May 25, 2017

Retrophin Announces Presentations for Late-Stage Clinical Development Programs at Upcoming Medical Congresses

Subgroup analysis from Phase 2 DUET study of sparsentan in FSGS to be presented at ERA-EDTA

PKAN-ADL scale in Phase 3 FORT study of RE-024 in PKAN to be highlighted at MDS

SAN DIEGO, May 25, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced upcoming presentations related to its late-stage development programs, sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS) and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), at two upcoming medical congresses.

The Company and its collaborators will present research from the sparsentan program, including a subgroup analysis from the Phase 2 DUET study, at the 54th European Renal Association - European Dialysis and Transplant Association (ERA-EDTA) Congress to be held June 3 to 6, 2017 in Madrid.

Additionally, a poster presentation supporting the use of the Pantothenate Kinase-associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale in the Phase 3 FORT study of RE-024 will be presented at the 21st International Congress of Parkinson's Disease and Movement Disorders (MDS), to be held June 4 to 8, 2017 in Vancouver, British Columbia.

Details for presentations at both medical congresses are as follows:

54th ERA-EDTA Congress 2017 Oral Presentation:

Antiproteinuric Effect of Sparsentan, A Dual Angiotensin II and Endothelin Type A Receptor Antagonist, In Patients With Primary Focal Segmental Glomerulosclerosis (FSGS): A Subgroup Analysis Of The Duet Trial
Session: Experimental Therapies
Sala Neptuno / IFEMA Feria de Madrid (North Congress Center)
Tuesday, June 6, 2017
10:45 a.m. CET / 4:45 p.m. ET

54th ERA-EDTA Congress 2017 Poster Presentation:

In Vitro Evaluation of Induction of Cytochrome P450 Enzymes by Sparsentan And Effects of Its Metabolism by Potential Co-Administered Drugs
Poster #SP124
Poster Area / IFEMA Feria de Madrid (North Congress Center)
Sunday, June 4, 2017
9:30 a.m. CET - 10:45 a.m. CET / 3:30 p.m. ET - 4:45 p.m. ET

21st International MDS Congress Poster Presentation:

Development of a Clinical Outcomes Assessment (COA) in Pantothenate Kinase-Associated Neurodegeneration (PKAN): Item Generation and Clinimetric Properties
Poster #1295
Exhibit Hall C, Vancouver Convention Centre - WEST
Thursday, June 8, 2017
1:15 p.m. - 2:45 p.m. PT

About Focal Segmental Glomerulosclerosis

FSGS is a rare disorder without a U.S. Food and Drug Administration (FDA)-approved pharmacologic treatment option that is estimated to affect up to 40,000 patients in the U.S. with similar prevalence in Europe. The disorder is defined by progressive scarring of the kidney and often leads to end-stage renal disease. FSGS is characterized by proteinuria, where protein is found in the urine due to a breakdown of the normal filtration mechanism in the kidney. Other common symptoms

include swelling in parts of the body known as edema, as well as low blood albumin levels, abnormal lipid profiles, and hypertension.

Reduction in proteinuria is widely regarded to be beneficial in the treatment of FSGS, and may be associated with a decreased risk of progression to end-stage renal disease. Achieving modified partial remission of proteinuria, defined as proteinuria levels of less than or equal to 1.5 g/g and greater than 40 percent reduction of proteinuria from baseline, is associated with long-term preservation of renal function in patients with FSGS. In the absence of an FDA-approved pharmacologic treatment, patients with FSGS are currently managed with angiotensin receptor blockers, angiotensin converting enzyme inhibitors, steroids or calcineurin inhibitors.

About Sparsentan

Sparsentan could be the first FDA-approved pharmacologic treatment for FSGS; its dual mechanism of action combines angiotensin receptor blockade with endothelin receptor type A blockade. In several forms of chronic kidney disease, endothelin receptor blockade has been shown to have an additive beneficial effect on proteinuria in combination with renin-angiotensin blockade via angiotensin receptor blockade or angiotensin converting enzyme inhibitors. Sparsentan has been granted orphan drug designation for the treatment of FSGS by the FDA and European Commission.

The Phase 2 DUET study of sparsentan met the primary efficacy endpoint for the combined treatment group, demonstrating a greater than two-fold reduction of proteinuria compared to irbesartan, after the eight-week, double-blind treatment period. Following an End of Phase 2 meeting with the FDA during the first quarter of 2017, the Company announced plans to initiate a pivotal Phase 3 clinical trial of sparsentan in FSGS. The study will include an interim analysis of proteinuria to serve as the basis for a New Drug Application (NDA) filing for Subpart H accelerated approval of sparsentan. The confirmatory endpoint of the study is expected to compare changes from baseline in estimated glomerular filtration rate, or eGFR, which is widely regarded as the best overall measure of kidney function. The Company expects to finalize the Phase 3 protocol and confirm alignment with the FDA in the second half of 2017, with the pivotal trial expected to initiate thereafter.

About Pantothenate Kinase-Associated Neurodegeneration

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 replacement therapy that has the potential to be the first approved treatment targeting the underlying cause of PKAN. Preclinical findings suggest RE-024 has the ability to distribute to the brain and restore CoA levels. In a Phase 1 study, RE-024 was found to be safe and well-tolerated in healthy volunteers. RE-024 has been granted orphan drug designation for the treatment of PKAN by the FDA and European Commission, as well as Fast Track status in the U.S., 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.

Recruitment activities for the Phase 3 FORT study evaluating RE-024 for the treatment of PKAN have begun, with the first patients expected to receive dosing mid-year 2017. In 2016, Retrophin reached agreement with the FDA on the design of the FORT study of RE-024 under the Special Protocol Assessment process. This agreement ensures that a trial has the potential to support an NDA that meets regulatory requirements for an FDA approval.

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 late-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 additional 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. 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. Specifically, the Company faces risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing therapies. 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 planned clinical trials will not proceed as planned. Specifically, the Company faces the risk that the planned Phase 3 clinical trial of sparsentan will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the Phase 3 clinical trial of RE-024 will not demonstrate that RE-024 is safe or effective or serve as the basis for an NDA filing as planned; and risk that the Company's product candidates will not be approved for efficacy, safety, regulatory or other reasons, and for each of the programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. 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 intellectual property rights of third parties; and 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 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.

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