Retrophin Reports Additional Positive Data from Open-Label Extension of Phase 2 DUET Study of Sparsentan for the Treatment of Focal Segmental Glomerulosclerosis (FSGS)

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Progressive reduction in proteinuria and stable eGFR observed in open-label treatment period

Findings presented at ASN Kidney Week 2017

SAN DIEGO, Nov. 03, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (Nasdaq:RTRX) today announced new positive data from the ongoing open-label extension of the Phase 2 DUET study of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney disorder that often leads to end-stage renal disease, and for which no U.S. Food and Drug Administration (FDA)-approved pharmacologic treatment exists. These data were presented today during an oral session at the American Society of Nephrology (ASN) Kidney Week 2017 in New Orleans, LA.

“We are encouraged to see progressive reduction in proteinuria combined with stable eGFR in FSGS patients treated with sparsentan out to 48 weeks in the DUET open-label extension,” said Bill Rote, PhD, senior vice president and head of research and development for Retrophin. “These findings, in addition to the other supportive data presented today at ASN, underscore the potential of sparsentan to be a durable approach to treating FSGS. We look forward to building upon the data from the DUET study and initiating our Phase 3 pivotal study in the near future, providing the opportunity for us to deliver the first approved therapy for this devastating rare kidney disorder.”

As previously reported, the sparsentan treatment group achieved statistical significance in the Phase 2 DUET study’s primary efficacy endpoint, demonstrating a greater than two-fold reduction in proteinuria compared to irbesartan, after an eight-week, double-blind treatment period.

New findings from the open-label extension of the Phase 2 DUET study presented today at ASN Kidney Week 2017 include:

- Patients with FSGS who remained on sparsentan for 40 weeks during the open-label period (n=38) achieved progressive reduction in proteinuria.
    - In patients who received sparsentan as part of the original eight-week, double-blind treatment period (n=26), median urine protein-to-creatinine ratio (UP/C) was reduced from 2.7 g/g at baseline (week 0), to 0.7 g/g at week 48.
    - Patients who crossed over to sparsentan from the original irbesartan treatment group (n=12) experienced additional and sustained reduction in proteinuria during the treatment period, with median UP/C decreasing from 2.3 g/g at crossover (week 8), to 1.7 g/g at week 48.
- The observed beneficial effects of sparsentan on proteinuria were associated with stable estimated glomerular filtration rate (eGFR).
- Transition to the sparsentan treatment group from the irbesartan group led to further reduction in proteinuria and long-term stability in eGFR.
- The observed beneficial effects of treatment with sparsentan were similar after the exclusion of data from patients who received new immunosuppression therapy during the open-label extension.
- Sparsentan continued to be generally safe and well-tolerated during the 40-week open-label extension period, including in patients who transferred from the original irbesartan group.
- Seventy-four patients continue to receive treatment with sparsentan in the ongoing open-label extension of DUET.

About Focal Segmental Glomerulosclerosis

FSGS is a rare kidney 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 appears 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 in proteinuria from baseline, appears to be associated with long-term preservation of renal function in patients with FSGS. Symptoms of FSGS are currently managed with angiotensin receptor blockers, angiotensin converting enzyme inhibitors, steroids or calcineurin inhibitors.

About Sparsentan

If approved, 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 in proteinuria compared to irbesartan, after the eight-week, double-blind treatment period. Irbesartan is part of a class of drugs used to manage FSGS in the absence of an FDA-approved pharmacologic treatment. Following an End of Phase 2 meeting with the FDA in 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 eGFR, which is widely regarded as the best overall measure of kidney function. The Company expects to confirm the Phase 3 protocol with the FDA in the second half of 2017, with the pivotal trial expected to initiate thereafter.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research exploring additional rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal®, Cholbam® and Thiola®.

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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 the risk that favorable results seen in the sparsentan Phase 2 DUET study's open-label extension to date will not continue or be replicated in the future, 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 associated with enrollment of clinical trials for rare diseases and risk the clinical trial 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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