NEWS RELEASE

Marinus Pharmaceuticals Receives Rare Pediatric Disease Designation from FDA for Ganaxolone for the Treatment of CDKL5 Deficiency Disorder (CDD)

7/30/2020

RADNOR, Pa.--(BUSINESS WIRE)--July 30, 2020 -- **Marinus Pharmaceuticals, Inc.** (Nasdaq: MRNS), a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare seizure disorders, announced today that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) Designation for the company's product candidate ganaxolone for the treatment of **CDKL5 deficiency disorder (CDD)**, a rare refractory form of pediatric epilepsy.

"We are pleased that ganaxolone has received Rare Pediatric Disease Designation from the FDA and are enthusiastic that the timing of this news aligns with our continued expectation to report top line data this quarter from our pivotal Phase 3 Marigold Study evaluating oral ganaxolone in patients with CDD," said Scott Braunstein, M.D., Chief Executive Officer at Marinus. "This designation for ganaxolone for CDD underscores the significant unmet medical need for children and young adults with this serious and rare genetic disease, which causes early-onset, difficult-to-control seizures and severe neuro-developmental impairment. Our goal is to advance a pipeline of programs that can treat rare seizure disorders, including CDD, tuberous sclerosis complex and PCDH19-related epilepsy, and bring much-needed medicines to patients as soon as possible."

FDA grants RPD Designation for diseases that affect fewer than 200,000 people in the U.S. in which the serious or life-threatening manifestations are primarily in individuals 18 years of age and younger. If a new drug application (NDA) for ganaxolone in CDD is approved, Marinus may be eligible to receive a priority review voucher from the FDA, which can be redeemed for priority review in a subsequent marketing application. The program is intended to encourage development of new drugs and biologics for the prevention and treatment of rare pediatric diseases.

“The data that were used to support the Rare Pediatric Disease Designation comprised an analysis of existing literature as well as unpublished data specific to the CDD population, documenting that the serious or life-threatening manifestations of CDD primarily affect children,” said Joseph Hulihan, M.D., Chief Medical Officer at Marinus. "We plan to publish these new data that will describe important clinical and health economic outcomes related to how seizure burden in children with CDD may affect future developmental milestones."

The current program for RPD Designation will expire after September 30, 2020 unless Congress renews the program; however, a drug designated for a rare pediatric disease on that date can still receive a voucher if the drug is submitted and approved by the September 30, 2022 deadline.

In advance of topline data from that study, Marinus has begun preparations for an Expanded Access Program (EAP) in CDD that will allow the company, on positive data, to offer ganaxolone to patients who were unable to participate in the Phase 3 study. The global, double blind, placebo-controlled Phase 3 Marigold Study evaluating the use of oral ganaxolone in children and young adults has enrolled 101 patients between the ages of 2 and 21 with a confirmed disease related CDKL5 gene variant.

About CDKL5 Deficiency Disorder

CDD 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 females and is characterized by early-onset, difficult-to-control seizures and severe neuro-developmental impairment. The CDKL5 gene encodes proteins essential for normal brain function. Most children affected by CDD cannot walk normally, talk, or care for themselves. Many also suffer from scoliosis, visual impairment, gastrointestinal difficulties and sleep disorders. There are no treatments approved specifically for CDD. Genetic testing is available to determine if a patient has a mutation in the CDKL5 gene. To our knowledge, no previous late-stage clinical trials have been conducted in this patient population.

About Ganaxolone

Ganaxolone, a positive allosteric modulator of GABAA receptors, is being developed in intravenous and oral formulations 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 GABAA receptors. Ganaxolone has been studied in more than 1,600 subjects, both pediatric and adult, at therapeutically relevant dose levels and treatment regimens for up to four years.

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc. is a pharmaceutical company dedicated to the development of innovative therapeutics to treat rare seizure disorders. Ganaxolone is a positive allosteric modulator of GABAA receptors that acts on a well-characterized target in the brain known to have anti-seizure, anti-depressant and anti-anxiety effects. Ganaxolone is being developed in IV and oral dose forms intended to maximize therapeutic reach to adult and pediatric patient populations in both acute and chronic care settings. Marinus has conducted the first ever Phase 3 pivotal trial in children with CDKL5 deficiency disorder and is conducting a Phase 2 trial in Tuberous Sclerosis Complex, as well as a Phase 2 biomarker driven proof of concept trial in PCDH19-related epilepsy. The company intends to initiate a Phase 3 trial in status epilepticus. 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 clinical development plans for ganaxolone; our expectations to release top line data from the pivotal Phase 3 Marigold Study in the third quarter of 2020; our expectations to initiate a Phase 3 trial in status epilepticus; the potential safety and efficacy of ganaxolone; expectations regarding our ability to receive and utilize a priority review voucher; the therapeutic potential of ganaxolone; and our plans for an expanded access program for ganaxolone. Forward-looking statements in this press 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, uncertainties and delays relating to the design, enrollment, completion, and results of clinical trials; unanticipated costs and expenses; early clinical trials may not be indicative of the results in later clinical trials; clinical trial results may not support regulatory approval or further development in a specified indication or at all; actions or advice of the U.S. Food and Drug Administration may affect the design, initiation, timing, continuation and/or progress of clinical trials or result in the need for additional clinical trials; our ability to obtain and maintain regulatory approval for our product candidate; our ability to obtain and maintain patent protection for our product candidates; delays, interruptions or failures in the manufacture and supply of our product candidate; our ability to raise additional capital; the effect of the COVID-19 pandemic on our business, the medical community and the global economy; and the availability or potential availability of alternative products or treatments for conditions targeted by us that could affect the availability or commercial potential of our product candidate. 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.

CONTACT:

Sasha Damouni Ellis
Vice President, Investor Relations & Corporate Communications
Marinus Pharmaceuticals, Inc.
484-253-6792
sdamouni@marinuspharma.com