July 25, 2017

Retrophin Commences Patient Dosing in International, Registrational Phase 3 Trial of RE-024 in PKAN

FORT Study to assess efficacy and safety of novel replacement therapy

SAN DIEGO, July 25, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that the first patient has been dosed in the FORT (FOsmetpantotenate Replacement Therapy) Study, an international, registrational Phase 3 clinical trial assessing the safety and efficacy of RE-024 (fosmetpantotenate) for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), a rare, genetic neurological disorder.

"Individuals with PKAN face a life-threatening neurological disorder for which there are no approved treatment options, and current therapeutic strategies are limited to symptom management," said Bill Rote, PhD, senior vice president and head of research and development for Retrophin. "The dosing of the first patient in the Phase 3 FORT Study is an important milestone for the RE-024 program, as it continues our progress towards developing a treatment with the potential to make a meaningful difference for the PKAN community."

RE-024 is a novel, investigational, small molecule replacement therapy that aims to restore levels of Coenzyme A (CoA), a naturally occurring molecule that is essential in many cellular functions and is decreased in individuals with PKAN.

About the FORT Study

The FORT Study is an international, randomized, double-blind, placebo-controlled study evaluating RE-024 for the treatment of PKAN. This Phase 3 clinical trial is designed to evaluate the safety and efficacy of RE-024 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 RE-024 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 the design of the pivotal trial can adequately support a New Drug Application (NDA) seeking U.S. approval of RE-024 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 CoA.

About RE-024 (fosmetpantotenate)

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 RE-024 has the ability to pass the blood-brain barrier and restore CoA levels. In a Phase 1 study, RE-024 was found to be safe and well-tolerated in healthy volunteers. RE-024 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 sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease, and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood. Research exploring the potential of early-stage assets in additional rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola®, Cholbam® and Chenodal®.

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 RE-024, the Company faces risk that the Phase 3 clinical trial of RE-024 will not demonstrate that RE-024 is safe or effective or serve as the basis for an NDA filing as planned; risk that RE-024 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

760-260-8600

IR@retrophin.com

Primary Logo

Source: Retrophin, Inc.

News Provided by Acquire Media