



NEWS RELEASE

Retrophin Announces Enrollment of First 190 Patients in Pivotal Phase 3 DUPLEX Study of Sparsentan in Focal Segmental Glomerulosclerosis

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Topline data from 36-week proteinuria endpoint anticipated in first quarter of 2021

SAN DIEGO, March 09, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the first 190 patients have been enrolled in the pivotal Phase 3 DUPLEX Study evaluating the safety and efficacy of sparsentan in focal segmental glomerulosclerosis (FSGS). The DUPLEX Study protocol provides for a pre-specified interim analysis to evaluate the proteinuria efficacy endpoint in the first 190 patients after 36 weeks of treatment. Successful achievement of this 36-week proteinuria endpoint is expected to serve as the basis for submission of filings for accelerated approval in the U.S. and Europe. Topline efficacy data from the 36-week proteinuria endpoint analysis are expected in the first quarter of 2021.

"Many people living with FSGS face a progression to kidney failure with no FDA or EMA approved medicine indicated for their condition," said Noah Rosenberg, M.D., chief medical officer of Retrophin. "Our continued collaboration with investigators and the FSGS community has generated recent momentum in the DUPLEX Study. Achieving 190 patients enrolled allows us to update the timing for topline data from the 36-week proteinuria analysis to the first quarter of next year, and brings us one step closer to realizing our goal for sparsentan to potentially shape the treatment paradigm for FSGS, if approved."

DUPLEX is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial expected to enroll approximately 300 patients with FSGS. Patients are randomized to receive either sparsentan or irbesartan, the active control. The proteinuria efficacy endpoint is the proportion of patients achieving FSGS partial remission of proteinuria (FPRE), which is defined as urine protein-to-creatinine ratio (Up/C) ≤ 1.5 g/g and a >40

percent reduction in Up/C from baseline, at Week 36. The confirmatory endpoint of the study is the slope of estimated glomerular filtration rate (eGFR) from Week 6 to Week 108, in approximately 300 patients.

About Focal Segmental Glomerulosclerosis

FSGS is a rare kidney disorder 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 (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 FPPE 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 in Phase 3 clinical development that has a dual mechanism of action combining endothelin receptor type A blockade with angiotensin receptor blockade. Retrophin is developing sparsentan for the treatment of FSGS, as well as for IgA nephropathy (IgAN), rare kidney disorders that often lead 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.

Retrophin is currently enrolling the pivotal Phase 3 DUPLEX Study of sparsentan for the treatment of FSGS (FSGSduplex.com), as well as the pivotal Phase 3 PROTECT Study of sparsentan for the treatment of IgAN (IgANprotect.com). Both studies contain 36-week proteinuria-based endpoints, which if achieved, are expected to serve as the basis for submission of a New Drug Application (NDA) under the Subpart H accelerated approval pathway in the U.S. as well as an application for Conditional Marketing Authorization (CMA) consideration in Europe. If approved, sparsentan could potentially be the first medicine approved 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 sparsentan, a

product candidate in late-stage development for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), rare disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including partnerships with leaders in patient advocacy and government research to identify potential therapeutics for NGLY1 deficiency and Alagille syndrome, conditions with no approved treatment options. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal[®], Cholbam[®], Thiola[®] and Thiola EC[®].

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 commercial 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 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 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 for each of its development programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing clinical trials may not proceed on expected timelines or may be delayed for safety, regulatory or other reasons and risk that the product candidates will not be approved for efficacy, 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; risks associated with regulatory interactions; and risks and uncertainties relating to competitive products, including potential generic competition

with certain of the Company's 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-Q, Form 10-K and other filings with the Securities and Exchange Commission.

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