Retrophin to Present Additional Data from Phase 2 DUET Study of Sparsentan in Late-Breaking Oral Session at ASN Kidney Week 2016

October 21, 2016

SAN DIEGO, Oct. 21, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (Nasdaq:RTRX) today announced that additional data from the Phase 2 DUET study of sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS) will be presented at the late-breaking High-Impact Clinical Trials oral session at the American Society of Nephrology (ASN) Kidney Week 2016. Positive top-line results from the DUET study were announced in September 2016. The Company and its collaborators will also present further supportive data from the sparsentan program, as well as observational cohort data characterizing the association between proteinuria reduction and improved clinical outcomes in FSGS patients. ASN Kidney Week 2016 is being held November 15-20 in Chicago.

Oral Presentations

Efficacy and Safety of Sparsentan, a Dual Angiotensin II (Ang II) and Endothelin (ET) Type A Receptor Antagonist, in Patients with Focal Segmental Glomerulosclerosis (FSGS): A Phase 2 Trial (DUET)
Abstract #6478
Session: High-Impact Clinical Trials
Room W375C-E
Saturday, November 19, 2016
12:10 p.m. CT

A Clinical Outcome Assessment of Proteinuria in Patients with Focal Segmental Glomerulosclerosis
Abstract #1016
Session: The Leaky Membrane: Nephrotic Syndrome
Room S106
Friday, November 18, 2016
5:30 p.m. CT

Poster Presentations

Renal Pharmacology and Preclinical Attributes of Sparsentan, a Dually Active Endothelin A and Angiotensin 1 Receptor Antagonist
Abstract #5728
Session: Pathologic Mechanisms
Poster #TH-PO166
Thursday, November 17, 2016
10:00 a.m. - 12:00 p.m. CT

Pharmacokinetics of Sparsentan in Healthy Subjects: In Vitro Metabolism and Effects of Food, Gender, Age, and Multiple-Dose Escalation
Abstract #5751
Session: Pharmaco: Kinetics, Dynamics, Genomics
Poster #SA-PO507
Saturday, November 19, 2016
10:00 a.m. - 12:00 p.m. CT

Antihypertensive Effects of Sparsentan, a Dual Angiotensin II and Endothelin Type A Receptor Antagonist
Abstract #5712
Session: Hypertension: Clinical
Poster #SA-PO685
Saturday, November 19, 2016
10:00 a.m. - 12:00 p.m. CT

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 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 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 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.

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