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Benefits of IVIG in Pediatric Acute-Onset Neuropsychiatric Syndrome (2411)

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Objective: To test the hypothesis that pediatric acute-onset neuropsychiatric syndrome (PANS) is related to an autoimmune dysfunction, a multi-site study explored intravenous immunoglobulin (IVIG)[Octagam 5%] for treatment.

Background: PANS is a clinical diagnosis in children who have an acute manifestation of varied neuropsychiatric symptoms, including obsessive compulsive disorder (OCD), eating disorders, tics, anxiety, irritability, and problems with attention/concentration. PANS may develop as a result of a post-infectious syndrome. Our hypothesis is that PANS may represent a new form of post-infectious autoimmunity, through molecular mimicry, suggesting a potential mechanism by which the disorder evolves.

Design/Methods: The primary endpoint was evaluation of IVIG efficacy over a period of 6 months/infusions based on mean changes in psychological evaluation scores using 6 validated assessments including the Children's Yale-Brown Obsessive Compulsive Scale (CY-BOCS). Secondary endpoints included evaluation of presenting immune/autoimmune panels and key potential biomarkers.

Results: The final cohort consisted of 21 subjects (7 per site) with moderate to severe PANS. The mean age was 10.86 years (range: 4–16 years). Results demonstrated statistically significant reductions in symptoms from baseline to end of treatment (infusion 6) in all 6 assessments measured. CY-BOCS results demonstrated statistically significant reductions in obsessive compulsive symptoms, resulting in > 50% improvement sustained for at least 8 weeks after the final infusion and up to 46 weeks in a subset of subjects.

Conclusions: In PANS, which may be associated with an underlying immune dysregulation, IVIG [Octagam 5%] successfully ameliorated psychological symptoms and dysfunction with sustained benefits for at least 8 weeks, and up to 46 weeks, following the final infusion. In addition, baseline immune and autoimmune profiles demonstrated

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significant elevations in a majority of subjects, which requires further evaluation, characterization, and study to clarify the potential immune dysfunction by which PANS manifests and progresses.

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