Researchers trial potential hemophilia treatment

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A global study involving McMaster University researchers has trialed a

potential new treatment that could be a game-changer for people living with hemophilia A, a life-threatening genetic blood disorder.

Co-principal investigator Davide Matino said that once-weekly injections of efanesoctocog alfa can prevent bleeding and promote near-normal activity by the clotting agent factor VIII, crucial for stopping the prolonged bleeding caused by hemophilia A. People who took efanesoctocog alfa also enjoyed improved joint function, better overall health and less pain.

Matino said that efanesoctocog alpha works by separating factor VIII from its associated protein carrier, known as the von Willebrand Factor, thereby overriding its "half-life." This means people with hemophilia A only need once-weekly instead of thrice-weekly injections.

Researchers published their findings in *The New England Journal of Medicine*.

"With this treatment, patients with hemophilia A are free to live the lives that they want as they feel safe, protected and many of them tell me they don't feel the daily joint pain they used to have. They may not be cured, but this treatment really is the next best thing," said Matino, an assistant professor of the Department of Medicine.

"With only one injection for an entire week, people with hemophilia A will enjoy normal or near-normal levels of factor VIII, which is unheard of. Previously, patients had to inject every other day, which was a huge burden for them, yet obtaining a lower level of protection" he said.

"The treatments for hemophilia A have improved more in the last decade than ever before. Now we have gene therapies and editing in the pipeline, monoclonal antibody treatments and cell-based therapies."

The trial, which concluded in 2021, found that 133 hemophilia patients treated weekly for a year improved their outcomes and quality of life compared to their previous treatment.

Matino and fellow McMaster study author Alfonso Iorio said that McMaster was the only center enrolling adult patients in Canada.

"The research infrastructure and leadership we built at McMaster as a leader for the Canadian hemophilia data collection network allowed us to attract international clinical studies on innovative treatments, such as the efanesoctocog alfa trial and gene therapy," said Alfonso Iorio, professor and director of McMaster's hemophilia clinic.

What is hemophilia?

Hemophilia A is a rare bleeding disorder caused by a mutation in the gene encoding factor VIII, effectively stopping clotting activity.

The condition causes repetitive bleeding into joints beginning in early childhood and can be life-threatening. Severe hemophilia A is when a person's factor VIII level is less than one percent of normal.

"The patients we enrolled in this study have been using efanesoctocog alfa for several years and we are delighted that McMaster managed to offer them this treatment and thus the opportunity to enjoy a vastly better quality of life," said Iorio.

More information: Annette von Drygalski et al, Efanesoctocog Alfa Prophylaxis for Patients with Severe Hemophilia A, *New England Journal of Medicine* (2023). DOI: 10.1056/NEJMoa2209226