September 7, 2016

Retrophin Announces Positive Top-Line Results from Phase 2 DUET Study of Sparsentan in Patients with Focal Segmental Glomerulosclerosis

Combined sparsentan treatment group experienced 44.8 percent reduction of proteinuria, more than double the reduction of irbesartan; achieves statistical significance in primary efficacy endpoint

Preliminary safety findings show sparsentan was generally safe and well-tolerated

Conference call scheduled for today at 8:30 a.m. ET

SAN DIEGO, Sept. 07, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced positive top-line results from the Phase 2 DUET study of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney disorder without an approved pharmacologic treatment that often leads to end-stage renal disease. The study achieved statistical significance in the primary efficacy endpoint for the overall sparsentan treatment group, demonstrating a greater than two-fold reduction of proteinuria compared to irbesartan after the eight-week, double-blind treatment period.

"We are very pleased with the robust top-line results from DUET, which suggest sparsentan could be a significant advancement in the treatment of FSGS," said Stephen Aselage, chief executive officer of Retrophin. "FSGS patients today face poor outcomes with limited medical options; we look forward to working with the FDA to find the most expeditious path forward that would deliver the first approved pharmacologic treatment to the FSGS community."

In the DUET study, the mean reduction of proteinuria from baseline after eight weeks of treatment for all patients treated with 200, 400, and 800 mg/day of sparsentan (n=64) was 44.8 percent, compared to a mean reduction of proteinuria for all patients receiving 300 mg/day of irbesartan (n=32) of 18.5 percent (p=0.006). Further, the mean reduction of proteinuria from baseline after eight weeks of treatment for all patients treated with 400 mg and 800 mg doses of sparsentan (n=51) was 47.4 percent, compared to a mean proteinuria reduction of 19.0 percent for patients receiving 300 mg of irbesartan (n=25) in these two dose cohorts (p=0.011). The comparison of individual sparsentan dose cohorts to irbesartan showed clear signals of relative improvement, but did not reach statistical significance.

"The results from DUET serve as proof of concept for sparsentan's novel approach of combining endothelin receptor type A blockade with angiotensin II inhibition for the treatment of FSGS," said Alvin Shih, M.D., executive vice president and head of research & development for Retrophin. "Significant reductions in proteinuria, along with a well-tolerated preliminary safety profile have us excited about being one step closer to providing a new treatment option for patients with FSGS."

Top-line results suggest sparsentan was generally safe and well-tolerated in the DUET study. One serious adverse event, anemia, classified as potentially related to treatment occurred in the sparsentan group but did not result in study discontinuation during the eight-week blinded treatment period. There were no withdrawals due to fluid retention during the eight-week blinded treatment period. All patients who completed the eight-week treatment period entered the ongoing open label extension study, and the vast majority of these patients continue to receive therapy.

The Company plans to present detailed study results, including data from the open label extension, at an upcoming medical meeting or in a peer-reviewed publication.

Conference Call Information

Retrophin will host a conference call and webcast today, Wednesday, September 7, 2016 at 8:30 a.m. ET to discuss the DUET study results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 76903331 shortly before 8:30 a.m. ET. The webcast can be accessed at www.retrophin.com, on the Events and Presentations page within the Investors section. A replay of the call will be available starting at 11:30 a.m. ET, September 7, 2016 until 11:59 p.m. ET, September 14, 2016. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 76903331.

About the DUET Study

The DUET study is an international, randomized, double-blind, Phase 2 clinical trial assessing the safety and efficacy of

sparsentan in 109 patients with focal segmental glomerulosclerosis (FSGS), of which 96 qualified for the evaluable database. The primary endpoint is the reduction of proteinuria, as compared to irbesartan, which is part of a class of drugs used to manage FSGS in the absence of an approved pharmacologic treatment. After a two-week washout period, patients were randomized to receive daily oral doses of 200 mg, 400 mg, and 800 mg of sparsentan or 300 mg of irbesartan. After completing the eight-week treatment period, all patients were eligible to receive sparsentan as part of the study's open-label extension.

About Focal Segmental Glomerulosclerosis (FSGS)

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

About Sparsentan

Sparsentan could be the first approved pharmacologic treatment for focal segmental glomerulosclerosis, or FSGS, a rare kidney disorder that often leads to end-stage renal disease. 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.

The Phase 2 DUET study of sparsentan met the primary efficacy endpoint for the overall treatment group, demonstrating a greater than two-fold reduction of proteinuria compared to irbesartan, after the eight-week, double-blind treatment period. The Company plans to engage the FDA to determine the most expeditious path forward to advance the development of sparsentan towards approval. 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 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 pre-clinical and clinical stage pipeline. Specifically, the Company faces risk associated with top-line data which may not be confirmed upon analysis of the full data set, risk that additional clinical trials will be required for regulatory approvals, risk that additional clinical trials, if any, will fail to demonstrate that sparsentan is safe or effective and risk that the sparsentan 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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