Experimental anti-cholesterol drug shows promise

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Cholesterol test

People taking an experimental drug called Repatha (evolocumab) for high cholesterol were half as likely to die or suffer a heart attack or stroke as those taking conventional statins, researchers said Sunday.

The findings were based on 4,465 patients who were studied for one year after completing an earlier phase of the drug's safety and efficacy testing.

The results could offer an alternative to the estimated one in three Americans with high low-density lipoprotein (LDL) cholesterol who have been unable to manage their condition with diet, exercise and statin drugs currently on the market.

Patients were randomized to either receive evolocumab, made by the

pharmaceutical company Amgen, injected under the skin in addition to standard care, or standard care alone, which meant taking the cholesterol-lowering statin drug recommended by their physician.

Evolocumab works differently than traditional statins. It is a human monoclonal antibody that blocks a harmful protein in the liver, freeing the organ up to remove LDL cholesterol from the blood.

This new class of drug is known as a PCSK9 inhibitor, and three different kinds, including evolocumab, are being studied in large clinical trials.

Previous research has shown evolocumab could lower LDL or "bad" cholesterol—the kind that contributes to plaque buildup in the arteries—by 61 percent.

The drug has yet to be approved by the US Food and Drug Administration, and more years of study are planned to test its longer term outcomes.

But after one year, the analysis showed the rate of cardiovascular events—such as death, heart attack, stroke, hospitalization or surgery to open blocked arteries—in the evolocumab group was 0.95 percent, compared to 2.18 percent in the traditional statin group, most of whom were taking moderate or high intensity regimens of the cholesterol-lowering drugs.

"The reduction in LDL was profound and that may be why we saw a marked reduction in cardiovascular events so quickly," said lead author Marc Sabatine, senior physician in the Division of Cardiovascular Medicine at Brigham and Women's Hospital in Boston.

"It suggests that if we can drive a patient's LDL cholesterol down a large

amount to a very low level, we may start to see a benefit sooner than would be expected with a more modest intervention."

High cholesterol is a major risk factor for heart disease, the leading killer worldwide.

The study, which was funded by the drugmaker, Amgen, showed few cardiovascular events among both groups—just 60 total, so more data from a long-term study of 27,000 people expected in 2017 should shed more light on the drug's effectiveness.

The FDA may decide to make the treatment available after an expected review of the data later this year.

"We won't have any definitive answers until this larger trial we are doing is complete, but these data now give us a sense for the potential clinical benefit of these drugs," Sabatine said.

The results were presented at the American College of Cardiology annual conference in San Diego, California, and published simultaneously in the *New England Journal of Medicine*.