Drug OK'd for deadly genetic condition tied to cholesterol

January 30 2013

(HealthDay)—Kynamro (mipomersen sodium) has been approved by the U.S. Food and Drug Administration to treat a rare inherited condition in which the body can't remove low-density lipoprotein (LDL) cholesterol from the blood.

LDL is the so-called "bad" cholesterol that can clog the arteries and cause heart attack and stroke. Many people with homozygous familial hypercholesterolemia (HoFH) have a heart attack and die before age 30, the FDA said in a news release.

HoFH affects approximately one of every 1 million people in the United States. Kynamro is a once-weekly injection designed to lower creation of blood lipid particles that ultimately form LDL, the agency said.

The drug was clinically evaluated among 51 people with HoFH. Among Kynamro users, LDL levels fell an average of about 25 percent during the first 26 weeks, the FDA said. The drug will carry a "black box" label warning of possible liver abnormalities that could lead to progressive liver disease.

More common side effects noted during clinical testing included injection-site reactions, flu-like symptoms, nausea, headache and elevated liver enzymes.

Kynamro is produced by Genzyme Corp., of Cambridge, Mass.

More information: To learn more about high cholesterol, visit the U.S. National Library of Medicine.