Retrophin Receives FDA Orphan Drug Designation for RE-024 for the Treatment of Pantothenate Kinase-Associated Neurodegeneration

May 5, 2015

SAN DIEGO--(BUSINESS WIRE)-- Retrophin, Inc. (NASDAQ:RTRX) today announced RE-024, the Company's investigational phosphopantothenate replacement therapy, has been granted orphan drug designation by the U.S. Food and Drug Administration (FDA) for the treatment of pantothenate kinase-associated neurodegeneration (PKAN). PKAN is a rare and lethal autosomal recessive neurodegenerative disorder believed to affect approximately one to three persons per million worldwide, with no approved treatment currently available. Retrophin has initiated the screening of healthy volunteers for its recently announced Phase 1 trial evaluating the safety and tolerability of single oral doses of RE-024.

The Orphan Drug Designation program is intended to encourage companies to develop therapeutics for diseases that affect fewer than 200,000 individuals in the United States. Orphan designation will provide RE-024 with seven years of marketing exclusivity if approved by the FDA for the treatment of PKAN. Prior to FDA approval, orphan designation provides incentives for sponsors including tax credits for clinical research expenses, the opportunity to obtain government grant funding to support clinical research, and an exemption from FDA user fees.

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), infantile spasms, 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, regarding the research, development and commercialization of pharmaceutical products. 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, as well as risks and uncertainties associated with the Company's pre-clinical and clinical stage pipeline as well as its sales and marketing strategies. Specifically, the risks and uncertainties the Company faces with respect to its RE-024 program include risk that the Company will be unable to initiate and/or complete Phase 1 clinical trials, risk that RE-024 will not progress to Phase 2 clinical trials for safety, regulatory or other reasons; risk associated with enrollment of clinical trials for rare diseases; risk that the company's later stage RE-024 clinical studies will fail to demonstrate that RE-024 is safe and effective. The Company also faces risk that it will be unable to raise additional funding 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 intellectual property rights of third parties; 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 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.