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Marinus Pharmaceuticals Receives FDA Orphan Drug Designation for Ganaxolone IV to Treat Status Epilepticus

April 15, 2016

RADNOR, Pa., April 15, 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 the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to the intravenous (IV) formulation of its CNS-selective GABA_A modulator, ganaxolone, for the treatment of status epilepticus. A Phase 1 clinical trial evaluating the safety, tolerability and pharmacokinetics of ganaxolone IV is expected to initiate in the first half of 2016.

"Status epilepticus is a life-threatening condition, associated with high mortality and if not treated immediately, can result in brain damage, cognitive impairment and death," remarked Christopher M. Cashman, Chief Executive Officer of Marinus Pharmaceuticals. "We are pleased to receive the Orphan Drug Designation for ganaxolone IV in this difficult to treat seizure disorder with limited treatment options."

Orphan Drug Designation is granted by the FDA Office of Orphan Products Development (OOPD) to novel drugs or biologics that treat a rare disease or condition affecting fewer than 200,000 patients in the U.S. The designation provides the drug developer with a seven-year period of U.S. marketing exclusivity, as well as tax credits for clinical research costs, the ability to apply for annual grant funding, clinical research trial design assistance and waiver of Prescription Drug User Fee Act (PDUFA) filing fees.

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 planned to enter clinical trials in 2016 and is being developed 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 is being evaluated in a Phase 3 multi-national clinical trial as adjunctive treatment of focal onset seizures in adults. Ganaxolone capsule and liquid are being studied in orphan pediatric indications with comorbidities in seizures and behavior disorders — PCDH19 epilepsy 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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