Retrophin Announces Enrollment of First 280 Patients in Pivotal Phase 3 PROTECT Study of Sparsentan in IgA Nephropathy

September 21, 2020

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

SAN DIEGO, Sept. 21, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the first 280 patients have been enrolled in the pivotal Phase 3 PROTECT Study evaluating the safety and efficacy of sparsentan in IgA nephropathy (IgAN). The PROTECT Study protocol provides for a pre-specified analysis to evaluate the proteinuria efficacy endpoint in at least 280 patients after 36 weeks of treatment. Successful achievement of the 36-week proteinuria endpoint is expected to support regulatory submissions for accelerated approval in the U.S. and Europe. Topline efficacy data from the 36-week proteinuria endpoint analysis are expected in the third quarter of 2021.

“With a lack of effective and durable treatment options available for people living with IgAN, many patients progress to end-stage renal disease requiring kidney transplant or dialysis,” said Noah Rosenberg, M.D., chief medical officer of Retrophin. “We believe the continued progress of our PROTECT Study reflects the unwavering dedication of patients and investigators to support the development of treatment options that could have the potential to delay transplant or dialysis. As a result of achieving this important enrollment milestone in the PROTECT Study ahead of schedule, we are positioned to report top-line data next year from both the PROTECT Study and our pivotal DUPLEX Study in FSGS.”

PROTECT is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial expected to enroll approximately 380 patients with IgAN. Patients are randomized to receive either sparsentan or irbesartan, the active control. The proteinuria efficacy endpoint is the change in proteinuria (urine protein-to-creatinine ratio) at Week 36 compared to baseline. Secondary efficacy endpoints include the rate of change in eGFR from the initiation of randomized treatment over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients.

About IgA Nephropathy

IgA nephropathy (IgAN), also called Berger's disease, is a rare kidney disorder characterized by the buildup of immunoglobulin A (IgA), a protein that helps the body fight infections, in the kidneys. The deposits of IgA cause a breakdown of the normal filtering mechanisms in the kidney, leading to blood in the urine (hematuria), and protein in the urine (proteinuria). Other symptoms of IgAN may include kidney pain, swelling (edema) and high blood pressure.

IgAN is the most prevalent primary chronic glomerular disease worldwide and a leading cause of end-stage renal disease (ESRD). IgAN 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. There are currently no approved treatments indicated for IgAN.

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 IgAN, as well as for focal segmental glomerulosclerosis (FSGS), rare kidney disorders that often lead 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 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 support 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 for both indications, 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 include, but are not limited to, references to the Company’s current expectations around timelines for reporting top-line data from the

proteinuria endpoints in the DUPLEX and PROTECT studies, expectations regarding potential regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe and the potential future regulatory approval of sparsentan for FSGS and IgAN. 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. The Company faces additional risks associated with the potential impacts the COVID-19 pandemic may have on its business, including, but not limited to (i) the Company’s ability to continue its ongoing development activities and clinical trials, (ii) the timing of such clinical trials and the release of data from those trials, (iii) the Company’s and its suppliers’ ability to successfully manufacture its commercial products and product candidates, and (iv) the market for and sales of its commercial 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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Source: Retrophin, Inc.