Aegerion Pharmaceuticals

December 24, 2012

FDA Approves Aegerion Pharmaceuticals' JUXTAPID(TM) (lomitapide) Capsules for Homozygous Familial Hypercholesterolemia (HoFH)

CAMBRIDGE, Mass., Dec. 24, 2012 (GLOBE NEWSWIRE) -- Aegerion Pharmaceuticals, Inc. (Nasdaq:AEGR), a biopharmaceutical company dedicated to the development and commercialization of novel, life-altering therapies for patients with debilitating, often fatal, rare diseases, today announced that the U.S. Food & Drug Administration (FDA) has approved JUXTAPID™ (lomitapide) capsules as an adjunct to a low-fat diet and other lipid-lowering treatments, including 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) in patients with homozygous familial hypercholesterolemia (HoFH).

"We are excited that JUXTAPID will become a new treatment option for patients with HoFH," said Marc Beer, Chief Executive Officer at Aegerion. "The approval of our first product also marks an important corporate milestone for Aegerion and reflects our commitment to help patients in need."

HoFH is a serious, rare genetic disease that impairs the function of the receptor responsible for removing LDL-C ("bad" cholesterol) from the body. A loss of LDL receptor function results in extreme elevation of blood cholesterol levels. HoFH patients often develop premature and progressive atherosclerosis, a narrowing or blocking of the arteries.

"The FDA approval of JUXTAPID is a major step forward for HoFH patients and their families, who have long been waiting for new therapies," said Katherine Wilemon, president and founder of The FH Foundation. "New treatments, combined with further understanding and awareness of this disease, can bring much needed hope to the HoFH community."

JUXTAPID contains a Boxed Warning citing the risk of hepatic toxicity. See below for Important Safety Information about JUXTAPID, including the Boxed Warning, Contraindications and Warnings and Precautions. The safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH. The effect of JUXTAPID on cardiovascular morbidity and mortality has not been determined. The safety and effectiveness of JUXTAPID have not been established in pediatric patients.

The FDA based its approval of JUXTAPID on Aegerion's pivotal Phase III study, which evaluated the safety and effectiveness of the medicine to reduce LDL-C levels in 29 adult patients with HoFH. The study was a multinational, single-arm, open-label, 78 week trial that was recently published in the November 2, 2012 online version of the *Lancet*. In this study, JUXTAPID was initiated at 5 mg daily and gradually escalated to doses of 10 mg, 20 mg, 40 mg, up to 60 mg, based on tolerability and acceptable liver enzymes levels.

When added to the existing lipid-lowering therapy of the HoFH patients in the study, JUXTAPID significantly reduced LDL-C from a baseline average of 336 mg/dL to 190 mg/dL (40% reduction) at Week 26 in the intent-to-treat population with last observation carried forward for the patients who discontinued prematurely. LDL-C was reduced by an average of 50 percent for the 23 patients who completed the study through Week 26. After Week 26, during the 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 III trial were gastrointestinal, reported by 27 (93%) of 29 patients. Adverse reactions, which were reported by ≥8 patients (28%) in the HoFH clinical trial, included diarrhea, nausea, vomiting, dyspepsia and abdominal pain. Other common adverse reactions, reported by 5 to 7 (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, including four patients who experienced liver enzymes greater than or equal to five times the upper limit of normal. 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 percent to a median absolute increase of 6 percent at 26 and 78 weeks.

Because of the risk of liver toxicity, JUXTAPID is available only through a restricted program called the JUXTAPID Risk Evaluation and Mitigation Strategy (REMS) Program. Aegerion will certify all health care providers who prescribe JUXTAPID and the pharmacies that will dispense the medicine. The goals of the REMS 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.

- To restrict access to therapy with JUXTAPID to patients with a clinical or laboratory diagnosis consistent with HoFH.

The safety and effectiveness of JUXTAPID have not been established in patients with hypercholesterolemia who do not have HoFH. The effect of JUXTAPID on cardiovascular morbidity and mortality has not been determined. The safety and effectiveness of JUXTAPID have not been established in pediatric patients.

To further understand JUXTAPID's long-term safety and effectiveness, Aegerion has made a commitment to the FDA to conduct a post-approval, observational cohort study.

As part of the planned launch of JUXTAPID, the company has developed a comprehensive support services program for patients and their healthcare providers. For more information, call this toll-free number, 1-85JUXTAPID (1-855-898-2743).

Important Safety Information, including BOXED WARNING which states:

WARNING: RISK OF HEPATOTOXICITY

JUXTAPID can cause elevations in transaminases. In the JUXTAPID clinical trial, 10 (34%) of the 29 patients treated with JUXTAPID had at least one elevation in alanine aminotransferase (ALT) or aspartate aminotransferase (AST) ≥ 3x upper limit of normal (ULN). There were no concomitant clinically meaningful elevations of total bilirubin, international normalized ratio (INR), or alkaline phosphatase.

JUXTAPID also increases hepatic fat, with or without concomitant increases in transaminases. The median absolute increase in hepatic fat 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.

Measure ALT, AST, alkaline phosphatase, and total bilirubin before initiating treatment and then ALT and AST regularly as recommended. During treatment, adjust the dose of JUXTAPID if the ALT or AST are ≥3x ULN. Discontinue JUXTAPID for clinically significant liver toxicity.

Because of the risk of hepatotoxicity, JUXTAPID is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the JUXTAPID REMS Program.

CONTRAINDICATIONS

- Pregnancy
- Concomitant administration of moderate or strong CYP3A4 inhibitors
- Moderate or severe hepatic impairment or active liver disease including unexplained persistent elevations of serum transaminases

WARNINGS AND PRECAUTIONS

JUXTAPID can cause elevations in transaminases and hepatic steatosis. Although cases of hepatic failure have not been reported, there is concern that JUXTAPID could induce steatohepatitis, which can progress to cirrhosis over several years. Modify the dose of JUXTAPID if elevations of transaminases are observed and discontinue JUXTAPID for persistent or clinically significant elevations. If transaminase elevations are accompanied by clinical symptoms of liver injury, increases in bilirubin ≥2x ULN, or active liver disease, discontinue treatment with JUXTAPID and identify the probable cause. Use JUXTAPID with caution when co-administered with agents known to be hepatotoxic. Alcohol may increase levels of hepatic fat and induce or exacerbate liver injury.

Measure ALT, AST, alkaline phosphatase, and total bilirubin before initiating treatment. During the first year, measure liver-related tests (ALT and AST at a minimum) prior to each increase in dose or monthly, whichever occurs first. After the first year, do these tests at least every 3 months and before any increase in dose.

Females of reproductive potential should have a negative pregnancy test before starting JUXTAPID and should use effective contraception during therapy with JUXTAPID.

Given its mechanism of action in the small intestine, JUXTAPID may reduce the absorption of fat-soluble nutrients. Patients treated with JUXTAPID should take daily supplements that contain 400 international units vitamin E and at least 200 mg linoleic acid, 210 mg alpha-linolenic acid (ALA), 110 mg eicosapentaenoic acid (EPA), and 80 mg docosahexaenoic acid (DHA).

Gastrointestinal adverse reactions are common and may lead to treatment discontinuation. To reduce the risk of gastrointestinal adverse reactions, patients should adhere to a low-fat diet supplying less than 20% of energy from fat and the dosage of JUXTAPID should be increased gradually.

Combination with CYP3A4 inhibitors increases exposure to lomitapide. Strong and moderate CYP3A4 inhibitors should not be used with JUXTAPID. JUXTAPID dosage should not exceed 30 mg daily when used concomitantly with weak CYP3A4 inhibitors.

Due to risk of myopathy associated with simvastatin or lovastatin, doses of these agents should be limited when co-administered with JUXTAPID.

JUXTAPID increases the plasma concentrations of warfarin. Increases or decreases in the dose of JUXTAPID may lead to supra- or subtherapeutic anticoagulation, respectively. Patients taking warfarin should undergo regular monitoring of the INR, especially after any changes in JUXTAPID dosage.

Avoid use of JUXTAPID in patients with rare hereditary disorders of galactose intolerance.

Conference Call Details

The Aegerion management team will hold a conference call with the investment community Monday, December 24, 2012, at 8:30 a.m. EST. 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 accessible on the same website shortly after the conclusion of the call and archived there for up to 90 days.

About Aegerion Pharmaceuticals

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. Our first approved product, JUXTAPID, is an oral once-daily capsule that offers a new treatment option to patients with homozygous familial hypercholesterolemia (HoFH) — a severe lipid disorder. For more information about the company, please visit www.aegerion.com.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding the availability of JUXTAPID and launch of JUXTAPID in the U.S., and the potential for JUXTAPID as a treatment for HoFH. 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 any delay or technical hurdle in completion of our validation work may delay availability of JUXTAPID for launch; the risk that JUXTAPID may not gain market acceptance; and the risk that restrictions imposed by regulatory authorities or the side effect profile may limit the commercial potential of JUXTAPID. 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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