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Retrophin Announces Positive Results from Preclinical Studies of RE-024 for PKAN

New York, NY (March 11, 2013) – Retrophin, Inc. (OTCQB: RTRX), a biotechnology company focused on the discovery and development of orphan drugs for the treatment of rare and life-threatening diseases for which there are currently no viable patient options, today announced positive results from a series of in vitro and in vivo experiments with RE-024. RE-024 rescued the phenotype of pantothenate kinase associated neurodegeneration (PKAN) in vitro and in vivo, demonstrating successful replacement therapy proof-of-concept.

“We are delighted with the outcome of the experiments conducted by St. Jude Children’s Research Hospital, which assessed RE-024 in a broad array of customized assays in PKAN,” said Martin Shkreli, founder and chief executive officer of Retrophin. “The promising results we’ve seen to date are a testament to our chemistry team’s design of RE-024. On the basis of these results, we are accelerating the timeline for filing of an IND for RE-024.”

PKAN is an inherited, progressive and fatal neurodegenerative disease. Symptoms of PKAN vary but often include ataxia, dystonia, and a general failure to thrive. Onset usually occurs before 10 years of age and typically results in premature death. While the exact incidence of PKAN is uncertain, it is estimated to affect one to three per million people worldwide. There is currently no FDA approved treatment for PKAN. [1, 2]

Full results of the preclinical studies of RE-024 will be presented at an upcoming scientific meeting.

About Retrophin

Retrophin is a biotechnology company focused on the discovery and development of orphan drugs for the treatment of rare and 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 the first half of 2013.

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.

1 National Institute of Neurological Disorders and Stroke: http://www.ninds.nih.gov/disorders/nbia/nbia.htm
2 Genetics Home Reference: http://ghr.nlm.nih.gov/condition/pantothenate-kinase-associatedneurodegeneration

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