Study first to confirm effectiveness and safety of new treatment for hemophilia

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An international research team led by Dr. Cindy Leissinger of Tulane University School of Medicine, along with Dr. Alessandro Gringeri from the University of Milan, has found that a drug commonly used to treat bleeding events in people with a type of severe hemophilia can also be used to prevent such events from happening in the first place. The study, the first to confirm the efficacy and safety of the drug FEIBA™ in bleed prevention is published in the Nov. 3, 2011 issue of the *New England Journal of Medicine*.

The study examined the ability of FEIBA to prevent bleeds in people with severe hemophilia A with inhibitors. People with this condition produce antibodies known as inhibitors. These inhibitors render ineffective the usual treatment for hemophilia, which involves replacement of the blood clotting factor (Factor VIII) that is absent in hemophilia patients. Approximately 30 percent of patients with severe hemophilia A develop such "inhibitor" antibodies. Hemophilia A patients with inhibitors require treatments with alternate forms of clotting factor concentrates, known as bypassing agents, and until recently primarily had to be infused with clotting factors "on demand" as bleeding episodes occur. Treatment of bleeding events in these patients is not always effective, leading to significant problems for patients, who typically experience repeated joint bleeding and progressive joint disease.

The Tulane-led study tested if preventive treatment with the bypassing agent FEIBA is safe and effective in preventing joint and other bleeds in

hemophilia A patients with inhibitors. Thirty-four patients were enrolled for the 15-month-long study.

The study showed that, compared to on-demand therapy, FEIBA infused three times per week resulted in an overall reduction of 62 percent in all bleeding events and a 72 percent reduction in target joint bleeding ("target joints" are joints that experience repetitive bleeding). Nearly two-thirds of patients showed a very good response to preventive FEIBA treatment, experiencing a reduction in bleeding events of 82 percent.

Provided by Tulane University