New drug might reduce sickle cell pain crises

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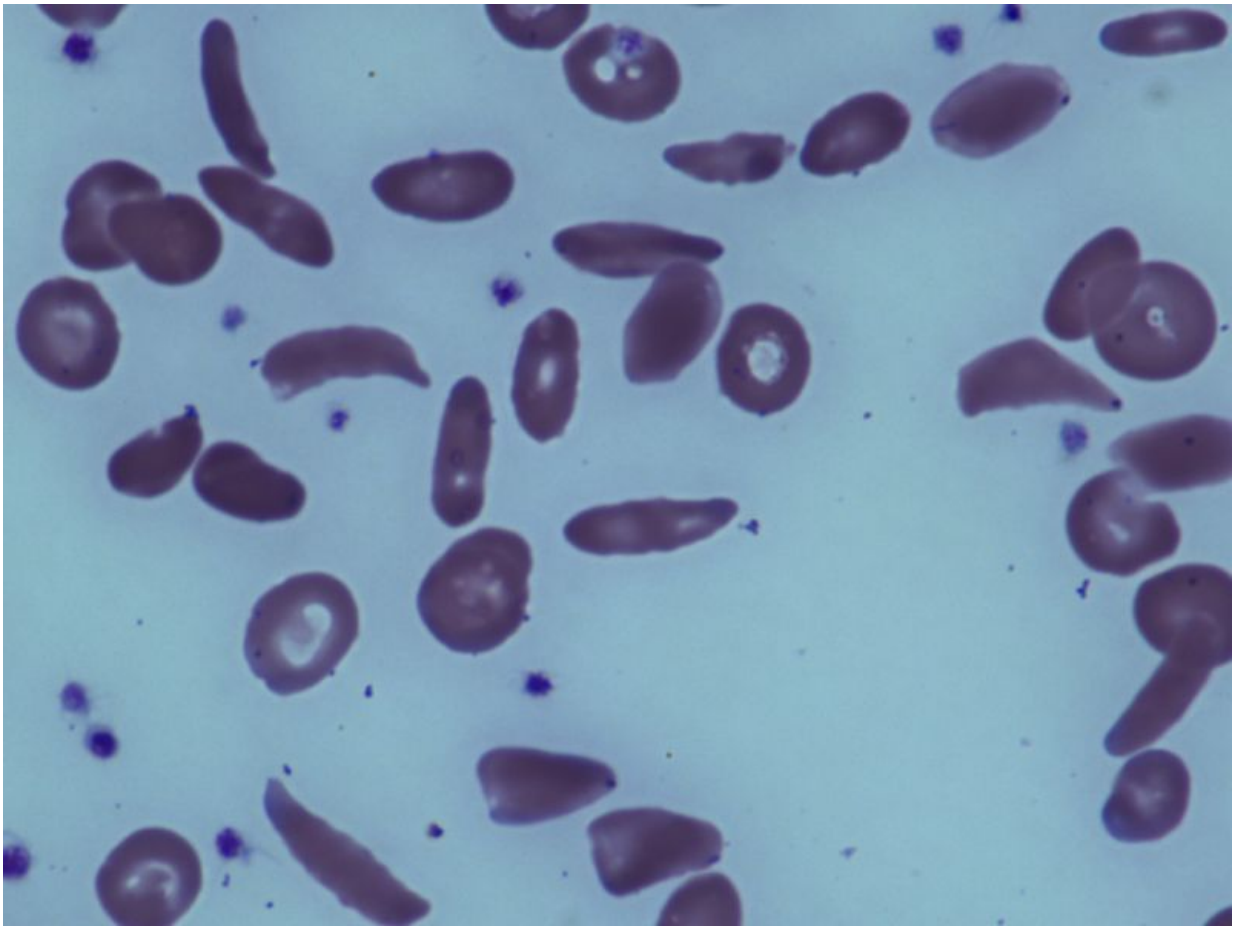

(HealthDay)—An experimental drug may help reduce episodes of pain associated with sickle cell disease, a new study finds.

Results of an early trial showed the drug—called SelG1 for now—reduced episodes of sickle cell-related pain crises by 45 percent. In addition, the drug appeared safe and was well-tolerated, researchers say.

"Patients with sickle cell disease have complications, the most common of which is pain crises that require medical care and sometimes hospitalization," said lead researcher Dr. Kenneth Ataga. He's director of the sickle cell program at the University of North Carolina at Chapel Hill.

People with inherited sickle cell disease have abnormally shaped red blood cells. These sickle-shaped cells stick to the walls of blood vessels, slowing and blocking normal blood flow. Blockage results in less blood and oxygen reaching cells, which in turn causes severe muscle pain.

According to the U.S. Centers for Disease Control and Prevention, roughly 100,000 Americans have sickle cell disease, with blacks disproportionately affected.

"We don't have many treatments for this pain," Ataga said. Currently, only one drug, hydroxyurea, is available to help prevent these episodes. And many patients continue to have acute pain episodes despite the therapy.

SelG1, an antibody, works differently from hydroxyurea, Ataga said. SelG1 attacks the molecule P-selectin, which is responsible for blocking normal blood flow. By stopping P-selectin, the drug keeps blood flowing, thus preventing the pain crises, he explained.

For their study, Ataga and colleagues randomly assigned 198 patients with sickle cell disease to receive one of two IV doses of SelG1 or a placebo. Patients received an initial dose, another dose two weeks later,

then a dose every four weeks for 50 weeks for a total of 14 doses.

Over the year of the study, patients who received the highest dose of SelG1 experienced a 45 percent reduction in the rate of sickle cell pain crises, the researchers found.

Side effects occurred in 5 percent or more of patients receiving SelG1. These included joint pain, itching, vomiting, chest pain, diarrhea, fatigue, muscle pain and stomach pain, the study reported.

The trial was the second of three research phases required for drug approval in the United States.

Since this trial, the drug giant Novartis has acquired Selexys Pharmaceuticals Corp., which developed SelG1 and funded the study. Novartis has not decided if or when it will start larger trials, a company spokeswoman said.

"It would be nice to have more than one drug to offer people," said Dr. Maggie Fader, a pediatric hematologist oncologist at Nicklaus Children's Hospital in Miami. "However, there isn't enough information yet to say that this drug is going to be better than what we have," she said.

Also, the study lasted only 12 months, so how well patients respond over time isn't known, Fader said. Larger and longer studies are needed, she added.

Patients who suffer from sickle cell pain crises sometimes try risky treatments or use powerful opioid painkillers, which can result in dependence and the need for increasingly higher doses, Fader said.

"The majority are going to have significant pain crises where they require narcotics or hospitalizations," she added. "Something that's a

disease-modifying agent that might reduce these complications would be welcome."

The study results were scheduled for presentation Sunday at the meeting of the American Society of Hematology, in San Diego. They'll also be published Dec. 3 in the *New England Journal of Medicine*.

More information: Kenneth I. Ataga et al. Crizanlizumab for the Prevention of Pain Crises in Sickle Cell Disease, *New England Journal of Medicine* (2016). DOI: 10.1056/NEJMoa1611770

For more on sickle cell disease, visit the U.S. National Heart, Lung and Blood Institute.