A multiple drug approach to preventing sickle cell crisis

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Sickle cell disease is characterized by recurrent episodes of "sickle crisis," also known as vaso-occlusive crisis, in which a patient's red blood cells change shape, clump together and block the flow of blood in small vessels resulting in pain and organ damage.

In an editorial in the current issue of the *New England Journal of Medicine*, a Boston University School of Medicine (BUSM) researcher stresses the need for a multi-pronged approach to treating sickle-cell disease in order to prevent these harmful episodes.

In a prior issue of the Journal, researchers (Ataga et. al.) reported testing a novel antibody targeting P-selectin, a protein involved in the adhesive interactions between red blood cells and other cells that occur during vaso-occlusive crises. They found that the rate of sickle cell crisis was reduced by 45 percent among patients who received injections of the antibody, called crizanlizumab, compared to patients who received placebo injections.

"Treatment for sickle cell anemia is very limited and we welcome new treatments that could prevent disease complications," explained author Martin Steinberg, MD, professor of medicine at BUSM. "If ultimately approved, this treatment, or something similar to this, could improve life for patients with sickle cell anemia."

Steinberg argues because there is no single proven treatment to eliminate sickle cell crisis, an approach involving multiple medications that target

different processes in the pathogenesis may ultimately be more successful. "Treatment for sickle cell anemia is now limited to a single drug, hydroxyurea, that helps most but not all patients. The trial showed that another drug, which works by a mechanism distinct from that of hydroxyurea, had beneficial effects and could be used alone or with hydroxyurea to prevent severe disease complications."

Steinberg points out that although there was a decrease in vaso-occlusive events, perceived clinical benefit as measured by patient questionnaire was low. Despite this, Steinberg is hopeful that this new therapeutic approach using antiadhesive agents may ultimately become available for patients. "This was a very well done important study with clinically significant results that when confirmed and extended could lead to an important new treatment for sickle cell anemia."

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