MedicalXpress

FDA weighs target population for Amgen cholesterol drug

June 8 2015, byMatthew Perrone

Federal health regulators said Monday a highly-anticipated, experimental drug from Amgen significantly lowers bad cholesterol. But officials have questions about who should take the drug and whether to approve it based on currently available data.

The Food and Drug Administration posted its review of Amgen's Repatha ahead of a public meeting to consider its approval. Repatha is the part of a new class of injectable, cholesterol-lowing drugs that work differently than older, statin drugs. The new drugs are considered the first major advance in lowering bad, or LDL, cholesterol in more than 20 years, and analysts expect them to generate billions in sales

But the prospect of approving pricey, new injectable drugs for one of the most common medical conditions in America is already drawing concerns from health insurers, providers and pharmacy benefits managers.

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 FDA is considering which patients should receive a prescription for drugs like Repatha.

Amgen Inc., based in Thousand Oaks, Calif., studied the drug in several different patient groups, including those already taking statins, those who

cannot take statins due to side effects, and patients with a rare genetic disorder that causes extremely high cholesterol levels.

On Wednesday the FDA will ask a panel of outside experts which patients are most likely to benefit from the drug, considering potential risks seen in studies, including higher rates of pancreatitis and kidney problems. The same panel of experts will review a similar drug from Sanofi on Tuesday.

Both drugs block a substance called PCSK9, which interferes with the liver's ability to remove cholesterol from the blood.

Key to FDA's consideration of both drugs is whether they ultimately reduce heart attacks and death in patients. For the last 20 years, the FDA has approved cholesterol drugs based on their ability to lower levels of the wax-like substance found in the bloodstream. Studies in older statin drugs have shown this reduction results in fewer heart problems. But several drug cases in the last decade have shown that lowering cholesterol does not always translate into real benefits for patients.

Amgen is conducting a 27,500-patient study to determine whether Repatha reduces heart attacks, but the results aren't expected before 2017. The FDA's experts will vote on whether Repatha should be approved despite the lack of cardiovascular data. The agency is not required to follow the group's recommendation, though it often does.

Companies that would pay out on those costs, like insurers, have pointed out the lack of long-term safety data as a reason to go slowly.

"We've seen lots of drugs that were touted as wonderful pulled from the market after large numbers of people got on them and it became clear there were side effects not seen in the initial trials," said Steve Miller, chief medical officer with Express Scripts, the nation's largest pharmacy

benefit manager.

Express Scripts has been one of the most vocal critics of escalating prices for specialty drugs, an issue that recently came to a head with the $1,000-a-pill price tag for Gilead Science's hepatitis C drug, Sovaldi. While Amgen will not discuss pricing plans for Repatha—and the FDA is barred from considering cost when reviewing drugs—Express Scripts and other companies are already raising concerns about the impact on health care budgets.

Some analysts estimate new PCSK9 drugs could cost about $10,000 per year, far more than currently-used statins, which usually run several hundred dollars per year. If 10 million U.S. patients take the new drugs, that could result in approximately $100 billion in new drug spending, according to Express Scripts.

"That's why there's so much anxiety about these coming products," Miller said.

Pharmacy benefit managers like Express Scripts are paid by insurers and employers to manage drug costs. Express Scripts ultimately lowered its hepatitis C drug bill by refusing to cover Gilead Science's medications and cutting a deal on rival Abbvie's competing drug. Miller said the company would take a similar strategy with new cholesterol drugs, using competition to extract pricing concessions.

The FDA is scheduled to make an approval decision on Sanofi's drug by July 24. A decision on Amgen's drug is due by August 27.

Shares of Amgen Inc. rose 8 cents Monday to $86.96 in afternoon trading.