



Retrophin Receives European Orphan Drug Designation for Sparsentan for the Treatment of Focal Segmental Glomerulosclerosis

November 16, 2015

SAN DIEGO, Nov. 16, 2015 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that the European Commission (EC) has granted sparsentan orphan drug designation for the treatment of focal segmental glomerulosclerosis (FSGS). Retrophin previously announced in January 2015 that sparsentan received orphan drug designation from the U.S. Food and Drug Administration (FDA) for the treatment of FSGS. Sparsentan is the Company's investigational therapeutic agent which acts as both a potent angiotensin receptor blocker, as well as a selective endothelin receptor antagonist, and is currently being evaluated in the Phase 2 DUET trial in FSGS patients. FSGS is a widely recognized cause of end-stage renal disease and there are no approved pharmacologic treatments currently available in the United States or Europe.

"Obtaining orphan designation for sparsentan in the European Union is an important milestone for both Retrophin and the FSGS patient community," said Alvin Shih, M.D., Executive Vice President and Head of Research & Development for Retrophin. "There is a clear unmet medical need in FSGS. As part of our ongoing commitment to developing treatment options for patients suffering from rare diseases like FSGS, we look forward to completing the DUET trial and reading out results in 2016."

Orphan drug designation from the EC provides regulatory and financial incentives for companies to develop and market therapies that treat life-threatening or very serious conditions that affect no more than five in 10,000 people in the European Union (EU), and where no treatment is currently approved. In addition to a 10-year period of marketing exclusivity in the EU upon product approval, orphan drug designation provides fee waivers, protocol assistance, and marketing authorization under the centralized procedure granting approval in all EU countries.

About Sparsentan

Sparsentan, also known as RE-021, is an investigational therapeutic agent which acts as both a potent angiotensin receptor blocker, as well as a selective endothelin receptor antagonist, with in vitro selectivity toward endothelin receptor type A. Sparsentan is being developed as a treatment for FSGS, which is a widely recognized cause of end-stage renal disease. Retrophin is currently enrolling patients for the DUET Phase 2 clinical study of sparsentan for the treatment of FSGS, which may be able to support an application for accelerated approval of sparsentan on the basis of proteinuria as a surrogate endpoint.

About Retrophin

Retrophin is a pharmaceutical company focused on the development, acquisition and commercialization of drugs for the treatment of serious, catastrophic or rare diseases for which there are currently no viable options for patients. The Company's approved products include Chenodal[®], Cholbam[®], and Thiola[®], and its pipeline includes compounds for several catastrophic diseases, including focal segmental glomerulosclerosis (FSGS), pantothenate kinase-associated neurodegeneration (PKAN), nephrotic syndrome and others. For additional information, please visit www.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. 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, as well as risks and uncertainties associated with the Company's research pre-clinical and clinical stage pipeline. Specifically, the Company faces risk that the sparsentan Phase 2 clinical trials will fail to demonstrate that sparsentan is safe or effective and risk that the sparsentan Phase 2 program will be delayed for regulatory or other reasons. 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 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 filings with the Securities and Exchange Commission.

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Source: Retrophin, Inc.

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