October 17, 2012

FDA Advisory Committee Recommends Approval of Lomitapide for Treatment of Homozygous Familial Hypercholesterolemia (HoFH)

CAMBRIDGE, Mass., Oct. 17, 2012 (GLOBE NEWSWIRE) -- **Aegerion Pharmaceuticals, Inc.** (Nasdaq:AEGR), an emerging biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat debilitating and often fatal rare diseases, announced that the Endocrinologic and Metabolic Drugs Advisory Committee (EMDAC) of the U.S. Food and Drug Administration (FDA) determined by a vote of 13 to 2 that Aegerion has presented sufficient safety and efficacy data to support marketing of its product, lomitapide, for the treatment of patients with Homozygous Familial Hypercholesterolemia (HoFH) when used as an adjunct to a low-fat diet and other lipid-lowering therapies.

"Today's Advisory Committee recommendation is an important milestone in our mission to provide patients with HoFH with urgently needed new treatment options to lower their cholesterol levels," said Marc Beer, Chief Executive Officer of Aegerion Pharmaceuticals. "We look forward to continuing to work closely with the FDA as the agency completes its review of our new drug application for lomitapide."

The FDA has assigned a Prescription Drug User Fee Act (PDUFA) action date of December 29, 2012, for completion of its review of the New Drug Application (NDA) for lomitapide. The EMDAC provides the FDA with independent expert advice and recommendations. The FDA is not bound by the EMDAC's recommendation, but will consider the committee's recommendation as the FDA completes its review of the lomitapide NDA.

Conference Call Details

The Aegerion management team will hold a conference call with the investment community to discuss the outcome of the EMDAC meeting today at 7:00 pm EDT. To listen to the live conference call, dial (866) 516-3002 (International callers dial (760) 298-5082). In addition, the conference call will be available through a live audio webcast in the "Investors" section of the Aegerion website, www.aegerion.com. The conference call will be archived and accessible on the same website shortly after the conclusion of the call.

About Lomitapide

Lomitapide is a microsomal triglyceride transfer protein (MTP) inhibitor that Aegerion is developing as a once-day capsule for the treatment of patients afflicted with certain severe lipid disorders, including HoFH. MTP exists in both the liver and intestines where it plays a role in the formation of lipoproteins containing cholesterol and triglycerides. Inhibiting MTP reduces the level of cholesterol that the liver and intestines assemble and secrete into the bloodstream. Currently, there is no MTP inhibitor approved by the FDA for any indication.

Lomitapide has been evaluated in fourteen Phase I and eight Phase II clinical trials, as well as a pivotal Phase III clinical trial in HoFH completed in 2011. An extension study to assess long-term safety is ongoing. Over 900 patients have been treated with lomitapide as part of these clinical trials.

The most frequent adverse events in the Phase III clinical trial were gastrointestinal, and were generally mild to moderate. These events typically decreased after the patients were established on the maximally tolerated dose. Elevations in liver enzymes and hepatic fat were also observed in the Phase III trial. Four patients experienced elevations in liver enzymes of between five times to eleven times the upper limit of normal. Hepatic fat increased from a baseline of 1% to 8.3% at week 26, and then stabilized through week 78.

Aegerion submitted a NDA to the Food and Drug Administration (FDA), and 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, total cholesterol, apolipoprotein B, and triglycerides in adults with HoFH.

About Homozygous Familial Hypercholesterolemia (HoFH)

HoFH is a rare genetic lipid disorder that, if left untreated, results in extremely high cholesterol levels, typically between 400 mg/dL and 1,000 mg/dL. Those affected are at severely high risk of experiencing premature cardiovascular events, such as heart attack or stroke, often experiencing their first cardiovascular event in their twenties. Despite current treatments, many

patients with HoFH do not survive beyond their mid-30's.

The disease is usually caused by defects in the low-density lipoprotein (LDL) receptor genes, resulting in impaired or total loss of function. The LDL receptor is a protein on the surface of cells that is responsible for binding to and removing cholesterol from the blood. A loss of LDL receptor function results in elevated accumulation of cholesterol in the blood.

About Aegerion Pharmaceuticals, Inc.

Aegerion Pharmaceuticals, Inc.is an emerging biopharmaceutical company focused on the development and commercialization of novel, life-altering therapeutics to treat debilitating and often fatal rare diseases. The company's lead drug candidate, lomitapide, is in late-stage development for the treatment of Homozygous Familial Hypercholesterolemia (HoFH), a rare life-threatening disease characterized by severely elevated cholesterol levels.

Aegerion is motivated by its commitment to patients first, as well as its core values of integrity, innovation, responsibility to healthcare providers and development of employees, with a constant focus on scientific and clinical excellence. For more information, visit www.aegerion.com.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the potential for regulatory approval and launch of lomitapide. These forward-looking statements are neither promises nor guarantees of future performance, and are subject to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. In particular, the risks and uncertainties include, among other factors: the risk that applicable regulatory authorities may ask for additional data, information or studies to be completed or provided prior to approval; the risk that the FDA may not follow the recommendations of EMDAC; the risk that applicable regulatory authorities may not agree with our validation plan or may require additional work related to the commercial manufacturing process to be completed prior to approval or may, in the course of the inspection of manufacturing facilities, identify issues to be resolved; the risks that the applicable regulatory authorities may not be satisfied with the safety profile of lomitapide; and the risk that we do not receive approval of lomitapide on a timely basis or at all. For additional disclosure regarding these and other risks we face, see the disclosure contained in our public filings with the U.S. Securities and Exchange Commission (available on the SEC's website at http://www.sec.gov), including the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q. We undertake no obligation to update or revise the information contained in this press release, whether as a result of new information, future events or circumstances or otherwise.

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