FDA panel backs first-in-class cholesterol drug

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Federal health advisers on Tuesday recommended approval for a highly-anticipated cholesterol drug from Sanofi and Regeneron Pharmaceuticals, but with the caveat that more data is needed about its long-term ability to reduce heart attacks.

The expert panel recommended by a 13-3 vote that the Food and Drug Administration approve the injectable drug, called Praluent.

But in an unexpected development, a number of panelists said the drug should only be used in patients with abnormally high cholesterol levels caused by an inherited disorder. Those panelists said they wanted to see more data about whether the drug ultimately reduces heart problems, before it is used more broadly.

"I personally fall on the side of having optimism, but I need to see the cardiovascular outcome study to know," said Dr. Philip Sanger of Stanford University, who voted for the drug.

Panelists said they would like to see follow-up data on a number of other potential side effects suggested by shorter company studies, including cognitive problems, allergic reactions and diabetes.

The FDA is not required to follow the group's advice, though it often does. The same panel of experts will review a similar drug from Amgen Inc. on Wednesday.

Both drugs block a substance called PCSK9, which interferes with the liver's ability to remove cholesterol from the blood. They lower cholesterol more than older medications called statins, which have been the standard treatment for more than 20 years.

Analysts expect the PCSK9 drugs to grow into a blockbuster class of medicines, generating tens of billions of dollars in new sales. But Tuesday's FDA panel review underscores the uncertainty about who should receive the drugs.

More than 73 million U.S. adults, or nearly one-third, have high LDL cholesterol, according to the Centers for Disease Control and Prevention.

Those patients have twice the risk of heart disease, the leading cause of death in the U.S.

Sanofi and Regeneron suggested marketing their drug to a broad swath of patients, including those who are at high-risk for heart attacks and those who cannot tolerate statin drugs due to side effects.

But many panelists said they would only be comfortable recommending the drug to a much narrower group of patients who have abnormally high cholesterol levels due to a genetic disorder, known as familial hypercholesterolemia.