



[Investors & Media](#)

[News](#)

[Events & Presentations](#)

[Financials](#)

[Stock Information](#)

[Governance](#)

[Resources](#)

News Details

[View All News](#) →

Marinus Pharmaceuticals Presents Clinical Data of Ganaxolone in Pediatric Epilepsies at Antiepileptic Drug and Device Trials XIII Conference

May 18, 2015

RADNOR, Pa., May 18, 2015 (GLOBE NEWSWIRE) -- [Marinus Pharmaceuticals, Inc.](#) (Nasdaq:MRNS), a biopharmaceutical company dedicated to the development of innovative epilepsy and other neuropsychiatric therapeutics, announced that Gail M. Farfel, Ph.D., Chief Clinical and Regulatory Officer of Marinus, presented clinical data on ganaxolone in drug-resistant pediatric epilepsies at the Antiepileptic Drug and Device Trials (AED) XIII conference on Friday, May 15, 2015.

Dr. Farfel shared clinical data on ganaxolone, a synthetic analog of the endogenous neurosteroid allopregnanolone, for the treatment of pediatric seizure disorders in children aged 4 months through 15 years. Across the one double-blind and four open-label studies of refractory pediatric subjects, approximately two thirds of subjects experienced improvements in seizures and one third of subjects experienced improvements of 50% or greater from baseline seizure frequency. The adverse event profile of ganaxolone in pediatric studies is similar to that seen in adults, without evidence of unique, clinically meaningful adverse events specific to the pediatric population. Most of the adverse events reported in the pediatric studies were mild or moderate in severity and expected based on ganaxolone pharmacology and the age group under evaluation.

In addition, during the presentation, Dr. Farfel highlighted the promising potential ganaxolone holds for additional pediatric seizure disorders, such as PCDH19 female pediatric epilepsy, a rare and serious epileptic syndrome caused by a mutation of the protocadherin 19 (PCDH19) gene and characterized by early-onset cluster seizures, cognitive and sensory impairment of varying degrees, and behavioral disturbances. Earlier this year, Marinus received orphan drug designation from the United States Food and Drug Administration for PCDH19 female epilepsy and initiated a multicenter, open-label, proof of concept Phase 2 clinical trial evaluating the safety and efficacy of ganaxolone in this condition. Further information about the Phase 2 study can be found on [ClinicalTrials.gov](#), using Identifier NCT02358538.

"In clinical studies, ganaxolone has consistently demonstrated the ability to reduce seizure frequency in pediatric patients," Dr. Farfel commented. "Based on ganaxolone's rational design and our evolving understanding of genetic contributions to epileptic syndromes, we believe the drug offers the potential to significantly reduce seizure frequency in girls with PCDH19 female epilepsy and other seizure disorders and provide these children with a treatment option that targets the pathophysiology of their condition."

About Ganaxolone

Ganaxolone is a small molecule that is a synthetic analog of allopregnanolone, an endogenous neurosteroid produced in the central nervous system that modulates GABA, the major brain inhibitory neurotransmitter. Ganaxolone was designed to have similar GABA modulation effects to allopregnanolone without unwanted steroidal side effects. Ganaxolone and allopregnanolone differ from other GABAA agents by interacting at both synaptic and extrasynaptic GABAA receptor binding sites that are located both within and outside the GABA synapse. Ganaxolone's activation of the extrasynaptic receptor is a unique mechanism that, in addition to other effects, increases tonic GABA signaling effects that we believe differentiates it from other available drugs that increase GABA levels in the brain. Preclinical studies provide evidence that the GABA modulatory activity of ganaxolone is responsible for its anticonvulsive activity in epileptic seizures and its anxiolytic effects in animal models of other neuropsychiatric disorders. Ganaxolone is being developed in three dose forms; oral capsule, oral liquid suspension, and intravenous formulation in order to bring convenient dosing to a variety of clinical situations.

About PCDH19 Female Pediatric Epilepsy

[PCDH19 female pediatric epilepsy](#) is a serious and rare epileptic syndrome that affects approximately 15,000-30,000 females in the United States. The condition, which is caused by an inherited mutation of the protocadherin 19 (PCDH19) gene, located on the X chromosome, is characterized by early-onset cluster seizures, cognitive and sensory impairment of varying degrees, and behavioral disturbances. The PCDH19 gene encodes a protein, protocadherin 19, which is part of a family of molecules supporting the communication between cells in the central nervous system. In case of mutation, protocadherin 19 may be malformed, reduced in its functions or not produced at all. The abnormal expression of protocadherin 19 is associated with occurrence of seizures

beginning in the early years of life, mostly focal clusters of seizures that last from one day to weeks. Often, but not always, the syndrome is also associated with a cognitive impairment of varying nature, and behavioral or social disorders with autistic traits. Currently, there are no approved therapies for PCDH19 female pediatric epilepsy.

About Marinus Pharmaceuticals

Marinus Pharmaceuticals, Inc., is a biopharmaceutical company dedicated to the development of innovative neuropsychiatric therapeutics. The Company's clinical stage drug candidate for the treatment of seizure disorders in adults and children with epilepsy is ganaxolone. Ganaxolone is a novel synthetic analog of the endogenous neurosteroid, allopregnanolone (known for its anticonvulsive and antianxiety effects) and was designed to avoid hormonal side effects associated with endogenous neurosteroids. The Company is currently conducting a multi-national, randomized, placebo-controlled, Phase 3 clinical trial to evaluate ganaxolone as adjunctive treatment of partial-onset seizures in adults. Ganaxolone is also being studied in a Phase 2 proof-of-concept clinical study for the treatment of the rare, genetic disorder, PCDH19 female pediatric epilepsy. To complement the existing formulations and to provide continuity of care, the Company is developing an IV formulation of ganaxolone for use in the hospital setting to control epileptic seizures. In addition, ganaxolone is being evaluated in a Phase 2 proof-of-concept investigator-sponsored clinical trial as a treatment for behaviors in 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", 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 expectations regarding our development plans for our product candidate, including, potential for orphan designation, optimizing a product's formulation, the clinical trial testing schedule, the ability to complete enrollment in our clinical trials, 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.

CONTACT: Company: Lisa M. Caperelli Senior Director, Investor Relations & Corporate Communications Marinus Pharmaceuticals, Inc. 484-801-4674 lcaperelli@marinuspharma.com
Media Contact: Tiberend Strategic Advisors, Inc. Amy S. Wheeler 646-362-5750 awheeler@tiberend.com

[View All News](#) →

CONTACT US

Jim DeNike
Senior Director, Investor
Relations
Marinus Pharmaceuticals, Inc.
jdenike@marinuspharma.com

Molly Cameron
Director, Corporate
Communications & Investor
Relations
Marinus Pharmaceuticals, Inc.
investors@marinuspharma.com

QUICK LINKS

[SEC FILINGS](#)
[INVESTOR FAQS](#)

INVESTOR EMAIL ALERTS

- | | |
|--|---|
| ☐ News | ☐ Events & Presentations |
| ☐ Quarterly Reports | ☐ Annual Reports |
| ☐ SEC Filings | ☐ End of Day Stock Quote |

[UNSUBSCRIBE](#)



© Marinus Pharmaceuticals, Inc.
Powered By Q4 Inc. 5.118.0.3

[Terms of Use](#)