Retrophin Announces Pivotal Phase 3 FORT Study of Fosmetpantotenate in PKAN Opens Enrollment to Pediatric Patients

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Independent Data Monitoring Committee's (DMC) review of available safety data supports continuing with study as planned; FORT Study now open to pediatric patients aged 6 to 17

Top-line data anticipated in the second half of 2019

SAN DIEGO, June 04, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that the independent Data Monitoring Committee (DMC) for the pivotal Phase 3 FORT Study, which is evaluating fosmetpantotenate for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), has completed its scheduled clinical safety review required to open enrollment for pediatric patients. Upon review of the available safety and tolerability data of fosmetpantotenate in adult patients with PKAN in the study to date, the DMC recommended that the pivotal trial continue as planned, and supported initiation of enrollment in pediatric patients aged 6 to 17.

"PKAN is a rare and life-threatening genetic disorder that typically begins in early childhood, so we are particularly pleased to reach this important milestone and begin pediatric enrollment in the FORT Study," said Bill Rote, PhD, senior vice president and head of research and development for Retrophin. "The DMC's review is encouraging for the overall safety and tolerability of fosmetpantotenate and supports our optimism that the FORT Study should ultimately enable us to deliver the first approved and transformative treatment for the PKAN community."

Fosmetpantotenate 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. The FORT Study is a pivotal Phase 3 clinical trial conducted under a Special Protocol Assessment (SPA) agreement with the U.S. Food and Drug Administration. Under the terms of the SPA agreement, the FORT Study protocol required an initial group of adult patients be treated with fosmetpantotenate to allow a safety assessment by the DMC prior to opening enrollment to pediatric patients aged 6 to 17. The Company anticipates completion of patient enrollment around year-end 2018, and top-line data to become available in the second half of 2019.

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 CoA.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. 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®.

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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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