Retrophin Announces Upcoming Presentations at the 15th International Symposium on IgA Nephropathy

September 27, 2018

SAN DIEGO, Sept. 27, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced two upcoming presentations related to its development program for sparsentan in the treatment of IgA nephropathy (IgAN), a rare kidney disorder often resulting in end-stage renal disease, during the 15th International Symposium on IgA Nephropathy (IIgANN 2018). IIgANN 2018 is being held September 27–29, 2018, in Buenos Aires, Argentina.

Oral Presentations:

PROTECT in Immunoglobulin A Nephropathy (IgAN): Study Design of a Phase 3, Randomized, Double-blind, International, Active-controlled Study of the Efficacy and Safety of Sparsentan
Abstract #: 0055
Location: Ballroom Cabildo
Saturday, September 29, 2018
11:00 a.m. ART

The PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan for the treatment of IgAN. Approximately 280 patients with primary IgAN, aged 18 years or older, are expected to be randomized to receive either sparsentan (initial dose of 200 mg daily for two weeks, titrating up to a target dose of 400 mg daily) or an active control, irbesartan (initial dose of 150 mg daily for two weeks, titrating up to a target dose of 300 mg daily). The primary efficacy endpoint is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. Retrophin expects that successful achievement of this endpoint will serve as the basis for Subpart H accelerated approval of sparsentan in the United States and Conditional Marketing Authorization (CMA) consideration in Europe. Secondary efficacy endpoints include change in estimated glomerular filtration rate (eGFR) from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Retrophin expects to initiate the PROTECT Study during the fourth quarter of 2018.

Effect of Sparsentan, a Dual Angiotensin II Type 1 (AT1) and Endothelin Type A (ETA) Receptor Antagonist, in the Rat anti-Thy1 Model of Glomerulonephritis
Abstract #: 0054
Location: Ballroom Cabildo
Friday, September 28, 2018
4:35 p.m. ART

In a preclinical study of glomerulonephritis in an anti-Thy1 rodent model that shares structural and pathophysiologic features with IgAN, sparsentan was shown to have dose-dependent reductions in inflammation, mesangial cell activation, interstitial myofibroblast activation, glomerular injury, mesangial cell proliferation, macrophage infiltration and proteinuria.

About IgA Nephropathy

IgA nephropathy (IgAN), also known as Berger's disease, is a rare chronic kidney disorder in which an estimated 20 to 40 percent of patients progress to end-stage renal disease (ESRD) within 10 to 20 years of diagnosis. IgAN is often defined by progressive proteinuria, hematuria and acute onset of nephrotic syndrome. The disorder is estimated to affect more than 100,000 people in the U.S. and is one of the leading causes of acute nephritis in Europe and Japan.

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 IgAN, as well as for the treatment of focal segmental glomerulosclerosis (FSGS), a rare kidney disorder that also often leads to ESRD. In several forms of chronic kidney disease, such as IgAN and FSGS, 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 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®.

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; risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing 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
760-260-8600
IR@retrophin.com

Source: Retrophin, Inc.