for the years ended December 31, 2012, 2011 and 2010, respectively.

13. Employee Benefit Plan

The Company maintains a defined contribution 401(k) plan (the "Plan") available to employees. Employee contributions are voluntary and are determined on an individual basis, limited by the maximum amounts allowable under federal tax regulations. As of December 31, 2012, the Company had elected to match up to 3% of the employees' contributions to the Plan. The Company recorded employer contribution expense of approximately $0.2 million, $0.1 million and $46,000 during the years ended December 31, 2012, 2011 and 2010, respectively.

14. Income Taxes

As of December 31, 2012 and 2011, the Company had gross deferred tax assets of approximately $76.5 million and $47.8 million, respectively. Realization of the deferred tax assets is dependent upon the Company generating future taxable income, if any, the amount and timing of which are uncertain. Accordingly, the deferred tax assets have been fully offset by a valuation allowance at December 31, 2012 and 2011. The net valuation allowance increased by approximately $28.6 million and $17.9 million for the years ended December 31, 2012 and 2011, respectively.

Deferred income taxes reflect the net effects of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes and the amounts used for income tax purposes. The Company's deferred tax assets relate primarily to net operating loss carryforwards. Significant components of the Company's deferred tax assets are as follows:

	December 31,	
	2012	2011
	(in thousands)	
Deferred tax assets:		
Net operating loss carryforwards	$ 44,209	$ 28,117
Research credits	22,821	11,005
Capitalized research expenses	4,419	5,686
Stock compensation	3,873	1,558
Impairment loss on available for sale securities/capital loss carryforward	686	694
Other temporary differences	456	789
Total gross deferred tax assets	76,464	47,849
Valuation allowance	(76,464)	(47,849)
Net deferred tax assets	$ —	$ —

A reconciliation of the statutory tax rates and the effective tax rates for the years ended December 31, 2012, 2011 and 2010 is as follows:

	December 31,		
	2012	2011	2010
Statutory rate	(34)%	(34)%	(34)%
State and local income taxes (net of federal tax benefit)	(4)	(6)	(5)
Other	7	12	2
Credits	(15)	(18)	—
Valuation allowance	46	46	26
Effective tax rates	— %	— %	(11)%

As of December 31, 2012, the Company had federal and state net operating loss carryforwards of $121.5 million and $55.4 million, respectively. The Company also had federal and state research and development tax credit carryforwards of $22.2 million and $0.8 million, respectively. The federal net operating loss and tax credit carryforwards will expire at various dates beginning in 2025, if not utilized. The state net operating loss and tax credit carryforwards will expire at various dates starting in 2014, if not utilized. The difference between the statutory tax rate and the effective tax rate is primarily attributable to the valuation allowance offsetting deferred tax assets.

The deferred tax assets above exclude $2.3 million of net operating losses related to tax deductions from the exercise of stock options subsequent to the adoption of the 2006 accounting standard on stock-based compensation. This amount represents an excess tax benefit and has not been included in the gross deferred tax assets.

The Company has not recorded any amounts for unrecognized tax benefits as of December 31, 2012 or 2011.

The Company's policy is to record estimated interest and penalties related to the underpayment of income taxes as a component of its income tax provision. As of December 31, 2012 and 2011, the Company had no accrued interest or tax penalties recorded. The Company's income tax return reporting periods since

December 31, 2009 are open to income tax examination by the federal and state tax authorities. In addition, because the Company has net operating loss carryforwards, the Internal Revenue Service is permitted to audit earlier years and propose adjustments up to the amount of net operating loss generated in those years. There are currently no federal or state audits in progress.

Utilization of the net operating loss carryforwards and credits may be subject to a substantial annual limitation due to the ownership change limitations provided by the Internal Revenue Code of 1986, as amended, and similar state provisions. The Company has not performed a detailed analysis to determine whether an ownership change under Section 382 of the Internal Revenue Code has occurred. The effect of an ownership change would be the imposition of an annual limitation on the use of net operating loss carryforwards attributable to periods before the change.

15. Related Party Transactions

In July 2005, the Company entered into a consulting agreement with Scheer & Company, Inc., a company affiliated with David Scheer, chairman of the Board of Directors and a stockholder of the Company, to provide general corporate advice and consulting. The Company expensed related consulting fees of approximately $19,000 for the year ended December 31, 2010. This consulting agreement was terminated in the connection with the closing of the IPO.

In April 2006, the Company entered into a consulting agreement with Antonio M. Gotto, Jr., M.D., a member of the Board of Directors, for nonspecific consulting activities on behalf of the Company. The Company expensed related consulting fees of approximately $22,000 for the year ended December 31, 2010. This consulting agreement was terminated in the connection with the closing of the IPO. In August 2012, the Company entered into a separate consulting agreement with Dr. Gotto for general scientific and medical consulting activities on behalf of the Company. The Company expensed related consulting fees of $19,500 in the first quarter of 2013.

16. Restructuring

In 2011, the Company consolidated facilities and related administrative functions into its Cambridge, Massachusetts headquarters. As a result, the Company closed its Bedminster, New Jersey facility effective December 31, 2011, and reduced headcount by five positions. The Company accounted for these actions in accordance with ASC 420, *Exit or Disposal Cost Obligations.*

Included in the restructuring charges are employee severance and outplacement service costs for five former employees, primarily in general and administrative positions, the net present value of the remaining lease obligation for the Company's Bedminster, New Jersey facility and a net write off of fixed assets, primarily computer equipment and leasehold improvements, that were no longer in use after vacating the facility, the expense for which was recognized during the quarter ended December 31, 2011.

In addition, the Company accelerated the vesting of 137,136 stock options granted in 2010, 2009, and 2008 to certain former New Jersey employees upon the termination of their employment in connection with the closing of the Bedminster, New Jersey facility. As such, the Company recognized expense related to those stock options in accordance with ASC 718, *Compensation—Stock Compensation.* The Company has recorded as expense the value of the modification over the remaining service periods for each of the employees.

The following table summarizes the Company's restructuring charges recorded to date:

	Year Ended December 31, 2012	Year Ended December 31, 2011	Cumulative to Date
		(in thousands)	
Stock option modification	$1,094	$580	$1,674
Severance and outplacement services	289	165	454
Abandonment of facilities	3	91	94
Write off (recovery) of fixed assets	(20)	76	56
	$1,366	$912	$2,278

In January 2012, the Company entered into a sublease agreement for the Bedminster, New Jersey facility for the remaining term of the lease. In determining the abandonment of facilities charge, the Company considered its sublease arrangement for the facility, including sublease terms and the sublease rates.

The following table summarizes the cash components of the Company's restructuring charges, which is included in accrued liabilities in the accompanying condensed consolidated balance sheets:

	Severance	Abandonment of Facilities	Total
		(in thousands)	
Balance at December 31, 2011	$ 165	$131	$ 296
Costs incurred	289	3	292
Payments made	(454)	(35)	(489)
Balance at December 31, 2012	$ —	$ 99	$ 99

17. Subsequent Events

The Company has evaluated all events or transactions that occurred after December 31, 2012 up through the date the Company issued these financial statements. There were no other material events that impacted the consolidated financial statements or disclosures.

18. Selected Quarterly Financial Data (Unaudited)

	Quarters Ended			
	March 31	June 30	September 30	December 31
	(in thousands, except per share amounts)			
2012				
Net loss attributable to common stockholders	$(11,665)	$(13,927)	$(14,874)	$(21,799)
Basic and diluted net loss per common share	$ (0.55)	$ (0.63)	$ (0.59)	$ (0.86)
2011				
Net loss attributable to common stockholders	$ (6,832)	$ (8,605)	$(10,120)	$(13,911)
Basic and diluted net loss per common share	$ (0.39)	$ (0.49)	$ (0.48)	$ (0.66)

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

None.

Item 9A. Controls and Procedures.

Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Annual Report on Form 10-K, the effectiveness of our disclosure controls and procedures. Based on that evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level in ensuring that information required to be disclosed by us in the reports that we file or submit under the Exchange Act, is recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Based upon that evaluation, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures were effective at December 31, 2012.

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting (as defined in Rule 13a—15(f) under the Exchange Act). Our internal control over financial reporting is a process designed under the supervision of our principal executive officer and principal financial officer to provide reasonable assurance regarding the reliability of financial reporting and the preparation of our financial statements for external purposes in accordance with accounting principles generally accepted in the U.S. of America. Management evaluated the effectiveness of our internal control over financial reporting using the criteria set forth by the Committee of Sponsoring Organizations of the Treadway Commission (COSO) in Internal Control — Integrated Framework. Management, under the supervision and with the participation of the principal executive officer and principal financial officer, assessed the effectiveness of our internal control over financial reporting as of December 31, 2012 and concluded that it was effective at a reasonable assurance level.

Our independent registered public accounting firm, Ernst & Young LLP, has audited our Consolidated Financial Statements included in this Annual Report on Form 10-K and have issued a report on the effectiveness of our internal control over financial reporting as of December 31, 2012. Their report on the audit of internal control over financial reporting appears below.

Changes to Internal Controls Over Financial Reporting

During our fourth quarter of fiscal 2012, there were no changes made in our internal control over financial reporting (as such term is defined in Rules 13a-15(f) and 15d-15(f) of the Exchange Act) that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Item 9B. Other Information.

None.

Report of Independent Registered Public Accounting Firm

The Board of Directors and Shareholders of Aegerion Pharmaceuticals, Inc.

We have audited Aegerion Pharmaceuticals, Inc.'s internal control over financial reporting as of December 31, 2012, based on criteria established in Internal Control—Integrated Framework issued by the Committee of Sponsoring Organizations of the Treadway Commission (the COSO criteria). Aegerion Pharmaceuticals, Inc.'s management is responsible for maintaining effective internal control over financial reporting, and for its assessment of the effectiveness of internal control over financial reporting included in the accompanying Management's Report on Internal Control over Financial Reporting. Our responsibility is to express an opinion on the company's internal control over financial reporting based on our audit.

We conducted our audit in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether effective internal control over financial reporting was maintained in all material respects. Our audit included obtaining an understanding of internal control over financial reporting, assessing the risk that a material weakness exists, testing and evaluating the design and operating effectiveness of internal control based on the assessed risk, and performing such other procedures as we considered necessary in the circumstances. We believe that our audit provides a reasonable basis for our opinion.

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

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

In our opinion, Aegerion Pharmaceuticals, Inc. maintained, in all material respects, effective internal control over financial reporting as of December 31, 2012, based on the COSO criteria.

We also have audited, in accordance with the standards of the Public Company Accounting Oversight Board (United States), the consolidated balance sheets of Aegerion Pharmaceuticals, Inc. as of December 31, 2012 and 2011, and the related consolidated statements of operations, comprehensive loss, stockholders' equity (deficiency), and cash flows for each of the three years in the period ended December 31, 2012 of Aegerion Pharmaceuticals, Inc. and our report dated March 18, 2013 expressed an unqualified opinion thereon.

/s/ Ernst & Young LLP

Metro Park, New Jersey
March 18, 2013

PART III

Item 10. Directors, Executive Officers and Corporate Governance

The information required by this item will be contained in our definitive proxy statement or Proxy Statement, which will be filed with the SEC in connection with our 2013 Annual Meeting of Stockholders. Such information is incorporated herein by reference.

Code of Ethics

Our Board of Directors has adopted a code of business conduct and ethics that applies to our directors, officers and employees. This code is available on the corporate governance section of our website (which is a subsection of the investor relations section of our website) at the following address: www.aegerion.com. We intend to disclose on our website any amendments or waivers to the Code that are required to be disclosed by SEC rules. You may also request a printed copy of the code, without charge, by writing to us at Aegerion Pharmaceuticals, Inc., 101 Main Street, Suite 1850 Cambridge, MA 02142 Attn: Investor Relations.

Item 11. Executive Compensation

The information required by this item will be contained in our Proxy Statement, which will be filed with the SEC in connection with our 2013 Annual Meeting of Stockholders. Such information is incorporated herein by reference.

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

The information required by this item will be contained in our Proxy Statement, which will be filed with the SEC in connection with our 2013 Annual Meeting of Stockholders. Such information is incorporated herein by reference.

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

The information required by this item will be contained in our Proxy Statement, which will be filed with the SEC in connection with our 2013 Annual Meeting of Stockholders. Such information is incorporated herein by reference.

Item 14. Principal Accountant Fees and Services

The information required by this item will be contained in our Proxy Statement, which will be filed with the SEC in connection with our 2013 Annual Meeting of Stockholders. Such information is incorporated herein by reference.

PART IV

Item 15. Exhibits, Financial Statement Schedules.

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

1. Financial statements (see Item 8).
2. All information is included in the financial statements or notes thereto.
3. Exhibits:

   See Exhibit Index.

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned, thereunto duly authorized.

AEGERION PHARMACEUTICALS, INC.

Date: March 18, 2013

By: /s/ Marc D. Beer

Marc D. Beer
Chief Executive Officer
(Principal Executive Officer)

POWER OF ATTORNEY

KNOW ALL PERSONS BY THESE PRESENTS, that each person whose signature appears below appoints severally, Mark J. Fitzpatrick and Anne Marie Cook and each one of them, his or her attorneys-in-fact, each with the power of substitution for him or her in any and all capacities, to sign any and all amendments to this Annual Report on Form 10-K and to file the same, with exhibits thereto and other documents in connection therewith, with the SEC, hereby ratifying and confirming all that each attorneys-in-fact, or his or her substitute or substitutes, may do or cause to be done by virtue hereof.

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

Signature	Capacity	Date
/s/ Marc D. Beer **Marc D. Beer**	Chief Executive Officer (Principal Executive Officer) and Director	March 18, 2013
/s/ Mark J. Fitzpatrick **Mark J. Fitzpatrick**	Chief Financial Officer (Principal Financial Officer and Accounting Officer)	March 18, 2013
/s/ David I. Scheer **David I. Scheer**	Chairman of the Board	March 18, 2013
/s/ Paul Thomas **Paul Thomas**	Director	March 18, 2013
/s/ Sol Barer **Sol Barer**	Director	March 18, 2013
/s/ Antonio M. Gotto Jr. **Antonio M. Gotto Jr.**	Director	March 18, 2013
/s/ Sandford Smith **Sandford Smith**	Director	March 18, 2013

EXHIBIT INDEX

Exhibit Number	Description of Document
3.1	Amended and Restated Certificate of Incorporation, attached as Exhibit 3.2 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
3.2	Amended and Restated By-laws, attached as Exhibit 3.4 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
4.1	Form of specimen certificate evidencing shares of common stock, attached as Exhibit 4.1 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
+10.1	2006 Stock Option and Grant Plan, as amended, and forms of agreement thereunder, attached as Exhibit 10.1 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
+10.2	2010 Stock Option and Incentive Plan, attached as Exhibit 10.2 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
#10.3	Patent License Agreement with University of Pennsylvania, dated May 19, 2006, as amended September 27, 2006, attached as Exhibit 10.6 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
10.4	Form of Indemnification Agreement, attached as Exhibit 10.12 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
10.5	Long-Term Incentive Plan under 2010 Stock Option and Incentive Plan, as attached as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on May 10, 2012, and incorporated herein by reference
+10.6*	Form of Incentive Stock Option Agreement for Executive Officers and forms of Non-Qualified Stock Option Agreement and Restricted Stock Award Agreement for Directors
+10.7*	Amended and Restated Non-Employee Director Compensation Policy, dated as of December 12, 2012
10.8	Lease by and between the Registrant and RREEF America REIT II CORP. PPP, dated January 1, 2011, attached as Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on January 6, 2011, and incorporated herein by reference
10.9	First Amendment to Lease by and between the Registrant and RREEF America REIT II CORP. PPP, dated November 7, 2011, as attached as Exhibit 10.22 to the Registrant's Annual Report on Form 10-K, filed with the SEC on March 15, 2012, and incorporated herein by reference
10.10	Second Amendment to Lease, dated as of September 4, 2012, between the Company and RREEF America REIT II Corp. PPP, as attached as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2012, and incorporated herein by reference
+10.11	Employment Agreement with Marc D. Beer, dated August 19, 2010, attached as Exhibit 10.16 to the Registrant's Registration Statement on Form S-1, as amended, filed with the SEC on August 10, 2010, and incorporated herein by reference
10.12	Loan and Security Agreement dated March 28, 2012 by and between the Company and Silicon Valley Bank, attached as Exhibit 10.1 to the Registrant's Current Report on Form 8-K, filed with the SEC on March 29, 2012, and incorporated herein by reference

Exhibit Number	Description of Document
10.13	First Loan Modification Agreement dated July 10, 2012 by and between the Company and Silicon Valley Bank, as attached as Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2012, and incorporated herein by reference
+10.14	Employment Agreement with Martha Carter, dated February 14, 2011, as attached as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on May 16, 2011, and incorporated herein by reference
+10.15	Employment Agreement with Mark Fitzpatrick, dated May 9, 2011, as attached as Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on May 16, 2011, and incorporated herein by reference
+10.16	Employment Agreement with Mark Sumeray, dated May 9, 2011, as attached as Exhibit 10.1 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 14, 2011, and incorporated herein by reference
+10.17	Amendment No. 1 to Employment Agreement with Mark Sumeray, dated May 9, 2011, as attached as Exhibit 10.2 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 14, 2011, and incorporated herein by reference
+10.18	Employment Agreement with Anne Marie Cook, dated December 5, 2011, as attached as Exhibit 10.19 to the Registrant's Annual Report on Form 10-K, filed with the SEC on May 15, 2012, and incorporated herein by reference
+10.19	Employment Agreement with Craig Fraser, dated November 7, 2011, as attached as Exhibit 10.3 to the Registrant's Quarterly Report on Form 10-Q, filed with the SEC on November 9, 2012, and incorporated herein by reference
+10.20*	Separation Agreement and Release with Mark Rothera, dated as of November 5, 2012
+10.21	Inducement Award Stock Option Plan and form of option agreement thereunder, attached as Exhibit 4.4 to the Registrant's Registration Statement on Form S-8 filed with the SEC on January 31, 2013, and incorporated herein by reference
23.1*	Consent of Ernst & Young LLP, Independent Registered Accounting Firm
24.1*	Power of Attorney (contained on signature page hereto)
31.1*	Certification of Marc D. Beer, Chief Executive Officer of the Company, filed pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (18 U.S.C. 1350)
31.2*	Certification of Mark J. Fitzpatrick, Chief Financial Officer of the Company, filed pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 (18 U.S.C. 1350)
32.1**	Certifications of Marc D. Beer, Chief Executive Officer of the Company, and Mark J. Fitzpatrick, Chief Financial Officer of the Company, furnished pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 (18 U.S.C. 1350)
101**	The following materials from Aegerion Pharmaceuticals, Inc.'s Annual Report on Form 10-K for the year ended December 31, 2012, are formatted in XBRL (Extensible Business Reporting Language): (i) the Consolidated Balance Sheets, (ii) the Consolidated Statements of Operations, (iii) the Consolidated Statements of Stockholders' Equity (Deficiency), (iv) the Consolidated Statements of Cash Flows and (v) Notes to Consolidated Financial Statements.

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* Filed herewith.

** Furnished herewith.

Confidential treatment has been received for certain provisions of this Exhibit. Confidential materials have been omitted and filed separately with the SEC.

\+ Management contract or compensatory plan or arrangement.

Exhibit 31.1

CERTIFICATIONS

I, Marc D. Beer, certify that:

1. I have reviewed this annual report on Form 10-K of Aegerion Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 18, 2013

/s/ Marc D. Beer

Name:Marc D. Beer
Title: Chief Executive Officer (Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, Mark J. Fitzpatrick, certify that:

1. I have reviewed this annual report on Form 10-K of Aegerion Pharmaceuticals, Inc.;

2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;

3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;

4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

   (a) Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;

   (b) Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;

   (c) Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and

   (d) Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and

5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):

   (a) All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and

   (b) Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: March 18, 2013

/s/ Mark J. Fitzpatrick

Name:Mark J. Fitzpatrick
Title: Chief Financial Officer (Principal Financial Officer)

Exhibit 32.1

CERTIFICATIONS PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with this Annual Report on Form 10-K of Aegerion Pharmaceuticals, Inc. (the "Company") for the fiscal year ended December 31, 2011 as filed with the Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers of the Company hereby certifies, pursuant to 18 U.S.C. (section) 1350, as adopted pursuant to (section) 906 of the Sarbanes-Oxley Act of 2002, that to the best of his knowledge:

(1) The Report fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934; and

(2) The information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

/s/ Marc D. Beer

Name: Marc D. Beer
Title: Chief Executive Officer (Principal Executive Officer)
Date: March 18, 2013

/s/ Mark J. Fitzpatrick

Name: Mark J. Fitzpatrick
Title: Chief Financial Officer (Principal Financial Officer)
Date: March 18, 2013

Executive Management Team

Marc Beer
Chief Executive Officer

Massimo Boriero, MD
President, EMEA

Martha Carter
Chief Regulatory Officer

Anne Marie Cook
Senior Vice President, General Counsel and Secretary

Mark Fitzpatrick
Chief Financial Officer

Craig Fraser
President, U.S. Commercial & Global Manufacturing and Supply Chain

Mark Sumeray, MD, MS, FRCS
Chief Medical Officer

Mary Weger
Senior Vice President, Human Resources

Board of Directors

David Scheer
Chairman of the Board, Aegerion Pharmaceuticals

Marc Beer
CEO, Aegerion Pharmaceuticals

Dr. Sol J. Barer, PhD
Former Chairman and CEO, Celgene

Dr. Antonio M. Gotto Jr., MD, DPhil
Co-Chairman of the Board of Overseers, Weill Medical College of Cornell University

Sandford ("Sandy") D. Smith
Former EVP and President International Group, Genzyme

Paul G. Thomas
Founder & CEO, Roka Bioscience

Anne VanLent
President, AMV Advisors

Annual Meeting

The Annual Meeting of Stockholders will be held Wednesday, June 26, 2013, at 9:00 a.m., ET, at One Main Street, East Arcade Conference Center, Cambridge, Massachusetts 02142.

Independent Auditors

Ernst & Young LLP, Boston, MA

Investor Inquiries

857.242.5024
amurphy@aegerion.com

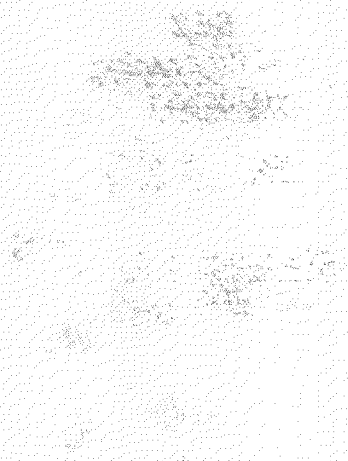
Stock Listing

NASDAQ: AEGR

Transfer Agent

Registrar and Transfer Company
Investor Relations Department
10 Commerce Drive, Cranford, NJ 07016
800.368.5948

Aegerion®
Pharmaceuticals



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Cambridge, MA 02142

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855.305.AEGR (2347)

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