New hemophilia treatment a victory for patients, Tulane doctor writes in New England Journal of Medicine

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A new treatment that helps people with hemophilia A maintain higher levels of a crucial blood clotting factor with fewer treatments is a victory for patients, according to a new editorial in The New England Journal of Medicine this week by Cindy Leissinger, MD, director of the Louisiana Center for Bleeding and Clotting Disorders at Tulane University School of Medicine.

An estimated 20,000 people in the U.S. have hemophilia A, including almost 300 in Louisiana. Those with hemophilia A lack clotting factor VIII in their blood, which can lead to painful and sometimes life- and limb-threatening bleeding.

The Food and Drug Administration fast-tracked a potential new therapy, efanesoctocog alfa, in 2021, and researchers published the results of their study in the Journal this week. The study shows the treatment helps patients maintain higher levels of the clotting factor VIII with only one infusion a week. Patients currently need two to three infusions a week with existing therapies that don't prevent all bleeding.

"Efanesoctocog alfa offers much better protection against bleeding with a more convenient dosing schedule for patients," Leissinger said. "Most hemophilia patients administer their own intravenous infusions of clotting factor, so reducing infusions from three times a week to once a week is a big help for patients."

Hemophilia is a rare disease but the burden of treatment for patients and society has been disproportionately high owing to the intense nature of therapy and its cost, Leissinger wrote in the Journal. In a crowded field of transformative therapies that have recently been approved, are under review or in late-stage clinical trials, efanesoctocog alfa stands out as a winner that could soon make life easier for the patients Leissinger sees at Tulane.

Although the Tulane center was not a part of the efanesoctacog alfa trial, Leissinger notes that other new therapies are being studied here, including a gene therapy trial that has the potential of a cure or "near-cure" for some patients with hemophilia.

"Because hemophilia is a rare disease, these kinds of advances are made only because most patients are willing to participate in research and volunteer for trials of new therapies," Leissinger said.

"The future for patients with hemophilia has never looked brighter thanks to cutting edge research and to the patients who are willing to be part of that research."