Retrophin Announces Acceptance of Four RE-024 Abstracts for Presentation at the 20th International Congress of Parkinson's Disease and Movement Disorders

March 24, 2016

SAN DIEGO, March 24, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that four abstracts related to the development of RE-024, the company's novel investigational replacement therapy for pantothenate kinase-associated neurodegeneration (PKAN), have been accepted for poster presentation at the 20th International Congress of Parkinson's Disease and Movement Disorders, to be held June 19-23, 2016 in Berlin, Germany. The abstracts include new case reports of two PKAN patients who received RE-024 as part of physician-initiated treatment.

"We are pleased that data for RE-024 will be presented at the MDS International Congress," said Alvin Shih, M.D., executive vice president and head of research & development for Retrophin. "The new data from two PKAN patients treated with RE-024 will add to the growing body of evidence supporting clinical development of this investigational therapy for the treatment of PKAN."

PKAN is a rare and life-threatening neurodegenerative condition characterized by a host of progressively debilitating movement disorders. There are no approved treatment options for PKAN and current therapeutic strategies focus on symptom management.

The following RE-024 abstracts will be presented as part of the congress poster sessions scheduled for June 23, 12:00 - 13:30 p.m. CEST in the Exhibit Hall, Hall B, Level 2:

- **Abstract #2096**: "RE-024: Mechanism of Action and Efficacy in Non-Clinical Models"
- **Abstract #2105**: "Development of a Human Neuroblastoma Model of Pantothenate Kinase-Associated Neurodegeneration"
- **Abstract #2106**: "A Healthy Volunteer Phase 1 Study of RE-024, A Potential Phosphopantothenate Replacement Therapy for Patients with Pantothenate Kinase-Associated Neurodegeneration (PKAN)"
- **Abstract #2108**: "RE-024: A Potential Phosphopantothenate Replacement Therapy in 2 Patients with Pantothenate Kinase-Associated Neurodegeneration (PKAN)"

About Pantothenate Kinase-Associated Neurodegeneration (PKAN)

Pantothenate kinase-associated neurodegeneration, or PKAN, is a rare, genetic, and life-threatening condition characterized by a host of progressively debilitating movement disorders that typically begin in early childhood. People suffering from PKAN may experience dystonia (sustained muscle contraction leading to abnormal posture), rigidity, dysphagia (problems swallowing), twisting and writhing, and visual impairment. There are no approved treatment options for PKAN and current therapeutic strategies focus on symptom management. 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) 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 with the potential to be the first approved replacement therapy targeting the underlying cause of PKAN. Preclinical findings suggest RE-024 has the ability to restore CoA levels and the potential to distribute to the brain in humans. Retrophin expects to initiate an efficacy trial of RE-024 in patients with PKAN in 2016.

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. In 2016, the European Commission granted orphan drug designation to RE-024, which is granted to a medicinal product intended to treat a life-threatening or chronically-debilitating rare disease with no approved treatment option.

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 preclinical 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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