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Marinus Announces Clinical Development Plans for Ganaxolone Intravenous Formulation

Plans to initiate clinical trial in Status Epilepticus

Preclinical Models Demonstrate Robust Efficacy in Benzodiazepine-Resistant Status Epilepticus

RADNOR, Pa., Oct. 29, 2015 (GLOBE NEWSWIRE) -- Marinus Pharmaceuticals, Inc. (Nasdaq:MRNS), a biopharmaceutical company dedicated to the development of innovative therapeutics to treat epilepsy and neuropsychiatric disorders, announced today the initiation of the clinical phase of its intravenous (IV) ganaxolone program in Status Epilepticus (SE). Data from preclinical studies yielded positive results testing ganaxolone IV in benzodiazepine-resistant SE, which support progressing ganaxolone IV to human clinical trials. Ganaxolone IV promoted survival and showed better or comparable reversal of seizures than the endogenous neurosteroid, allopregnanolone, in clinically translatable rodent models of SE. The studies were conducted at two separate laboratories using different measurements.

Dr. Albena Patroneva, Chief Medical Officer of Marinus Pharmaceuticals, commented, "These preclinical results provide strong evidence for the efficacy of ganaxolone and support our plans to move ganaxolone IV into the clinic. We anticipate commencing a Phase 1 clinical trial to evaluate the safety, tolerability and pharmacokinetics of ganaxolone IV before initiating a clinical trial in SE patients later in 2016. There is a significant unmet medical need for an approved treatment for SE with available oral continuation therapy for patients transitioning from hospital to outpatient care."

The following results were observed in a preclinical study conducted to evaluate the therapeutic potential of ganaxolone compared to allopregnanolone in a benzodiazepine-resistant model of SE:

- Ganaxolone IV (12 or 15 mg/kg) administered in rats at 15 minutes after SE onset, showed a longer response than allopregnanolone (15 mg/kg), five hours vs. one hour, respectively.
- Ganaxolone IV administered at 60 minutes after SE onset, which represents the diazepam-resistant population and models refractory SE, produced an immediate reduction in seizure, lasting up to five hours while allopregnanolone produced only a partial reduction in seizure activity, lasting less than 30 minutes.

The following results were observed in a preclinical study conducted to evaluate the dose- and time-dependent therapeutic potential of ganaxolone for the treatment of SE, as well as to measure behavioral seizures and survival:

- Ganaxolone IV halted SE and produced a dose-dependent reduction in seizures associated with SE when administered at 3 different doses over four separate time points after the first observed convulsive seizure. Significant activity was observed at a dose of 6 mg/kg and maximal protection at dose levels between 9 and 12 mg/kg.
- Ganaxolone IV promoted survival at rates similar to those observed with administration of allopregnanolone as measured at 24 hours after SE onset in SE induced rats.

Ganaxolone IV was also tested in three Good Laboratory Practice (GLP) preclinical toxicity studies to assess the tolerability, hemolytic potential and human plasma compatibility. In these studies, ganaxolone IV did not cause hemolysis and was compatible with human plasma. In addition, ganaxolone was well tolerated when given up to 14 days intravenously to rats and as a single dose intravenously or peri-venously to rabbits.

Christopher M. Cashman, Chief Executive Officer of Marinus Pharmaceuticals, commented, "We are very encouraged with the data demonstrating ganaxolone IV safety and tolerability in GLP animal studies and robust activity in well accepted and translatable SE animal models. These studies indicate ganaxolone is a potential therapeutic for the treatment of SE, a life-threatening condition, which, if not arrested immediately, can result in marked neuronal damage, cognitive impairment and death. We are now kicking off the clinical development of ganaxolone IV as a potential treatment option for SE. Our strategy is to develop ganaxolone IV to complement our existing oral liquid and capsule formulations; expanding ganaxolone's reach into the acute care setting."

Marinus is planning to submit the preclinical data for presentation at an upcoming medical conference.

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 is currently being studied in a multi-national Phase 3 clinical trial in adults with focal onset seizures, an exploratory Phase 2 proof-of-concept clinical trial in children with PCDH19 female epilepsy, and in an exploratory Phase 2 proof-of-concept clinical trial in children with Fragile X syndrome. Ganaxolone has been studied in more than 1,300 subjects, including pediatric and adults, 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, proven efficacy, 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 the clinic 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 additional information, please visit the Company's website at 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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