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# Octapharma Launches Phase 3 Superiority Study for Pediatric Acute-onset Neuropsychiatric Syndrome

*Multicenter Clinical Trial to Compare the Effectiveness of PANZYGA® Versus Placebo in Children and Adolescents from age 6 to 17*

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PARAMUS, N.J.--(BUSINESS WIRE)--Octapharma has launched a phase 3, multicenter superiority study to compare the effectiveness of PANZYGA® (immune globulin intravenous, human - ifas) 10% Liquid Preparation versus placebo in patients with pediatric acute-onset neuropsychiatric syndrome (PANS).

PANS is a condition defined by sudden onset of obsessive-compulsive symptoms and/or severe eating restrictions, along with at least two other cognitive, behavioral, or neurological symptoms, according to the National Institutes of Health (NIH).

"PANS is a newly defined symptom-based condition that mainly occurs in children and adolescents," said Octapharma USA President Flemming Nielsen. "Octapharma is proud to sponsor this important research because few studies have described PANS clinical characteristics. We are hopeful that PANZYGA® can make a difference in the lives of children and adolescents impacted by the syndrome."

Researchers aim to enroll 92 patients from age 6 to 17 with a confirmed diagnosis of moderate to severe PANS. Approximately 30 study sites are planned for the prospective, randomized, double-blind, parallel group, placebo-controlled superiority study. The primary objective of the trial is to evaluate if PANZYGA® is superior to placebo (0.9% w/v sodium chloride) for reducing the severity of symptoms associated with PANS in pediatric patients. The secondary objectives of this study are to determine the sustainability of the reduction of the severity of symptoms in pediatric patients treated with PANZYGA®; and to assess the efficacy of PANZYGA® treatment in reducing functional impairment associated with PANS.

"PANS has been a very challenging disease to treat," said Michael Daines, M.D., Division chief of allergy, immunology and rheumatology at the University of Arizona and the study's lead investigator. "It is very hard on families to see their children afflicted with sudden onset, debilitating, and difficult to treat OCD as well as other cognitive and behavioral issues. These changes impact the ability of children to function in school and at home. The search for safe and effective therapies for PANS has been difficult, but there is strong evidence that immunomodulation can mitigate or cure this disease. IVIG has been used in prior studies and in some has shown significant efficacy. This phase 3 trial with PANZYGA® should help define the role of IVIG as an immunomodulatory drug for the management of children with PANS."

The study will include three infusions of PANZYGA® or placebo administered over two days every three weeks for a total of nine weeks, with an additional double-blind, crossover safety and efficacy follow-up phase of three infusions of PANZYGA® or placebo administered over two days every three weeks for a total of nine weeks. The trial will be conducted in a two-

stage adaptive design with one interim analysis. An unblinded interim analysis will be performed by an independent statistician after 40 patients have completed the first nine-week treatment period, to adjust sample size, if required. The entire study team will remain blinded until the end of the study.

"The PACE Foundation is very proud to have been involved with facilitating the launch of this pivotal study in conjunction with Octapharma, our consortium of leading academic research centers including the University of Arizona, Stanford University, UCLA, Harvard University, University of Wisconsin, University of Arkansas and our corporate partner Banner Health," said Paul Ryan of the PACE Foundation. "As a leading national non-profit organization dedicated to improving the diagnosis, treatment and quality of life of persons with Pediatric Autoimmune Neurological Disorders, through advocacy, education and research, the PACE Foundation is committed to building public/private partnerships that lead to meaningful outcomes for patients in need."

For more study information, please contact Huub Kreuwel, Ph.D., Octapharma USA, Vice President of Scientific and Medical Affairs, at usmedicalaffairs@octapharma.com or visit clinicaltrials.gov (Identifier: NCT04508530).

***About PANZYGA®***

PANZYGA® is an immune globulin intravenous, (human) - ifas 10% liquid preparation indicated for the treatment of primary humoral immunodeficiency (PI) in patients two years of age and older; and chronic immune thrombocytopenia (ITP) in adults.

**WARNING: THROMBOSIS, RENAL DYSFUNCTION, and ACUTE RENAL FAILURE**

– Thrombosis may occur with immune globulin intravenous (IGIV) products, including PANZYGA®. Risk factors may include: advanced age, prolonged immobilization, hypercoagulable conditions, history of venous or arterial thrombosis, use of estrogens, indwelling vascular catheters, hyperviscosity, and cardiovascular risk factors.

– Renal dysfunction, acute renal failure, osmotic nephropathy, and death may occur with the administration of IGIV products in predisposed patients. Renal dysfunction and acute renal failure occur more commonly in patients receiving IGIV products containing sucrose. PANZYGA® does not contain sucrose.

– For patients at risk of thrombosis, renal dysfunction, or renal failure, administer PANZYGA® at the minimum infusion rate practicable. Ensure adequate hydration in patients before administration. Monitor for signs and symptoms of thrombosis and assess blood viscosity in patients at risk for hyperviscosity.

Please see full prescribing information for complete boxed warning at https://a.storyblok.com/f/54881/x/75be532561/panzygapi.PDF.

***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 more than 10,000 people worldwide to support the treatment of patients in over 115 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 Paramus, N.J. Octapharma operates three 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 octapharmausa.com.

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