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## **FDA Grants Orphan Drug Designation for Ganaxolone in CDKL5 Disorder**

RADNOR, Pa., June 29, 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 ganaxolone for the treatment of CDKL5 Disorder. Ganaxolone is currently being evaluated in children with CDKL5 Disorder in a Phase 2 clinical trial.

"CDKL5 Disorder is a severe, rare genetic disorder that affects children at an early age and causes difficult-to-control seizures and neuro-developmental impairment," remarked Christopher M. Cashman, Chief Executive Officer of Marinus Pharmaceuticals. "There are no approved therapies for children with CDKL5 Disorder, and a great need for new treatment options that can control both the seizures and co-morbidities of the disease to improve the quality of life for the child and their family. We are pleased to receive Orphan Drug Designation for ganaxolone in CDKL5 Disorder and look forward to presenting the data from our ongoing Phase 2 trial in the upcoming months."

Orphan Drug Designation is granted by the FDA Office of Orphan Products Development 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 CDKL5 Disorder**

CDKL5 Disorder is a serious and rare genetic disorder that is caused by a mutation of the cyclin-dependent kinase-like 5 (CDKL5) gene, located on the X chromosome. It predominantly affects girls and is characterized by early-onset, difficult-to-control seizures and severe neuro-developmental impairment. The CDKL5 gene encodes a protein essential for normal brain function. Most children affected by CDKL5 cannot walk, talk, or care for themselves. Many also suffer from scoliosis, visual impairment, gastrointestinal difficulties, and sleeping disorders. Currently, there are no approved therapies for CDKL5 Disorder.

### **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. Unlike benzodiazepines, ganaxolone exhibits anti-seizure and anti-anxiety activity 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 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 women with PPD and 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. For more information visit [www.marinuspharma.com](http://www.marinuspharma.com). Please follow us on Twitter: @MarinusPharma.

*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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