Retrophin Announces New Data from Physician-Initiated Treatment with RE-024 at the 20th International Congress of Parkinson's Disease and Movement Disorders

June 23, 2016

SAN DIEGO, June 23, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced new data from physician-initiated treatment with RE-024, the company's novel investigational replacement therapy for pantothenate kinase-associated neurodegeneration (PKAN), a rare and life-threatening genetic condition characterized by a host of progressively debilitating movement disorders. Key findings suggest RE-024 was safe and well tolerated in two adults with PKAN who experienced clinically meaningful improvements, followed by stabilization of disease progression over 47 weeks of treatment.

These and data from three additional posters were presented at the 20th International Congress of Parkinson's Disease and Movement Disorders in Berlin, Germany.

"We are encouraged by the meaningful clinical improvements observed in both of these patients treated with RE-024," said Alvin Shih, M.D., executive vice president and head of research & development for Retrophin. "These data add to the growing body of evidence demonstrating the potential of RE-024 in patients with PKAN and support our plans to initiate an efficacy study during the second half of 2016."

The newly released data describe the experiences of two sibling males with PKAN who received physician-initiated treatment with RE-024. For each patient, initial symptoms presented in childhood and both eventually lost their ability to walk independently. The patients developed parkinsonism, dystonia, dysarthria, and extreme impulsivity. Decreased quality of life and significantly impaired function in activities related to daily living were also reported.

In both patients, treatment with RE-024 was associated with clinically meaningful improvements, including the regained ability to walk unassisted for short distances. These improvements were demonstrated on Parts II and III of the Movement Disorder Society-Unified Parkinson's Disease Rating Scale (MDS-UPDRS) assessing activities of daily living and neurological impairment, respectively. After 47 weeks of treatment with RE-024, mean improvements of 13.0 points (40.6 percent) on Part II, and 17.5 points (26.7 percent) on Part III of the scale were observed. Most or all of the clinically meaningful improvements were observed over a six-month period, and were followed by stabilization at the improved levels, suggesting the potential durability of sustained treatment with RE-024 in this otherwise progressive disease. The treating physician noted that although this degree of improvement followed by stabilization is not typical of the natural history of PKAN, placebo response cannot be ruled out in an uncontrolled setting. The physician also concluded that controlled studies should evaluate the safety and efficacy of RE-024 in patients with PKAN.

No treatment-associated adverse events or other medical conditions were observed in either patient during the 47 weeks of treatment with RE-024, and both patients elected to remain on treatment.

Patient 1

Patient 1 initially presented with frequent falls at age 10, followed by progressive deterioration such that he was unable to walk without assistance by age 18. Additional manifestations of PKAN included widespread presentation of dystonia, as well as bradykinesia, dysphagia, cognitive decline, extreme impulsivity, and dysarthria. His ability to speak, write, dress, maintain personal hygiene and eat were significantly affected.

At age 24, treatment with RE-024 was initiated at a low dose and gradually increased over five days to 180 mg daily, administered orally as 60 mg three times per day.

Within eight weeks of initiating treatment with RE-024, the patient experienced gradual improvement in gait, such that he was consistently walking unassisted for short distances. Gait continued to improve over several months of treatment. The physician reported improvements in orofacial dystonia and speech, as well as hand dystonia and writing. At week 47, these results translated to improvements of 11.0 points (40.7 percent) on Part II and 12.0 points (21.4 percent) on Part III of the MDS-UPDRS scale. In addition, the physician and family members reported that impulsivity was dramatically improved, allowing for the discontinuation of concomitant treatment with risperidone.

Patient 2

Patient 2 initially presented with frequent falls, widespread presentation of dystonia, and bradykinesia at age 10. This was followed by progressive deterioration such that he was unable to speak, and in need of assistance to walk and perform most activities of daily living by age 23. The patient also required a feeding tube by age 26. Additional manifestations of PKAN included dysphagia, freezing, extreme impulsivity, and dysarthria.

At age 29, treatment with RE-024 was initiated at a low dose and gradually increased over five days to 120 mg daily, administered orally as 40 mg three times per day.

Within eight weeks of initiating treatment with RE-024, the patient experienced gradual improvement in gait such that he was able to walk unassisted for short distances. Gait continued to improve over several months of treatment. The physician reported that the patient is more attentive and interactive, has shown greater independence, and is able to get out of bed without assistance. At week 47, these results translated to improvements of 15.0 points (40.5 percent) on Part II, and 23.0 points (30.7 percent) on Part III of the MDS-UPDRS scale. In addition, the physician and family reported that impulsivity was notably improved.

Additional Data Presentations

Previously released data for RE-024 were also presented at the MDS International Congress, and included: a Phase 1 single ascending dose study of 40 healthy adult volunteers; preclinical data describing the development of the first human cellular model of PKAN; and animal studies supporting the proposed mechanism of action of RE-024 and its potential to distribute to the brain in humans. These data were originally presented at the 2016 American College of Medical Genetics and Genomics Annual Clinical Genetics Meeting in March 2016.

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), and twisting and writhing, as well as visual impairment. There is no approved treatment for PKAN and current therapeutic strategies are limited to 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 that has 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 to distribute to the brain. Retrophin is planning to initiate an efficacy trial of RE-024 in patients with PKAN in the second half of 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 significant unmet medical needs, 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 in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola®, Cholbam® and Chenodal®.

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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Source: Retrophin, Inc.