Disruptive technology for the treatment of hemophilia

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An international team of hematologists including Guy Young, MD, of Children's Hospital Los Angeles, has found that in patients with hemophilia A with inhibitors, a novel therapy called emicizumab, decreases incidence of bleeding episodes by 87%. Results of this multicenter phase III study called HAVEN 1, will be presented at the International Society of Thrombosis and Hemostasis and published in the *New England Journal of Medicine* on July 10.

Individuals with hemophilia A experience excessive bleeding and require prophylactic treatment with intravenously administered clotting factor usually 3 times each week. However, repeated administration of clotting factor can result in the development of antibodies, called inhibitors, that attack and destroy the necessary factor - making treatment of these patients very difficult.

Emicizumab, a novel monoclonal antibody, is given subcutaneously only once per week. According to Alan S. Wayne, MD, director of the Children's Center for Cancer and Blood Diseases, "This is a breakthrough for individuals with hemophilia A who no longer respond to conventional clotting therapies. The standard treatment for patients with hemophilia A and inhibitors has been to use medications called 'by-passing agents'. This new therapy is dramatically more effective at preventing bleeding. Additionally, in comparison to by-passing agents, emicizumab is easier to administer, requires less frequent dosing, and based on this study, appears to have an improved safety profile."

Guy Young, MD, director of the Hemostasis and Thrombosis Program and lead physician for study efforts at Children's Hospital Los Angeles explains further, "While the standard medications allow us to 'bypass' the need for Factor VIII, they don't do the job as efficiently or as well for these patients. Bleeding is harder to stop, and episodes last longer and do more damage to the patients. A patient treated with this new therapy had been in a wheelchair for three years because of joint damage from repeated bleeding incidents. Now, he's walking."

The study enrolled 109 males over the age of 12 with hemophilia A with inhibitors. Individuals treated with emicizumab experienced 87% fewer bleeds compared to untreated patients and 79% fewer bleeds compared to patients treated with bypassing agents. No anti-drug antibodies were detected.

"This is the most significant advancement I have seen during my 20 years working in the field of hemophilia," said Young, who is also a professor of Pediatrics at the Keck School of Medicine of USC and treats the most pediatric patients with hemophilia in California. "We've had families flying in from all over the country to get access to this medication."

Studies investigating the use of emicizumab in hemophilia A patients without inhibitors are underway. This medication is only available to patients as part of a clinical trial but is currently being evaluated by the FDA.

Provided by Children's Hospital Los Angeles