

April 8, 2013

# **Retrophin Sponsors Seventh International NBIA Disorders Association Family Conference**

## **Overview of RE-024 for PKAN presented**

**New York, NY (April 8, 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 that it sponsored the Seventh International NBIA Disorders Association Family Conference, which was held April 4-7 in San Antonio, TX.

"We are proud to have been part of this important conference that brought together patients with neurodegeneration with brain iron accumulation disorders, their families, and the medical community," said Martin Shkreli, founder and chief executive officer of Retrophin. "It was a wonderful opportunity for us to spend time with children who have PKAN, a devastating ultra-orphan pediatric disease for which our preclinical studies of RE-024 have shown positive results. We look forward to presenting detailed findings at an upcoming scientific meeting."

On Saturday, April 6, Suzanne Jackowski, PhD, principal investigator of the preclinical studies, presented an overview of RE-024 for PKAN. The presentation is available at <u>www.retrophin.com</u>.

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. <sup>1, 2</sup>

### **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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