



GEN News Highlights

Apr 13, 2011

UC-Davis Gets \$3M DoD Grant to Study Marinus' Ganaxolone in Fragile X Syndrome

Three University of California, Davis (UC-Davis) researchers won a \$3 million grant from the U.S. Department of Defense to study ganaxolone, Marinus Pharmaceuticals' lead candidate, which is being studied as a treatment for fragile X syndrome (FXS).

Ganaxolone is a synthetic neurosteroid and a derivative of the naturally occurring neuromodulator allopregnanolone. It modulates GABA-A ion channels by selective binding to the neurosteroid receptor. Early research suggests that normalizing neurosteroid levels with ganaxolone treatment may eliminate the behavioral symptoms associated with FXS, the leading cause of inherited mental disability and the most common single-gene cause of autism, according to the scientists.

The three researchers who won the grant were Randi Hagerman, M.D., medical director of UC-Davis' MIND Institute and a researcher specializing in neurodevelopmental disorders and fragile X-related disorders; psychophysicologist David Hessler, Ph.D.; and epilepsy researcher Michael Rogawski, M.D., Ph.D.

"Over the next few months, we will work with UC-Davis to provide ganaxolone for the FXS study and work on obtaining regulatory approval from the Food and Drug Administration to allow the researchers to conduct this study under Marinus' investigational new drug application," says Kenneth Shaw, Ph.D., svp, R&D at Marinus.

Ganaxolone has been administered to more than 950 volunteers in Phase I and Phase II studies. Completed mid-stage epilepsy trials have generated data supportive of the efficacy and safety of ganaxolone in the treatment of both children and adults suffering from refractory epilepsy, namely patients who continue to have seizures despite taking multiple anticonvulsant drugs, according to according to Marinus.

The firm hopes to bring ganaxolone to market for the treatment of not only FXS and epilepsy but posttraumatic stress disorder (PTSD) as well. A clinical trial to investigate the safety and efficacy of ganaxolone in PTSD patients is under way. That study is being conducted by the INTRuST (injury and traumatic stress) consortium and is funded by the defense department, with results expected in 2012.