Retrophin Initiates Global Access for RE-024 in Pantothenate Kinase-Associated Neurodegeneration (PKAN)

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First Patient Scheduled to Be Dosed Outside the U.S. This Week

NEW YORK--(BUSINESS WIRE)-- Retrophin, Inc. (NASDAQ:RTRX) today announced that it has made RE-024 available to physicians who are treating critically ill patients with pantothenate kinase-associated neurodegeneration (PKAN) worldwide under local "compassionate use" regulations. RE-024 is a replacement therapy for phosphopantothenate, the reaction product and substrate that is missing in patients with PKAN.

A European health regulator approved the initiation of dosing RE-024 in PKAN under a named patient program late last week. These patients will be the first humans to receive RE-024. Retrophin expects that up to 15 patients will receive the drug as their physicians receive permission to dose in countries across the world. Retrophin is working closely with the U.S. Food and Drug Administration to enable as rapid access to RE-024 in the United States as possible.

Retrophin's Founder and Chief Executive Officer Martin Shkreli noted, "PKAN is a deadly disease that has never received attention from pharmaceutical companies. We are proud to be part of this important step forward for patients and their families. We are eager to learn how patients taking RE-024 fare as we move towards a placebo-controlled double-blind study."

About PKAN

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 from PKAN within 10 years of being diagnosed.

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 marketed products include Chenodal® and Vecamyl®, and its pipeline includes compounds for several catastrophic diseases, including focal segmental glomerulosclerosis (FSGS), pantothenate kinase-associated neurodegeneration (PKAN), schizophrenia, autism, infantile spasms, nephrotic syndrome and others. Retrophin intends to reintroduce Syntocinon Nasal Spray in the U.S. to assist initial postpartum milk ejection. 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. Forward-looking statements in the press release should be evaluated together with the many uncertainties that affect the Company's business. 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 filings with the Securities and Exchange Commission.

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