Retrophin Announces Expansion of Intellectual Property Estate for Sparsentan with Newly Issued U.S. Patent

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SAN DIEGO, June 14, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that the United States Patent and Trademark Office (USPTO) has issued a new patent covering the Company's product candidate sparsentan which, in addition to being in Phase 3 clinical development for the treatment of focal segmental glomerulosclerosis (FSGS), is advancing to Phase 3 clinical development for the treatment of IgA nephropathy (IgAN). U.S. Patent No. 9,993,461 expands the Company's current intellectual property by providing coverage for the use of sparsentan in the treatment of IgAN and broadening the existing coverage to include all doses of sparsentan between 200 and 800 mg/day. The patent has a stated expiration date of March 30, 2030.

"We are very pleased to broaden the intellectual property coverage for sparsentan with this newly issued patent in the United States," said Stephen Aselage, chief executive officer of Retrophin. "As we advance our development efforts, this patent will help us position sparsentan as a first-in-class therapy for people living with FSGS, as well as those living with IgA nephropathy, and further support our efforts to deliver a novel treatment option for these rare disorders with significant unmet needs."

About Sparsentan

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.

In April 2018, Retrophin initiated the pivotal Phase 3 DUPLEX Study of sparsentan for the treatment of FSGS, a disease with no U.S. Food and Drug Administration (FDA)-approved pharmacological therapies. 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 FSGS. Sparsentan has been granted orphan drug designation for the treatment of FSGS by the FDA and European Commission.

The Company is also advancing the development of sparsentan in IgA nephropathy, a rare immune complex mediated glomerulonephritis characterized by proteinuria and variable rates of progressive renal failure. Study start-up activities are underway in anticipation of initiating a pivotal study in the fourth quarter of 2018.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. 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 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 proceed as planned or will not demonstrate that sparsentan is safe or effective or serve as the basis for an NDA filing as planned; and more generally, 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, may not progress as expected, 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-Q, Form 10-K and other filings with the Securities and Exchange Commission.

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