



Retrophin to Present Long-Term Data from Phase 2 DUET Study of Sparsentan in FSGS at ASN Kidney Week 2018

October 5, 2018

SAN DIEGO, Oct. 05, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that it will present new data examining the long-term effects of sparsentan in focal segmental glomerulosclerosis (FSGS), at the American Society of Nephrology (ASN) Kidney Week 2018. The Company will present an 84-week analysis of the open-label extension portion of the Phase 2 DUET Study, as well as preclinical findings demonstrating that treatment with sparsentan provides nephroprotection in an autosomal mouse model of Alport syndrome. FSGS and Alport Syndrome are rare, progressive kidney disorders that often lead to end-stage renal disease (ESRD). ASN Kidney Week 2018 is being held October 23–28, 2018, in San Diego, CA.

Oral Presentation:

Long-term Effects of Sparsentan, a Dual Angiotensin and Endothelin Receptor Antagonist in Primary Focal Segmental Glomerulosclerosis (FSGS): Interim 84-Week Analysis of the DUET Trial

Abstract Program #: FR-OR087

Session: Glomerular Diseases: Clinical, Outcomes, and Trials

Location: Room 24A, San Diego Convention Center

Date & Time: Friday, October 26, 2018, 6:18 p.m. – 6:30 p.m. PT

Poster Presentation:

Sparsentan, a Dual Angiotensin II Type 1 (AT1) and Endothelin Type A (ETA) Receptor Antagonist, Prevents Renal Disease in COL4A3 -/- Autosomal Alport Mice

Poster #: FR-PO624

Session: Genetic Diseases of the Kidney Non-Cystic II

Location: Exhibit Hall C, San Diego Convention Center

Date & Time: Friday, October 26, 2018, 10:00 a.m. – 12:00 p.m. PT

About Sparsentan

Sparsentan's dual mechanism of action combines angiotensin receptor blockade with endothelin receptor type A blockade. Retrophin is developing sparsentan for the treatment of FSGS, as well as for the treatment of 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 (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 FDA-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 FDA-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 fosmetopantotenate 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[®].

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. 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 risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing therapies. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that current or future clinical trials will not proceed as planned. Specifically, the Company faces the 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 the basis for accelerated approval of sparsentan as planned; and risk that the Company's product candidates will not be approved for efficacy, safety, regulatory or other reasons, and for each of the programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials 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 exclusivity periods 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.

Contact:
Chris Cline, CFA
Vice President, Investor Relations & Corporate Communications
646-564-3680
IR@retrophin.com



Source: Retrophin, Inc.