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Ganaxolone Phase 1 Data Supports Progressing to Phase 2 in Patients with Status Epilepticus

Data Presented at the 141st Annual Meeting of the American Neurological Association

RADNOR, Pa., Oct. 19, 2016 (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 that in its Phase 1 dose-escalation study, ganaxolone intravenous (IV) achieved dose levels targeted for efficacy in patients with status epilepticus (SE) and other indications. Status epilepticus is a life-threatening medical emergency associated with high mortality and limited treatments. Typically, single or combination IV antiepileptic drugs are used in an attempt to break the seizures, however there are approximately 45,000 patients in the U.S. who do not respond to first-line treatment.

Albena Patroneva, M.D., chief medical officer at Marinus, commented, "We are pleased with the results from our Phase 1 study which has provided predictable PK to enable dosing of ganaxolone IV in clinical studies in SE. There is a significant need for therapies that can rapidly stop the seizures in patients with SE. We believe ganaxolone IV could be a promising therapeutic option in this difficult-to-treat seizure disorder and look forward to advancing our clinical studies into patients with SE."

The Phase 1 clinical study enrolled 36 subjects at Duke University Medical Center and was designed to determine the pharmacokinetics (PK), pharmacodynamics (PD), and safety of ganaxolone IV administered as an ascending bolus dose (Stage 1) or continuous infusion (Stage 2). Four cohorts of subjects were enrolled in Stage 1 and received escalating doses of ganaxolone, and one cohort of subjects was enrolled in Stage 2. The primary study objective was to evaluate the safety and PK of ganaxolone IV. The secondary study objectives included the PD effects of ganaxolone IV on electroencephalogram (EEG) parameters, as well as the effect on clinical sedation scores.

Every dose regimen of ganaxolone IV administered, either bolus or continuous infusion, was generally safe and well tolerated and reached targeted dose levels in a short period of time. Following treatment, six treatment-emergent adverse events were reported, all of which were mild in severity and resolved without intervention. Only headache was considered possibly related to treatment with ganaxolone IV. No subject discontinued due to an adverse event and no serious adverse events were reported. Ganaxolone IV plasma concentrations were generally proportional to the administered dose, with potential anti-convulsant plasma concentrations achievable with a bolus dose. Additionally, the continuous infusion of ganaxolone IV achieved the targeted PK levels.

The data was presented by Dr. Julia Tsai, senior director of clinical development and project management at Marinus, in a poster entitled, "Phase 1 study to determine the pharmacokinetics, pharmacodynamics, and safety of IV ganaxolone in healthy adults," at the 141st Annual Meeting of the American Neurological Association (ANA), October 16-18, 2016.

Marinus is making preparations to commence its Phase 2 clinical study in patients with SE in 2017. Earlier this year, the U.S. Food and Drug Administration granted Orphan Drug Designation to ganaxolone IV for the treatment of SE.

About Ganaxolone

Ganaxolone is a CNS-selective $GABA_A$ modulator 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. Ganaxolone acts on a well-characterized synaptic and extrasynaptic $GABA_A$ target known for anti-seizure and anti-anxiety activity. Ganaxolone has been studied in more than 1,300 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, with the most commonly reported adverse events of 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 CNS-selective $GABA_A$ modulator that acts on a well-characterized target in the brain known to have both anti-seizure and anti-anxiety 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. Ganaxolone IV is advancing into Phase 2 studies to treat status epilepticus. Ganaxolone IV is complemented by its oral dose forms, providing the potential for IV-to-oral continuation therapy for patients transitioning from acute care to outpatient settings. Ganaxolone capsule and liquid is being studied in orphan pediatric indications with comorbidities in seizures and behavior disorders — PCDH19, CDKL5, Lennox-Gastaut Syndrome and Fragile X Syndrome. 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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