January 3, 2017

# Marinus Pharmaceuticals Receives FDA Orphan Drug Designation for Ganaxolone to Treat Fragile X Syndrome

RADNOR, Pa., Jan. 03, 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 that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to its CNS-selective $GABA_A$ modulator, ganaxolone, for the treatment of Fragile X Syndrome (FXS).

"We are pleased to receive Orphan Drug Designation from the FDA for ganaxolone in Fragile X Syndrome," stated Christopher M. Cashman, chief executive officer of Marinus Pharmaceuticals. "This designation underscores the significant unmet medical need for children suffering from a genetic mutation that causes autism-like symptoms including anxiety, mood swings and attention deficit."

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 Fragile X Syndrome**

Fragile X syndrome (FXS) is the most common genetic cause of autism and caused by a mutation in the FMR1 gene. FXS is characterized by a range of developmental problems and symptoms, including cognitive impairment, learning disabilities and behavioral challenges. Approximately one million individuals in the United States have, or are at risk for developing a Fragile X associated disorder, with approximately 100,000 people having FXS. According to the Centers for Disease Control and Prevention, FXS affects 1 in 3,600 to 4,000 males and 1 in 4,000 to 6,000 females of all races and ethnic groups. Patients with FXS exhibit autism-like symptoms including anxiety and mood swings, attention deficit and heightened stimuli. Approximately 7% of women and 18% of men with FXS have seizures. Currently, there are no known cures or approved therapies for FXS.

**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 1,400 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, 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. 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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