Retrophin Announces Topline Results from Phase 3 FORT Study of Fosmetpantotenate in Patients with PKAN

August 22, 2019

FORT Study did not achieve its primary or secondary endpoints

Company to host conference call and webcast today at 8:30 a.m. ET

SAN DIEGO, Aug. 22, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the Phase 3 FORT Study evaluating the safety and efficacy of fosmetpantotenate compared to placebo in patients with pantothenate kinase-associated neurodegeneration (PKAN) did not meet its primary endpoint and did not demonstrate a difference between treatment groups. The study also did not meet its secondary endpoint. Fosmetpantotenate was observed to be generally safe and well-tolerated in the study.

“We are very disappointed in the topline results from the FORT Study, particularly because we have seen the devastating impact of PKAN on patients and their families, and a significant unmet need remains with no approved treatment option. We would like to thank the patients, their caregivers, study investigators and our employees, whose dedication made this study possible," said Eric Dube, Ph.D., chief executive officer of Retrophin. "We will work closely with the investigators to further analyze the results of the study and share them with the PKAN community to contribute to the growing knowledge of this rare disorder."

Data from the FORT Study will be further analyzed and are expected to be presented at an upcoming scientific congress, and the Company will be working with study investigators to determine the appropriate next steps for the FORT Study, including the ongoing open-label extension of the study.

Consistent with its mission of identifying, developing and delivering life-changing therapies to people living with rare disease, Retrophin remains focused on progressing its two pivotal Phase 3 programs evaluating sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS) and IgA nephropathy, and continuing the advancement of its commercial portfolio.

Conference Call Information

Retrophin will host a conference call and webcast today, Thursday, August 22, 2019 at 8:30 a.m. ET to discuss the FORT Study results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 1859204 shortly before 8:30 a.m. ET. The webcast can be accessed at retrophin.com, in the Events and Presentations section, and will be archived for at least 30 days. A replay of the call will be available from 11:30 a.m. ET, August 22, 2019 to 11:30 a.m. ET, August 29, 2019. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 1859204.

About the FORT Study

The FORT Study was an international, randomized, double-blind, placebo-controlled, Phase 3 clinical trial assessing the safety and efficacy of fosmetpantotenate in 84 patients with PKAN. Patients received either three times-daily dosing of fosmetpantotenate or placebo using a 1:1 randomization over 24 weeks. The primary endpoint in the study was the change from baseline in the PKAN-ADL scale through 24 weeks of treatment. After completing the 24-week treatment period, all patients were eligible to receive fosmetpantotenate as part of an open-label extension.

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 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. 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. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that current clinical trials will not proceed as planned. With respect to fosmetpantotenate, the Company faces risk associated with the close-out of the FORT Study, including the on-going open label extension, and other actions to be taken following the unfavorable FORT Study read-out. 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 exclusivity periods and intellectual property rights of third parties; risks associated with regulatory interactions; risks and uncertainties relating to competitive products, including potential generic competition with certain of the Company's 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
888-969-7879
IR@retrophin.com

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