Retrophin Inc Logo

Retrophin Announces Presentation of New Data from Physician-Initiated Treatment with Fosmetpantotenate at the Child Neurology Society's 26th Annual Meeting

October 5, 2017

Patient with PKAN showed persistent improvement in function over 30-month period of treatment with fosmetpantotenate

SAN DIEGO, Oct. 05, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced the presentation of new data from physician-initiated treatment with fosmetpantotenate (RE-024), the Company's novel, investigational replacement therapy for the treatment of pantothenate kinase-associated neurodegeneration (PKAN). Key findings suggest that 30-month treatment with fosmetpantotenate in a single patient with PKAN was associated with persistent improvement of the patient's functioning. The data were presented at the Child Neurology Society's (CNS) 26th Annual Meeting, taking place in Kansas City, MO, October 4-7, 2017.

"PKAN is a rare and life-threatening neurological disorder characterized by a host of progressively debilitating movement disorders, and it is encouraging to see persistent improvement of function over a 30-month period in this patient with PKAN who received fosmetpantotenate treatment," said Bill Rote, PhD, senior vice president and head of research and development for Retrophin. "We believe these observations provide additional support for our ongoing Phase 3 FORT Study, which positions us to make a meaningful difference for the PKAN community by providing the opportunity to deliver the first approved therapy."

A poster presentation titled, "Thirty Months of Treatment of a Patient with Pantothenate Kinase-Associated Neurodegeneration (PKAN) with Fosmetpantotenate (RE-024), A Phosphopantothenate Replacement Therapy," reported results from an open label, uncontrolled, physician-initiated treatment showing that an adult patient with PKAN experienced persistent improvement of functioning over a 30-month treatment period upon receiving fosmetpantotenate, with the newly-reported results covering months 15 to 30. These improvements were measured by the treating physician using the Unified Parkinson's Disease Rating Scale (UPDRS) Part II and the EuroQol 5-Dimensional 3-Level Scale (EQ-5D-3L). The treating physician reported that gait, and thus functional independence, also improved, and this improvement remained stable over the 30-month treatment period. Symptoms of dystonia also showed stabilization, and symptoms of parkinsonism were more variable over the 30-month time period, though overall did not show deterioration. Data describing the patient's initial 12-month period of treatment were originally presented at the 2016 American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting.

Also at the CNS 26th Annual Meeting, the Company presented a poster titled, "Fosmetpantotenate (RE-024) for Pantothenate Kinase-Associated Neurodegeneration (PKAN): Maximum Tolerated Dose (MTD) and Therapeutic-Dose Food Effect Studies in Adult Health Volunteers," which concluded that fosmetpantotenate was well tolerated at the planned clinical dose of 300 mg with and without food.

About the Phase 3 FORT Study

The FOsmetpantotenate Replacement Therapy (FORT) Study is an international, randomized, double-blind, placebo-controlled study evaluating fosmetpantotenate for the treatment of PKAN. This Phase 3 clinical trial commenced patient dosing in July 2017, and is designed to evaluate the safety and efficacy of fosmetpantotenate in approximately 82 patients with PKAN aged 6 to 65 years. The primary endpoint will be the change in score on the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale, from baseline through 24 weeks of treatment. After completing the 24-week treatment period, all patients will be eligible to receive fosmetpantotenate as part of an open-label extension.

The PKAN-ADL is a novel, PKAN-specific, patient-reported outcome scale measuring motor abilities to function in daily living for patients with PKAN. The scale is an adaptation of Part II of the comprehensive and widely-referenced Unified Parkinson's Disease Rating Scale (UPDRS). For the purposes of this trial, the UPDRS was adapted to be optimally relevant to PKAN through a systematic revision involving experts, patient advocacy leaders and regulatory interaction.

The FORT Study is being conducted under a Special Protocol Assessment (SPA) agreement, which indicates concurrence by the U.S. Food and Drug Administration (FDA) that if the data are positive, the design of the pivotal trial can adequately support a New Drug Application (NDA) seeking U.S. approval of fosmetpantotenate for the treatment of PKAN.

For additional information about the FORT Study and to learn more about eligibility, patients can visit pkanfortstudy.com.

About Pantothenate Kinase-Associated Neurodegeneration (PKAN)

PKAN is a rare, genetic and life-threatening neurological disorder characterized by a host of progressively debilitating symptoms that typically begin in early childhood. People suffering from PKAN may experience movement disorders such as dystonia (sustained muscle contraction leading to abnormal posture), rigidity, dysphagia (problems swallowing), and twisting and writhing, as well as visual impairment. PKAN is estimated to affect up to 5,000 people worldwide.

PKAN is caused by a mutation in the PANK2 gene, which encodes a critical protein that phosphorylates vitamin B5 (pantothenate), generating phosphopantothenate. The disruption of this metabolic pathway ultimately leads to decreased levels of coenzyme A (CoA).

About Fosmetpantotenate

Fosmetpantotenate (previously known as RE-024) is a novel investigational small molecule replacement therapy designed to pass the blood-brain barrier and be converted to phosphopantothenic acid (PPA), with the potential to be the first approved treatment targeting the underlying cause of PKAN. PPA synthesis is a key step in the biosynthesis of CoA, which is essential in biochemical reactions impacting energy metabolism, membrane integrity, signaling and other critical processes.

Preclinical findings suggest fosmetpantotenate has the ability to pass the blood-brain barrier and restore CoA levels. In a Phase 1 study, fosmetpantotenate was found to be safe and well-tolerated in healthy volunteers. Fosmetpantotenate has been granted orphan drug designation for the treatment of PKAN by the FDA and European Commission, as well as Fast Track status in the U.S.

About Retrophin

Retrophin is a fully-integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research exploring additional rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal®, Cholbam® and Thiola®.

Retrophin.com

Forward-Looking Statements

This press release contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the Company's business and finances in general, success of its commercial products, as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, with respect to fosmetpantotentate, the Company faces risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; risk that observations from the physician-initiated treatment that is the subject of this press release will not be replicated in the FORT study; risk that fosmetpantotenate will not be approved for efficacy, safety, regulatory or other reasons, risk associated with enrollment of clinical trials for rare diseases and risk the clinical trial may not succeed or may be delayed for safety, regulatory or other reasons. The Company faces risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates; risk relating to the Company's dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and intellectual property rights of third parties; and risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events or otherwise. Investors are referred to the full discussion of risks and uncertainties as included in the Company's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

Contact:
Chris Cline, CFA
Vice President, Investor Relations & Corporate Communications
646-564-3680
IR@retrophin.com

Primary Logo

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