Retrophin Announces FDA Review of IND for RE-024 and Clearance to Proceed With Phase 1 Clinical Study in Healthy Adult Volunteers

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SAN DIEGO--(BUSINESS WIRE)-- Retrophin, Inc. (NASDAQ:RTRX) today announced that the U.S. Food and Drug Administration (FDA) has reviewed the Company's Investigational New Drug (IND) application for RE-024, a novel phosphopantothenate replacement therapy, and has granted clearance to begin clinical studies. The Company is initiating a Phase 1 trial in healthy adult subjects to evaluate safety, tolerability, and pharmacokinetics, with enrollment expected to begin in the coming weeks. RE-024 is being developed for the treatment of pantothenate kinase-associated neurodegeneration (PKAN), a rare and lethal autosomal recessive neurodegenerative disorder believed to affect approximately one to three persons per million worldwide, with no approved treatment currently available.

"We are excited to see RE-024 move forward into the clinic and get one step closer to reaching patients who are suffering from this rare and debilitating disease," said Dr. Alvin Shih, Executive Vice President and Head of Research & Development for Retrophin. "With the continued support of the scientific and patient communities, our research and development team will continue to rapidly advance RE-024 through clinical development with the hope of providing these patients with a much needed treatment option."

The Phase 1 trial is a randomized, double-blind, placebo-controlled single ascending dose study of orally administered RE-024 in healthy volunteer subjects. The primary objective of the study is to assess safety and tolerability of single oral doses of RE-024. As a secondary objective, pharmacokinetics of RE-024 will be assessed at multiple time points. Pending study completion, safety and pharmacokinetic data will be submitted for publication.

About RE-024 & PKAN

RE-024 is a small molecule discovered by Retrophin that is being developed as a replacement therapy for phosphopantothenate. Preclinical studies in PANK-deficient animal models and cell lines have demonstrated the ability of RE-024 to restore Coenzyme A (CoA) levels. PKAN is caused by a mutation in the PANK2 gene, which encodes a critical protein that metabolizes vitamin B5 (pantothenate) to phosphopantothenate. The disruption of this metabolic pathway ultimately leads to decreased levels of CoA, an important substrate for many functions such as mitochondrial energy metabolism. Clinical manifestations of PKAN include developmental delay in children, dystonia sometimes causing intractable pain, parkinsonism with Parkinson's-like freezing and bradykinesia, choreoathetosis, status dystonicus, dysarthria, spasticity, rigidity, and dysphagia often leading to feeding tube placement.

About Retrophin

Retrophin is a pharmaceutical company focused on the development, acquisition and commercialization of drugs for the treatment of serious, catastrophic or rare diseases for which there are currently no viable options for patients. The Company's approved products include Chenodal®, Cholbam™, and Thiola®, and its pipeline includes compounds for several catastrophic diseases, including focal segmental glomerulosclerosis (FSGS), pantothenate kinase-associated neurodegeneration (PKAN), infantile spasms, nephrotic syndrome and others. For additional information, please visit www.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, regarding the research, development and commercialization of pharmaceutical products. 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, as well as risks and uncertainties associated with the Company's pre-clinical and clinical stage pipeline as well as its sales and marketing strategies. Specifically, the risks and uncertainties the Company faces with respect to its RE-024 program include risk that the Company will be unable to initiate and/or complete Phase 1 clinical trials, risk that RE-024 will not progress to Phase 2 clinical trials for safety, regulatory or other reasons; risk associated with enrollment of clinical trials for rare diseases; risk that the company's later stage RE-024 clinical studies will fail to demonstrate that RE-024 is safe and effective. The Company also faces risk that it will be unable to raise additional funding 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; 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 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 filings with the Securities and Exchange Commission.

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