Retrophin Reports Positive Long-Term Data from Open-Label Extension of Phase 2 DUET Study of Sparsentan for the Treatment of Focal Segmental Glomerulosclerosis

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Increasing achievement of FSGS partial remission of proteinuria and stable eGFR observed out to 84 weeks in open-label extension

Findings presented at ASN Kidney Week 2018

SAN DIEGO, Oct. 26, 2018 (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 (ESRD). These data were presented today during an oral session at the American Society of Nephrology (ASN) Kidney Week 2018, being held October 23–28, 2018, in San Diego, CA.

“The open-label extension of the DUET Study continues to provide compelling data that suggests sparsentan has the potential to provide long-term nephroprotective benefits for people living with FSGS,” said Noah Rosenberg, M.D., chief medical officer of Retrophin. “We are highly encouraged to see an increasing proportion of patients reach the FSGS partial remission of proteinuria endpoint, as well as a stabilization of estimated glomerular filtration rate after receiving sparsentan for up to 84 weeks. These data increase our confidence in the sparsentan FSGS development program and the potential for the ongoing Phase 3 DUPLEX Study to provide support for sparsentan to become the first approved therapy for people living with FSGS.”

As previously reported, the overall sparsentan treatment group demonstrated a greater than two-fold reduction in proteinuria compared to the irbesartan treatment group, after an eight-week, double-blind treatment period in the Phase 2 DUET Study. Additional results showing that patients with FSGS who remained on sparsentan for 40 weeks during the open-label extension period of DUET achieved progressive reduction in proteinuria and stable estimated glomerular filtration rate (eGFR), were reported at ASN Kidney Week 2017.

New findings from the open-label extension of the Phase 2 DUET Study presented at ASN Kidney Week 2018 include:

- Patients with FSGS who remained on sparsentan for 76 weeks during the open-label period (n=71) achieved additional progressive reduction of proteinuria.
    - In patients who received sparsentan as part of the original eight-week, double-blind treatment period (n=45), median urine protein-to-creatinine ratio (UP/C) was reduced from 2.8 g/g at baseline (week 0) to 0.9 g/g at week 84.
    - Patients who crossed over to sparsentan from the original irbesartan control group (n=26) experienced additional and sustained reduction in proteinuria during the treatment period, with median UP/C decreasing from 2.3 g/g at crossover (week eight) to 1.1 g/g at week 84.
- An increasing proportion of patients achieved the FSGS partial remission of proteinuria endpoint (FPRE), defined as UP/C: ≤1.5 g/g and >40 percent reduction of proteinuria from baseline, with ongoing sparsentan treatment in the open-label extension.
    - In patients who received sparsentan as part of the original eight-week, double-blind treatment period (n=45), the proportion of patients who achieved FPRE increased from 28 percent at week eight to 60 percent at week 84.
    - The proportion of patients who crossed over to sparsentan from the original irbesartan control group (n=26) and achieved FPRE increased from nine percent at week eight to 50 percent at week 84.
- Treatment with sparsentan in the open-label extension was associated with a stabilization of eGFR out to week 84.
- The observed beneficial effects of sparsentan on proteinuria were associated with a sustained reduction in mean systolic and diastolic blood pressure.
- Sparsentan continued to be generally well-tolerated during the open-label extension period.
- Sixty-two 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 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 ESRD. 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 ESRD. Achieving FPRE 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

Sparsentan is an investigational product candidate that has a dual mechanism of action that combines angiotensin receptor blockade with endothelin

receptor type A blockade. Retrophin is developing sparsentan for the treatment of FSGS, as well as for IgA nephropathy (IgAN), a rare kidney disorder that also often leads to ESRD. In several forms of chronic kidney disease, such as FSGS and IgAN, 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 in FSGS met its 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 and IgAN in the absence of an approved pharmacologic treatment. In April 2018, Retrophin initiated the pivotal Phase 3 DUPLEX Study of sparsentan for the treatment of FSGS. The study includes an interim efficacy endpoint based on proteinuria to serve as the basis for a New Drug Application (NDA) filing for Subpart H accelerated approval of sparsentan in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe. In addition, Retrophin expects to initiate the pivotal Phase 3 PROTECT Study evaluating the safety and efficacy of sparsentan for the treatment of IgAN during the fourth quarter of 2018. If approved, sparsentan could potentially be the first approved pharmacologic treatment for FSGS and IgAN.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare disease. 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) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. 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 Phase 3 clinical trial of sparsentan in FSGS will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the planned Phase 3 clinical trial of sparsentan in IgAN 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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