Retrophin Reaches Agreement with FDA under Special Protocol Assessment for Pivotal Trial Evaluating RE-024 in PKAN

November 10, 2016

SAN DIEGO , Nov. 10, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (Nasdaq:RTRX) today announced it has reached an agreement with the U.S. Food and Drug Administration (FDA) under the Special Protocol Assessment (SPA) process for a Phase 3 clinical trial evaluating RE-024, the Company's novel investigational replacement therapy, for the treatment of pantothenate kinase-associated neurodegeneration (PKAN). The SPA indicates concurrence by the 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. The Company plans to initiate this trial before year-end 2016.

"This SPA agreement marks a major milestone for the RE-024 program, as we now have a single pivotal trial design that clarifies our regulatory pathway and positions us to deliver the first approved treatment for PKAN," said Stephen Aselage, chief executive officer of Retrophin. "We look forward to initiating the trial before year-end and enrolling patients as quickly as possible, with the hope of filling this significant unmet medical need."

As outlined in the agreement, the Phase 3 clinical trial will be an international, randomized, double-blind, placebo-controlled study evaluating RE-024 for the treatment of PKAN. This pivotal trial will 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.

About Special Protocol Assessment (SPA)

SPA is a process in which sponsors engage the U.S. Food and Drug Administration (FDA) to reach agreement on key study design features, such as size, entry criteria, dose, endpoints and planned analyses. This agreement ensures that a trial has the potential to support a New Drug Application (NDA) that meets regulatory requirements for an FDA approval. An SPA agreement does not guarantee that the agency will accept an NDA, or that the trial results will be adequate to support approval.

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. There is no approved treatment for PKAN and current therapeutic strategies are limited to symptom management. 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) to phosphopantothenate. The disruption of this metabolic pathway ultimately leads to decreased levels of coenzyme A (CoA), which plays an important role in many cellular functions.

About RE-024

RE-024 is a novel small molecule in Phase 3 clinical development that could be the first approved replacement therapy targeting the underlying cause of PKAN. Preclinical findings suggest RE-024 has the ability to distribute to the brain and restore CoA levels. In a Phase 1 study, RE-024 was found to be safe and well-tolerated in healthy volunteers. In 2016, Retrophin reached agreement with the U.S. Food and Drug Administration (FDA) on the design of its Phase 3 clinical trial for RE-024 under the Special Protocol Assessment process. The Company plans to initiate the trial before year-end 2016.

In 2015, the FDA granted orphan drug designation and Fast Track status to RE-024 for the treatment of PKAN. In 2016, the European Commission granted orphan drug designation to RE-024 for the treatment of PKAN.

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 clinical-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 several 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. 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, as well as risks and uncertainties associated with the Company's research, preclinical and clinical stage pipeline. Specifically, the risks and uncertainties the Company faces with respect to its RE-024 program include risk associated with enrollment of clinical trials for rare diseases and risk that the Company's Phase 3 clinical trial will fail to demonstrate that RE-024 is safe and effective. 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 forward-looking statements, 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 filings with the Securities and Exchange Commission.

Contacts:

(Investors)

Chris Cline, CFA

Senior Director, Investor Relations

646-564-3680

IR@retrophin.com

(Media)

Scott Santiamo

Associate Director,

Corporate Communications

646-564-3672

scott.santiamo@retrophin.com

Source: Retrophin, Inc.