November 19, 2016

# Retrophin Reports Additional Positive Data from Phase 2 DUET Study of Sparsentan in Focal Segmental Glomerulosclerosis at ASN Kidney Week 2016

*Significant reduction of proteinuria compared to irbesartan*

*Statistically significant difference in modified partial remission; complete remission also observed*

*Further analysis supports sparsentan generally safe and well-tolerated*

SAN DIEGO, Nov. 19, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced additional results from the Phase 2 DUET study of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney disorder without an FDA-approved pharmacologic treatment that often leads to end-stage renal disease. These new findings are being presented today in the late-breaking High-Impact Clinical Trials oral session at the American Society of Nephrology (ASN) Kidney Week 2016 in Chicago.

"The prevalence of FSGS is on the rise and without an approved therapy, many patients diagnosed with the disorder face a progressive decline and the high likelihood of end-stage renal disease," said Howard Trachtman, MD, Professor of Pediatrics; Director, Division of Pediatric Nephrology, NYU School of Medicine, NYU Langone Medical Center. "These findings from the DUET study underscore the potential of sparsentan as a first-in-class treatment for FSGS."

As announced in September, top-line data from DUET showed the sparsentan treatment group achieved statistical significance in the study's primary efficacy endpoint, reduction of proteinuria. These results showed a greater than two-fold reduction of proteinuria compared to irbesartan, after an eight-week, double-blind treatment period.

An analysis of the secondary endpoint presented today showed that a significantly greater proportion of patients receiving sparsentan achieved modified partial remission of proteinuria, compared to irbesartan-treated patients. Modified partial remission, 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 FSGS. In addition, four patients receiving sparsentan achieved complete remission, compared to zero irbesartan-treated patients. Also presented today was a post-hoc, intention-to-treat (ITT) analysis showing that the sparsentan treatment group again demonstrated a greater than two-fold reduction of proteinuria, compared to irbesartan. Further analysis of the safety database from the initial eight-week, double-blind treatment period presented today showed sparsentan was generally safe and well-tolerated.

"These new results add to the growing body of evidence from the DUET study, reinforcing our confidence that sparsentan may represent a significant advancement in the treatment of FSGS," said Stephen Aselage, chief executive officer of Retrophin. "We thank the DUET investigators for their diligence, as well as the patients and their families for their commitment to finding new and better treatment options for FSGS."

New findings from the DUET study presented at ASN Kidney Week include:

- An analysis of the secondary endpoint, which showed that after the eight-week, double-blind treatment period, 28.1 percent of patients receiving sparsentan (n=64) achieved modified partial remission of proteinuria, compared to 9.4 percent of irbesartan-treated patients (n=32, p=0.040).
- The proportion of patients achieving modified partial remission increased during the open label period. After 48 weeks of treatment with sparsentan (n=26), 57.7 percent of patients achieved modified partial remission. In addition, 50.0 percent of patients that transferred from irbesartan to sparsentan at the beginning of the open label period achieved modified partial remission after 40 weeks of treatment (n=12).
- Complete remission, defined as proteinuria less than 0.3 g/g, was achieved by four patients receiving sparsentan during the eight-week, double-blind treatment period, compared to zero irbesartan-treated patients.
- A post-hoc ITT analysis (imputing zero change in proteinuria for the 13 patients missing baseline or week 8 data) showed a statistically significant difference in the mean reduction of proteinuria from baseline for the sparsentan treatment group (n=73), compared to the irbesartan group (n=36), after the study's eight-week, double-blind treatment period. The sparsentan group achieved a 42.7 percent mean reduction of proteinuria compared to 15.7 percent for the irbesartan group (p=0.004).
- The ITT analysis also showed that after eight weeks of treatment with 400 mg and 800 mg of sparsentan (n=60), the

mean reduction of proteinuria from baseline was 44.8 percent, compared to 15.9 percent for the irbesartan-treated patients in these two cohorts (n=28, p=0.008).

- During the eight-week, double-blind period, the incidence of treatment-emergent adverse events (TEAE) for the sparsentan group was similar to the irbesartan group, except for edema. The severity of edema did not significantly worsen from baseline and no patients withdrew from the study as a result of edema during the eight-week, double-blind treatment period. The most common TEAEs in the study were headache, hypotension, dizziness, edema, nausea, diarrhea, vomiting and upper abdominal pain. The incidence of serious adverse events was similar across both groups.
- 84 percent of patients who completed the eight-week, double-blind treatment period continue to receive sparsentan in the open-label extension.

**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 primary focal segmental glomerulosclerosis (FSGS), of which 96 qualified for the evaluable efficacy 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 FDA-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 an initial eight weeks of randomized treatment, 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 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, calcineurin inhibitors, and steroids.

**About Sparsentan**

Sparsentan could be the first FDA-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 combined treatment group, demonstrating a greater than two-fold reduction of proteinuria compared to irbesartan, after the eight-week, double-blind treatment period. The Company is working with the FDA to determine the most expeditious path forward to advance the development of sparsentan towards approval. In 2015, the FDA 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, preclinical, and clinical-stage pipeline. Specifically, the Company faces the 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 statements, 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.

Contacts:

(Investors)

Chris Cline, CFA

Senior Director, Investor Relations

646-564-3680

IR@retrophin.com

(Media)

Scott Santiamo

Associate Director

Corporate Communications

718-344-5843

scott.santiamo@retrophin.com

Primary Logo

Source: Retrophin, Inc.