New clinical trial to examine medication to treat social withdrawal in Fragile X and autism

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Children and adults with social withdrawal due to Fragile X syndrome, the most common cause of inherited intellectual disability and the most common known single gene cause of autism, may benefit from an experimental drug under study by pediatric neurologists at Rush Children's Hospital at Rush University Medical Center.

Rush is the only site in Illinois and one of 21 hospitals in the U.S. participating in the trial for Fragile X.

Fragile X syndrome is a neurodevelopmental disorder characterized by impaired social function, cognition and speech, as well as attention deficits and anxiety.

People with Fragile X, autism or autism spectrum disorders often display social impairment including social withdrawal and anxiety and have difficulty communicating and interacting with others. Although there are behavioral and psychological interventions, there are no approved medications for the treatment of social or communication difficulties in Fragile X, autism and autism spectrum disorders.

"The condition can be severely debilitating and this medication has the potential to play a much needed role in improving the core symptoms of fragile X syndrome and helping patients and their families achieve an improved quality of life," said Dr. Elizabeth Berry-Kravis, pediatric

neurologist at Rush and principal investigator of the study.

The study is sponsored by Seaside Therapeutics, Inc, and will test the efficacy, safety and tolerability of the drug called STX209 (arbaclofen).

Racemic baclofen (mixture of arbaclofen and esbaclofen) is approved by the FDA to treat spasticity and stiff muscles due to cerebral palsy or other forms of brain or spinal cord injury, but arbaclofen, the more active form of baclofen, is not FDA approved.

"There is some evidence that the medication may help with social behaviors in people with developmental disabilities," said Berry-Kravis, who is a professor of pediatrics, neurology and biochemistry at Rush University.

Participants in the randomized, double-blind, placebo controlled phase III trial will be randomized to receive either the study drug, STX209, or a placebo. The clinical trial will include screening, treatment, withdrawal of medication, and a follow-up period. Subjects who complete the study may be eligible to enroll in a subsequent open-label study in which all subjects are treated with STX209.

STX209 has been studied in a previous small placebo-controlled trial in children and adults with fragile X syndrome and showed evidence of benefit for social withdrawal.

"Previous research has found that from one-quarter to one-half of people with fragile X have autism spectrum disorders," said Berry-Kravis.

"This trial is exciting, because it represents the culmination of 20 years work in fragile X research since discovery of the fragile X gene in 1991," said Kravis. "We're not expecting this to cure fragile X or autism, but it's a very important step in the development of new treatments."

Provided by Rush University Medical Center