Retrophin Completes Enrollment of Pivotal Phase 3 FORT Study of Fosmetpantotenate in PKAN

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Top-line data anticipated in the third quarter of 2019

SAN DIEGO, Dec. 20, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced completion of patient enrollment in the pivotal Phase 3 FORT Study, which is evaluating the safety and efficacy of fosmetpantotenate for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), a rare, progressive neurodegenerative genetic disorder. The FORT Study is designed to be registration-enabling in the U.S. and Europe, and top-line results are expected in the third quarter of 2019.

“Reaching this milestone brings us closer to potentially delivering fosmetpantotenate as the first disease-modifying therapy to patients with PKAN,” said Noah Rosenberg, M.D., chief medical officer of Retrophin. “We are very grateful to the PKAN community for their support in raising awareness of this important trial, and we look forward to the readout of top-line data in the third quarter of 2019.”

The Phase 3 FORT Study is an international, pivotal clinical trial designed to assess the safety and efficacy of fosmetpantotenate in approximately 82 patients with PKAN aged 6 to 65 years. The primary endpoint of the study is the change from baseline in the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale 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 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 trial can adequately support the filing of a New Drug Application (NDA).

About 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), which is essential in biochemical reactions impacting energy metabolism, membrane integrity, signaling and other critical processes.

About Fosmetpantotenate

Fosmetpantotenate is a novel investigational small molecule replacement therapy designed to pass the blood-brain barrier and be converted to phosphopantothenic acid (PPA). PPA synthesis is a key step in the biosynthesis of CoA. Preclinical findings suggest fosmetpantotenate has the ability to pass the blood-brain barrier and restore CoA levels.

Fosmetpantotenate, which has the potential to be the first approved treatment targeting the underlying cause of PKAN, 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. by the FDA. In a Phase 1 study, fosmetpantotenate was found to be generally well-tolerated in healthy volunteers and it is currently being evaluated in the pivotal Phase 3 FORT Study conducted under a SPA agreement with the FDA.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare disease. 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) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. 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 fosmetpantotenate, 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 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.

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