



Investigational Drug Study for Adolescents and Adults with Fragile X Syndrome

Kennedy Krieger Institute is looking for adolescents and adults, ages 12 through 25 years, with fragile X syndrome to participate in a research study.

The purpose of this study is to determine if an investigational drug, arbaclofen, is an effective treatment for social withdrawal symptoms associated with fragile X syndrome. This study will also assess whether arbaclofen is safe and tolerated by individuals with fragile X syndrome.

Social Withdrawal behaviors may include:

- Doing nothing but sitting and watching others
- Isolating himself/herself from other children or adults
- Being difficult to reach, contact, or get through to
- Showing fixed facial expressions or lack of emotions

Eligible participants will receive up to 2 months of treatment with the study drug, arbaclofen, or a placebo (no active study drug). Participants have a 50/50 chance (like the flip of a coin) of receiving the study drug. Neither the study team nor the participants will know if the participant is receiving the study drug or the placebo.

During the treatment period, participants will meet with the study doctor every 2 to 4 weeks and will be monitored for side effects. At the end of the treatment period, the investigational drug will be gradually withdrawn over a 4-week period. Visits will consist of physical and medical exams (including blood draws and ECG testing) and behavioral assessments. For females, there will be a urine pregnancy testing. The risks of this study include side effects associated with arbaclofen such as drowsiness, dizziness, weakness and fatigue (tiredness). Some participants may also find medical procedures such as blood draws, electrocardiogram (ECG), urine testing and physical examination upsetting or uncomfortable.

Participation in this study will require up to 6 visits (each lasting 2-4 hours) to the Kennedy Krieger Institute in Baltimore, MD and up to 14 between-visit phone calls with the study doctor over an 18-week period. For each completed visit, participants will receive \$50 compensation for their time and travel expenses. The investigational drug and all study-related evaluations are provided at no cost to the participants.

If you are interested in learning more about this study, please contact:

Study Coordinator 443-923-7619 or email: ResearchTrials@kennedykrieger.org
Walter Kaufmann MD, Principal Investigator, NA_00047949 www.clinicaltrials.gov (#NCT01282268)

Project sponsors:

This study is sponsored by Seaside Therapeutics (209FX301) and is being performed at up to 25 other sites in the U.S.



Approved November 15, 2011