Retrophin Receives European Orphan Drug Designation for RE-024 for the Treatment of Pantothenate Kinase-Associated Neurodegeneration

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SAN DIEGO, Feb. 24, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that the European Commission has granted orphan drug designation to RE-024, the Company's novel investigational phosphopantothenate replacement therapy for pantothenate kinase-associated neurodegeneration (PKAN), a rare and life-threatening genetic disorder with no approved treatment option.

"Receiving orphan drug status in the EU is another encouraging step in the development of RE-024 for the treatment of PKAN," said Alvin Shih, M.D., executive vice president and head of research & development for Retrophin. "Without an approved therapy, many people living with PKAN currently face a significant burden of illness. We look forward to continuing our development of RE-024 with the ultimate goal of delivering an effective therapeutic option to these patients."

In the European Union (EU), orphan drug designation is granted to a medicinal product intended to treat a life-threatening or chronically debilitating rare disease with no approved treatment option. In the EU, a disease is designated as rare if it affects no more than five in 10,000 people. In addition to a 10-year period of EU market exclusivity upon product approval, orphan drug designation provides fee waivers, protocol assistance, and single marketing authorization under the centralized procedure that is valid in all EU countries.

In 2015, the U.S. Food and Drug Administration granted orphan drug designation to RE-024 for the treatment of PKAN, as well as Fast Track status, which is designed to facilitate the development and expedite the review of medicines to treat serious conditions with unmet medical needs in order to reach patients earlier.

About Pantothenate Kinase-Associated Neurodegeneration (PKAN)

Pantothenate kinase-associated neurodegeneration, or 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), twisting and writhing, and tremors, as well as visual impairment. Currently, there is no approved treatment option for PKAN, which 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 1 clinical development as a phosphopantothenate replacement therapy for PKAN. Preclinical findings from studies of PANK-deficient animal models and cell lines suggest the ability of RE-024 to restore CoA levels.

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 no approved treatment options, 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, including RE-034, in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's marketed 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. 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 pre-clinical and clinical stage pipeline. Specifically, the risks and uncertainties the Company faces with respect to its RE-024 program include risk that RE-024 will not progress to Phase 2 or later-stage 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. 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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