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

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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.

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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

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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.

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Explore Litigation Insights

Docket Alarm provides insights to develop a more informed litigation strategy and the peace of mind of knowing you're on top of things.

Real-Time Litigation Alerts

Keep your litigation team up-to-date with **real-time alerts** and advanced team management tools built for the enterprise, all while greatly reducing PACER spend.

Our comprehensive service means we can handle Federal, State, and Administrative courts across the country.

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With over 230 million records, Docket Alarm's cloud-native docket research platform finds what other services can't. Coverage includes Federal, State, plus PTAB, TTAB, ITC and NLRB decisions, all in one place.

Identify arguments that have been successful in the past with full text, pinpoint searching. Link to case law cited within any court document via Fastcase.

Analytics At Your Fingertips

Learn what happened the last time a particular judge, opposing counsel or company faced cases similar to yours.

Advanced out-of-the-box PTAB and TTAB analytics are always at your fingertips.

API

Docket Alarm offers a powerful API (application programming interface) to developers that want to integrate case filings into their apps.

LAW FIRMS
Build custom dashboards for your attorneys and clients with live data direct from the court.

Automate many repetitive legal tasks like conflict checks, document management, and marketing.

FINANCIAL INSTITUTIONS
Litigation and bankruptcy checks for companies and debtors.

E-DISCOVERY AND LEGAL VENDORS
Sync your system to PACER to automate legal marketing.