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## Ganaxolone IV Clinical Data Presented at Leading International Status Epilepticus Medical Conference

RADNOR, Pa., April 10, 2017 (GLOBE NEWSWIRE) -- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq:MRNS), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, presented Phase 1 clinical data showing the safety and tolerability of ganaxolone IV, the Company's intravenous formulation of its positive allosteric modulator of GABA<sub>A</sub>, at the 6<sup>th</sup> London-Innsbruck Colloquium on Status Epilepticus and Acute Seizures in Salzburg, Austria, April 6-8, 2017. Status epilepticus (SE) is a life-threatening medical emergency associated with high mortality and limited treatments. The biennial meeting focuses on highlighting cutting edge research and clinical practice in the field of SE with the aim to lead to improvements in the treatment and outcome of this condition.

The poster, titled "Phase 1 study to determine the pharmacokinetics, pharmacodynamics, and safety of IV ganaxolone in healthy adults," was selected as one of twenty Best Posters by the Colloquium and presented by Dr. Julia Tsai, Executive Director of Clinical Development at Marinus. The poster highlighted a study evaluating the pharmacokinetics (PK), pharmacodynamics (PD), and safety of ganaxolone IV administered as an ascending bolus dose or continuous infusion in 36 subjects. The results showed that every dose regimen of ganaxolone IV administered was generally safe and well-tolerated, and reached targeted dose levels in a short period of time. Ganaxolone concentrations in plasma were generally proportional to the administered dose, with potential anti-convulsant plasma concentrations achievable with a bolus dose. Bispectral Index (BIS) and qualitative EEG changes showed a dose-response relationship consistent with expected GABA<sub>A</sub> mechanism of action. Additionally, the observed predictable PK will guide dosing of ganaxolone IV in future studies.

"We are honored to have our ganaxolone IV data selected as a Best Poster for presentation at this premier medical conference," commented Jaakko Lappalainen, vice president of clinical development of Marinus Pharmaceuticals. "Best Posters are being offered for publication as a supplement in *Epilepsia*, the world's leading journal of original scientific research and commentary in epileptology. We look forward to partnering with the Colloquium and the medical community in their goal to improve outcomes and treatment options for patients with SE."

SE is a life-threatening occurrence of continuous or intermittent seizures lasting more than five minutes without full recovery to baseline. If SE is not treated immediately, permanent neuronal damage may occur, which contributes to high rates of morbidity and mortality. A patient is considered to have refractory status epilepticus (RSE) if first and second line therapies have both failed to control SE. According to LexisNexis, there are approximately 45,000 cases of hospitalized RSE treated in the United States annually. RSE patients who continue to be in status are generally placed under IV anesthesia as a last attempt to stop the seizures and prevent further damage to the brain and/or death. Those patients whose SE continue for ≥24 hours after the onset of anesthetic therapy or recur upon the reduction or withdrawal of anesthetics are considered super-refractory status epilepticus (SRSE). Few therapies are approved for the treatment of SE and the urgent need for more efficacious therapies remains.

Dr. Lappalainen also said, "There is a significant need for therapies that can rapidly stop the seizures in patients with status epilepticus. 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 status epilepticus later this year."

### About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABA<sub>A</sub>, 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<sub>A</sub> receptors. Ganaxolone has been studied in more than 1,500 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<sub>A</sub> 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](http://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 clinical and preclinical studies, assessment of positive nature and notability of preliminary data, 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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