Retrophin Reports Positive Survival Data from Preclinical Trial of RE-024 for the Treatment of the Ultra-Orphan Disease Pantothenate Kinase-Associated Neurodegeneration (PKAN)

August 16, 2013

Company to Hold Update Conference Call Today, August 16, 2013 at 10:00 a.m. ET

NEW YORK--(BUSINESS WIRE)-- Retrophin, Inc. (OTCQB: RTRX) today announced that it has received positive survival results from interim preclinical tests for the Company's compound, RE-024, for the treatment of the ultra-orphan disease Pantothenate Kinase-Associated Neurodegeneration (PKAN). RE-024 is a replacement therapy for phosphopantothenate, the substrate that is missing in patients with PKAN. Tests were conducted on mice that were administered a PANK inhibitor to induce a PKAN-like phenotype.

The interim results of this two-week study assessed the effect of RE-024 in mice dosed with RE-024 and Hopantenate (HoPan), a pantothenate kinase inhibitor that simulates PKAN by inhibiting PANK, compared to mice dosed with HoPan alone. Data from the study showed that 92.5% (37/40) of the mice dosed with HoPan and RE-024 survived the experiment, while no (0/14) mice dosed with HoPan alone were alive at the end of the study period (p &#60 0.0001).

PKAN is a rare and life-threatening neurological disorder caused by a mutation in the PANK2 gene, which prevents patients from being able to properly metabolize vitamin B5 (pantothenate) into phosphopantothenate. The disruption of this metabolic pathway ultimately leads to decreased levels of Coenzyme A (CoA) and iron accumulation in the brain. As a result, patients present with dystonia (sustained muscle contraction leading to abnormal posture), rigidity, dysphagia (problems swallowing), weakness, pigmentary retinopathy (visual impairment), tremors, as well as a number of other symptoms. Onset of PKAN typically occurs prior to the age of 10 and has an estimated prevalence of 5,000-10,000 patients worldwide. Many patients die within 10 years of being diagnosed.

"We are proud of what we have accomplished with this program in a short period of time," said Martin Shkreli, Founder, President and Chief Executive Officer of Retrophin. "When our team read about the plight of PKAN patients, we moved quickly to develop a series of phosphopantothenate analogs that we believed would rescue the phenotype of patients suffering from this horrific, catastrophic disease. Today I am so proud of our team for its dedication to these patients. We expect that we will be able to start a human study in first-quarter 2014."

Suzanne Jackowski, PhD, a member of the faculty at St. Jude Children's Research Hospital and a leading expert in Coenzyme A metabolism, commented, "I am encouraged by the results of this most recent experiment with RE-024. The positive preclinical survival data generated by this study serve to further confirm our own proof-of-concept research, conducted earlier this year, pointing to RE-024 as a potential promising new approach to treating this debilitating and life-threatening disease."

Company to Host Conference Call

Management will host a corporate update conference call and audio webcast on Friday, August 16, 2013 at 10:00 a.m. ET. To participate in the conference call, dial 866-953-6857 (U.S.) or 617-399-3481 (International), confirmation code 95270920 shortly before 10:00 a.m. ET. The audio webcast can be accessed at: www.retrophin.com, in the Investor Relations section. A replay of the call will be available from 12:00 p.m. ET on Friday August 16, 2013, through 11:59 p.m. ET on Friday, August 23. The replay number is 888-286-8010 (U.S.) or 617-801-6888 (International), confirmation code 74457089.

About Retrophin

Retrophin is a pharmaceutical company focused on the discovery and development of drugs for the treatment of debilitating and often life-threatening diseases for which there are currently no viable patient options. The Company is currently focused on several catastrophic diseases affecting children, including Focal Segmental Glomerulosclerosis (FSGS), Pantothenate Kinase-Associated Neurodegeneration (PKAN), Duchenne Muscular Dystrophy and others. Retrophin's lead compound, RE-021, is scheduled to begin enrollment in a potentially pivotal Phase 2 clinical trial for FSGS during 2013. 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. 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. Forward-looking statements in the press release should be evaluated together with the many uncertainties that affect the Company's business. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise.

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