Retrophin Completes Enrollment of Phase 2 DUET Study of Sparsentan in Focal Segmental Glomerulosclerosis

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SAN DIEGO, March 30, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced the Company has completed enrollment of the Phase 2 DUET study of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney disorder without an approved pharmacological treatment option that often leads to end-stage renal disease. The DUET study exceeded its enrollment target of 100 patients, and top-line results are expected in the third quarter of 2016.

"Completing enrollment of the DUET study is an important step towards generating data that we hope will support the approval of sparsentan for the treatment of FSGS," said Alvin Shih, M.D., executive vice president and head of research & development for Retrophin. "We are pleased to see that DUET exceeded its enrollment target and look forward to sharing top-line results in the third quarter of 2016."

The DUET study is an international, randomized, double-blind Phase 2 clinical trial assessing the safety and efficacy of sparsentan as compared to an active control, irbesartan. Angiotensin receptor blockers, such as irbesartan, are used to manage FSGS in the absence of an approved pharmacological treatment option. After a two-week washout period, patients are randomized 3:1 (sparsentan:irbesartan) with dosing arms of 200 mg, 400 mg, and 800 mg of sparsentan, compared to 300 mg of irbesartan. The primary endpoint of the study is reduction of proteinuria compared to irbesartan following eight weeks of treatment. After completing the eight-week observation period, all patients are eligible to receive sparsentan as part of an open-label extension.

About Focal Segmental Glomerulosclerosis (FSGS)

Focal segmental glomerulosclerosis, or FSGS, is a rare disorder without an approved pharmacological 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. In the absence of an approved pharmacological treatment option, FSGS patients are currently managed with steroids, calcineurin inhibitors, angiotensin receptor blockers and angiotensin converting enzyme inhibitors.

About Sparsentan

Sparsentan, also known as RE-021, is an investigational therapy with the potential to be the first approved pharmacologic treatment option for patients with FSGS. Sparsentan's 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.

Top-line results from DUET, the Phase 2 study of sparsentan for the treatment of FSGS, are expected in the third quarter of 2016 and may support an application for accelerated approval of the therapy using proteinuria as a surrogate endpoint. In 2015, the U.S. Food and Drug Administration and European Commission each granted sparsentan orphan drug designation for the treatment of FSGS.

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, 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 Company faces risk that the sparsentan Phase 2 clinical trials will fail to demonstrate that sparsentan is safe or effective and risk that the sparsentan Phase 2 program will be delayed for regulatory or other reasons. 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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