Retrophin Announces Publication of Phase 2 DUET Study of Sparsentan for the Treatment of Focal Segmental Glomerulosclerosis in the Journal of the American Society of Nephrology

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DUET publication highlighted during Best of ASN Journals session at ASN Kidney Week 2018

SAN DIEGO, Oct. 25, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the Journal of the American Society of Nephrology (JASN) has published online (doi: 10.1681/ASN.2018010091) the positive results from 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). As previously reported, these results demonstrated that the sparsentan treatment group experienced a greater than two-fold reduction of proteinuria compared to the irbesartan treatment group after an eight-week, double-blind treatment period. The publication is being highlighted as part of the Best of ASN Journals session at ASN Kidney Week 2018 and will also appear in the November 2018 print issue of JASN.

“Patients with FSGS face a significant unmet need caused by a progressive decline in kidney function, which often requires a kidney transplant or dialysis,” said principal investigator Howard Trachtman, M.D., Division of Pediatric Nephrology, Department of Pediatrics at NYU School of Medicine. “The results from the Phase 2 DUET Study suggest that sparsentan has the ability to reduce proteinuria, an independent predictor of renal survival in patients with primary FSGS, significantly more than angiotensin receptor blockers currently used to manage FSGS. Furthermore, the greater proportion of sparsentan-treated patients that achieved the FSGS partial remission of proteinuria endpoint in the study offers promise for longer-term outcomes and potential clinically meaningful benefits for patients.”

Stephen Aselage, chief executive officer of Retrophin commented, “The DUET data provided compelling clinical support for the advancement of sparsentan in FSGS. We continue to make progress with our program, including the initiation of our pivotal Phase 3 DUPLEX Study in FSGS earlier this year. The data from DUET also provided a platform to evaluate the potential benefit of sparsentan for other patients affected by rare glomerular disease, including IgA nephropathy, for which we plan to start the pivotal Phase 3 PROTECT Study in the fourth quarter of this year.”

In the randomized, double-blind, controlled Phase 2 DUET Study, the overall sparsentan treatment group achieved statistical significance in the primary efficacy endpoint, demonstrating a greater than two-fold reduction in proteinuria compared to the irbesartan treatment group, after an eight-week, double-blind treatment period. Treatment with sparsentan resulted in greater reductions in urinary protein-to-creatinine ratio (UP/C) compared with irbesartan when all dose cohorts (44.8 percent versus 18.5 percent; p=0.006) were combined or when the 400 and 800 mg dose cohorts (47.4 percent vs. 19.0 percent; p=0.011) were combined.

An analysis of the secondary endpoint of the study showed that during the eight-week, double-blind treatment period, a significantly greater proportion of patients receiving sparsentan (28.1 percent) 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, compared to irbesartan-treated patients (9.4 percent; p=0.040).

In addition, data from patients who were followed out to 48 weeks in the open-label sparsentan treatment period of DUET demonstrated a steady rise in the percentage of patients who achieved FPRE, reaching approximately 60 percent in patients originally randomized to either sparsentan or irbesartan.

The data also showed sparsentan was generally well-tolerated during the eight-week, double-blind period, with the overall incidences of treatment-emergent adverse events (TEAEs), drug-related TEAEs, or serious TEAEs similar between the sparsentan and irbesartan groups.

New data from an 84-week analysis of the open-label portion of the DUET Study will be presented at ASN Kidney Week 2018 during the Glomerular Diseases: Clinical, Outcomes, and Trials session on Friday, October 26, 2018, at 6:18 p.m. PT.

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 U.S. Food and Drug Administration 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 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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