Early work indicates drug used to treat alcoholism may help those with Fragile X and autism

April 4 2011

In small, early clinical trials, adults and children with autism and Fragile X syndrome have shown improved communication and social behavior when treated with acamprosate, according to Craig Erickson, M.D., assistant professor of psychiatry at the Indiana University School of Medicine and chief of the Riley Hospital for Children Christian Sarkine Autism Treatment Center at Indiana University Health.

Acamprosate, which affects chemicals in the brain by blocking certain receptors associated with mental health, has approval from the Food and Drug Administration for the treatment of alcoholism in adults.

Dr. Erickson is the inventor on a pending utility patent for the use of acamprosate as a therapeutic agent for Fragile X syndrome, the most common inherited form of intellectual disability and the most frequent single gene cause of autism.

"We have been treating small numbers of both adults and children," said Erickson. "We have observed improvements in eye contact, social interaction and speech. This is very early work, but it appears promising.

"We have a lot to do. We need to determine appropriate doses and forms for the best drug delivery. Larger studies will be needed to determine effectiveness and tolerability. And we expect to find many interesting things along the road, for example whether this drug could work better in

those with Fragile X who have autism than in those whose autism is from an unknown cause."

In November 2010 Dr. Erickson and colleagues reported in the Journal of Autism and Developmental Disorders on the first trial of acamprosate in adults with Fragile X syndrome and autism. In the three patients studied, acamprosate was associated with improved linguistic abilities. During five months of treatment all three showed unexpected marked communication improvement.

In a study supported by the Indiana University Clinical and Translational Sciences Institute, Dr. Erickson is currently recruiting five-to-17-year-old children with Fragile X syndrome to a clinical trial designed to learn if acamprosate reduces various symptoms including inattention, hyperactivity, language impairment, irritability, and social deficits. In addition to investigating the effectiveness and tolerability of acamprosate in youth with Fragile X syndrome, IU School of Medicine researchers are assessing neurobiological differences between Fragile X syndrome and autism spectrum disorders of unknown cause.

Fragile X syndrome is the most common inherited form of developmental disability. Fragile X syndrome is inherited from a carrier parent, most frequently the mother. Up to two thirds of individuals with Fragile X syndrome display evidence of autism spectrum disorders.

"Dr. Erickson's research and work in this area is unique. The Indiana University Research and Technology Corporation has applied for a use patent because, while we are far from definitive knowledge and treatment, as early data is acquired, we believe this drug has real potential as a therapy for both children and adults with autism," said Bradley Fravel, Ph.D., MBA, an IURTC senior technology manager. IURTC works to enhance the research and development capability of Indiana University, creating new Indiana-based companies, and provides

support for entrepreneurial development.

Dr. Erickson strongly cautions that while preliminary data on acamprosate in individuals with Fragile X syndrome appears promising, large scale multi-center clinical trials of acamprosate are needed to determine whether it can help those with Fragile X and autism.

Provided by Indiana University School of Medicine