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Ganaxolone Reduces Seizures and Improves Behaviors in Angelman Syndrome Preclinical Model

Data Published in Neuropharmacology

RADNOR, Pa., Feb. 14, 2017 (GLOBE NEWSWIRE) -- Marinus Pharmaceuticals, Inc. (Nasdaq:MRNS), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, today announced the publication of preclinical data in the January issue of *Neuropharmacology*, showing that its positive allosteric modulator of $GABA_A$, ganaxolone ameliorates many of the behavioral abnormalities in a mouse model of adult Angelman syndrome (AS). Evidence from experimental models suggests that AS may be associated with deficient extrasynaptic $GABA_A$ receptor function. AS is a rare neurogenetic disorder characterized by severe developmental delay, motor impairments and epilepsy.

The published article, co-authored by, among others, Michael A. Rogawski, M.D., Ph.D, professor of neurology and pharmacology at the School of Medicine, University of California, Davis, and titled "Effects of the synthetic neurosteroid ganaxolone on seizure activity and behavioral deficits in an Angelman syndrome mouse model," provided the outcomes of a study in an AS mouse model which evaluated the potential therapeutic benefit that could be achieved by restoring extrasynaptic $GABA_A$ receptor function. The results showed that ganaxolone-treated mice exhibited improved anxiety measures, positive motor benefits, and reduced seizures with both short- and long-term treatment. Importantly, there was no tolerance to the antiseizure efficacy of ganaxolone or to its other therapeutic actions.

As stated in the published article, "Our present results indicate that ganaxolone might be particularly well suited as a symptomatic treatment for AS, with the potential to not only treat the seizures but also to provide long-lasting improvement in the diverse neurobehavioral and motor symptoms."[1]

Dr. Jaakko Lappalainen, vice president of clinical development at Marinus Pharmaceuticals commented, "I am impressed with these data showing that ganaxolone was anxiolytic, anticonvulsant, and improved motor deficits in a mouse model of AS. One of the most notable effects of ganaxolone is the reversal of the motor and learning deficits in the AS mice, which if translatable to humans, could significantly improve the quality of life in AS patients. These data support the rationale to investigate ganaxolone in the future as a symptomatic treatment for AS."

[1] Ciarlone, S.L., Wang, X., Rogawski, M.A., Weeber, E.J., Effects of the synthetic neurosteroid ganaxolone on seizure activity and behavioral deficits in an Angelman syndrome mouse model, *Neuropharmacology* (2017), doi: 10.1016/j.neuropharm.2016.12.009.

About Ganaxolone

Ganaxolone, a positive allosteric modulator of $GABA_A$, is being developed in three different dose forms (intravenous, capsule, and liquid) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Ganaxolone exhibits antiseizure and antianxiety actions via its effects on synaptic and extrasynaptic $GABA_A$ receptors. Ganaxolone has been studied in more than 1,400 subjects, both pediatric and adult, at therapeutically relevant dose levels and treatment regimens for up to two years. In these studies, ganaxolone was generally safe and well tolerated. The most commonly reported adverse events were somnolence, dizziness and fatigue.

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a biopharmaceutical company dedicated to the development of ganaxolone, which offers a new mechanism of action, demonstrated efficacy and safety, and convenient dosing to improve the lives of patients suffering from epilepsy and neuropsychiatric disorders. Ganaxolone is a positive allosteric modulator of $GABA_A$ that acts on a well-characterized target in the brain known to have both antiseizure and antianxiety effects. Ganaxolone is being developed in three different dose forms (IV, capsule and liquid) intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus is currently evaluating ganaxolone in orphan

pediatric indications for the treatment of genetic seizure and behavior disorders, and preparing to initiate Phase 2 studies in status epilepticus, an orphan indication, and postpartum depression. For more information visit www.marinuspharma.com.

Forward-Looking Statements

To the extent that statements contained in this press release are not descriptions of historical facts regarding Marinus, they are forward-looking statements reflecting the current beliefs and expectations of management made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Words such as "may", "will", "expect", "anticipate", "estimate", "intend", "believe", and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. Examples of forward-looking statements contained in this press release include, among others, statements regarding our interpretation of preclinical studies, development plans for our product candidate, including the development of dose forms, the clinical trial testing schedule and milestones, the ability to complete enrollment in our clinical trials, interpretation of scientific basis for ganaxolone use, timing for availability and release of data, the safety, potential efficacy and therapeutic potential of our product candidate and our expectation regarding the sufficiency of our working capital. Forward-looking statements in this release involve substantial risks and uncertainties that could cause our clinical development programs, future results, performance or achievements to differ significantly from those expressed or implied by the forward-looking statements. Such risks and uncertainties include, among others, the uncertainties inherent in the conduct of future clinical trials, the timing of the clinical trials, enrollment in clinical trials, availability of data from ongoing clinical trials, expectations for regulatory approvals, and other matters, including the development of formulations of ganaxolone, that could affect the availability or commercial potential of our drug candidates. Marinus undertakes no obligation to update or revise any forward-looking statements. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to the business of the Company in general, see filings Marinus has made with the Securities and Exchange Commission.

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