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FOR IMMEDIATE RELEASE

Octapharma Announces Two Hemophilia A Clinical Trials to Utilize WILATE®

HOBOKEN, N.J. (November 14, 2016) – Octapharma USA has announced that WILATE®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection will be utilized for two important clinical trials for hemophilia A launching in December.

Octapharma USA will fund an investigator-initiated multi-center clinical trial that aims to investigate one of the major challenges facing hemophilia A treatment – the time it takes to decrease inhibitor levels. Jonathan Ducore, M.D., M.P.H. of the University of California Davis School of Medicine and Courtney Thornburg, M.D., M.S., of the Rady Children's Hospital-San Diego are the principal investigators for the study, entitled "Individualized ITI Based on FVIII Protection by VWF (INITIATE)." The study evaluates a personalized medicine approach to eradicating inhibitors in hemophilia A treatment.

The rationale is that an optimal regimen for immune tolerance induction (ITI) has not been defined, and patients with hemophilia A and inhibitors have high morbidity, mortality, and associated costs. In one arm of the study, patients' plasma will be tested against different WILATE® lots in vitro before treatment to determine the batch that has the highest residual Factor VIII activity left after incubation with the patient's plasma.

"Anti-FVIII inhibitor formation is the most serious complication of hemophilia A treatment today, occurring in up to 20 to 30 percent, or more, of those with severe hemophilia A," said Dr. Ducore. "Use of FVIII is generally futile in this group, because the inhibitor will rapidly inactivate the infused concentrate. ITI can result in inhibitor resolution, but can take several years to achieve success. Preliminary studies have suggested that individualized lot selection (batch-matching) can shorten this time by 67 percent."

This double-blinded, randomized clinical trial will compare the time to inhibitor titer <0.6 BU using individualized lot selection and random lot selection with the plasma-derived von Willebrand factor (VWF)/coagulation factor (FVIII) complex concentrate for ITI in patients with congenital hemophilia A, FVIII activity ≤2%, and a historical high-titer inhibitor [≥5 Bethesda Unit (BU)]. Participants will be randomized on a one-to-one basis between the two study arms, individualized lot selection (alternative treatment arm) and random lot selection (standard treatment arm, current U.S. clinical practice in ITI). Study sites, participants and investigators will be blinded to the treatment status assigned. Octapharma will make the individually selected factor lots available for subjects until the specific lot is depleted.

The time to inhibitor titer <0.6 BU, the study's primary endpoint, is hypothesized to correlate with a reduction in break-through bleeding, morbidity, healthcare costs and improved quality of life. The Centers for Disease Control and Prevention (CDC) recently demonstrated a higher rate of mortality in FVIII inhibitor patients.[1] In multivariate analysis, the odds of death were 70 percent higher among patients with a current inhibitor compared to those without an inhibitor ($p<0.01$), and the deaths among inhibitor patients were primarily attributed to bleeding complications.

Study participants are sought from U.S. hemophilia treatment centers with congenital hemophilia with FVIII activity ≤2% and historical high-inhibitor (≥5 BU) will be eligible. The investigators plan to enroll 120 participants, 60 in each arm of the study, who will meet specific inclusion criteria.

For more information on the INITIATE clinical trial, please contact Dr. Ducore at (916) 734-2781 or jmducore@ucdavis.edu; or Dr. Thornburg at (858) 966-5811 or cthornburg@rchsd.org.

The second study announced by Octapharma will investigate the pharmacokinetics, efficacy, safety, and immunogenicity of WILATE® in previously treated patients with severe hemophilia A. This prospective, international, multi-center Phase 3 study seeks 55 male participants at clinical trial sites in the U.S. and Europe.

The study will start in December. Study participants must have severe hemophilia A and be age 12 or older. For more information, please contact Sylvia Werner, Octapharma USA Director of Clinical Operations, at (201) 604-1149 or sylvia.werner@octapharma.com. The complete study protocol is available at www.ClinicalTrials.gov; ClinicalTrials.gov Identifier: NCT02954575.

About WILATE®

In the U.S., WILATE® is indicated in children and adults with von Willebrand disease for on-demand treatment and control of bleeding episodes; and perioperative management of bleeding. WILATE® is not indicated for the treatment of Hemophilia A in the U.S. Internationally, WILATE® is approved for treatment in 62 countries.

Important Safety Information

WILATE® is contraindicated for patients who have known hypersensitivity reactions, including anaphylactic or severe systemic reaction, to human plasma-derived products, any ingredient in the formulation, or components of the container. Thromboembolic events have been reported in VWD patients receiving coagulation factor replacement therapies. FVIII activity should be monitored to avoid sustained excessive FVIII levels. Development of neutralizing antibodies to FVIII and to VWF, especially in VWD Type 3 patients, may occur. WILATE® is made from human plasma. The risk of infectious agents, including viruses and, theoretically, the Creutzfeldt-Jacob disease agent, cannot be completely eliminated. The most common adverse reactions to treatment with WILATE® in patients with VWD were hypersensitivity reactions, urticaria, and dizziness.

Seroconversions for antibodies to parvovirus B19 not accompanied by clinical signs of disease have been observed. The most serious adverse reactions to treatment with WILATE® in patients with VWD were hypersensitivity reactions. For full prescribing information on WILATE®, please visit www.wilateusa.com.

About the Octapharma Group

Headquartered in Lachen, Switzerland, Octapharma is one of the largest human protein products manufacturers in the world and has been committed to patient care and medical innovation since 1983. Its core business is the development and production of human proteins from human plasma and human cell lines. Octapharma employs approximately 6,000 people worldwide to support the treatment of patients in over 100 countries with products across the following therapeutic areas: Hematology (coagulation disorders), Immunotherapy (immune disorders) and Critical Care. The company's American subsidiary, Octapharma USA, is located in Hoboken, N.J. Octapharma operates two state-of-the-art production sites licensed by the U.S. Food and Drug Administration (FDA), providing a high level of production flexibility. For more information, please visit www.octapharmausa.com.

REFERENCES

1 - Walsh CE, Soucie JM, Miller CH, the United States Hemophilia Treatment Center N. Impact of inhibitors on hemophilia a mortality in the United States. *American Journal of Hematology.* Jan 23 2015.

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