News & Events

FDA NEWS RELEASE

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FDA approves new orphan drug for rare cholesterol disorder

On Dec. 21, the U.S. Food and Drug Administration approved Juxtapid (lomitapide) to reduce low-density lipoprotein (LDL) cholesterol, total cholesterol, apolipoprotein B, and non-high-density lipoprotein (non-HDL) cholesterol in patients with homozygous familial hypercholesterolemia (HoFH). Juxtapid is intended for use in combination with a low fat diet and other lipid-lowering treatments.

HoFH is a rare inherited condition that makes the body unable to remove LDL cholesterol, often called the "bad" cholesterol, from the blood, causing abnormally high levels of circulating LDL cholesterol. In the United States, HoFH occurs in approximately one in one million individuals. For those with HoFH, heart attacks and death often occur before age 30. Juxtapid works by impairing the creation of the lipid particles that ultimately give rise to LDL.

Juxtapid is a capsule taken once a day, without food, and at least two hours after the evening meal. Patients should take supplements that contain fat-soluble vitamins and essential fatty acids daily while taking Juxtapid.

"Juxtapid, in addition to diet changes and other cholesterol-lowering treatments, is a new option for those suffering with HoFH and the serious health consequences resulting from this condition," said Eric Colman, M.D., deputy director of the Division of Metabolism and Endocrinology Products in the FDA's Center for Drug Evaluation and Research.

The safety and effectiveness of Juxtapid were evaluated in a clinical trial of 29 patients with HoFH. On average, levels of LDL cholesterol fell by approximately one-half during the first 26 weeks among those who tolerated the drug. Juxtapid carries a Boxed Warning regarding a serious risk of liver toxicity because it is associated with liver enzyme abnormalities and accumulation of fat in the liver, which could potentially lead to progressive liver disease with chronic use. Juxtapid also reduces the absorption of fat-soluble nutrients and interacts with several other medications.

The FDA approved Juxtapid with a Risk Evaluation and Mitigation Strategy (REMS) that consists of elements to ensure safe use including prescriber and pharmacy certification and documentation of safe-use conditions consisting of a prescription authorization form that will be required to accompany each new prescription.

The FDA is requiring three postmarketing studies for Juxtapid: an animal study to evaluate the potential for toxicity in children and teens; a long-term registry of patients with HoFH treated with Juxtapid to determine the long-term safety; and an enhanced pharmacovigilance program to monitor reports of malignancy, teratogenicity, and hepatic abnormalities.

The most common adverse reactions in the clinical trial included diarrhea, nausea, vomiting, indigestion, and abdominal pain.

Juxtapid is marketed by Cambridge, Mass.-based Aegerion Pharmaceuticals Inc.

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