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Retrophin and St. Jude Children's Research Hospital to Collaborate on Rare Disease Research Program

Program led by Dr. Suzanne Jackowski will test Retrophin replacement therapy library for treatment of PKAN, a rare neurodegenerative disease

New York (July 16, 2012) – Retrophin, LLC, a privately-held New York-based, biotechnology company focused on discovering and developing treatments for rare and life-threatening diseases, today announced that it has entered a sponsored research agreement with St. Jude Children's Research Hospital. Retrophin will supply funding and a library of replacement therapy compounds for St. Jude to test for potential use in the treatment of PKAN disease.

PKAN, or pantothenate kinase-associated neurodegeneration, is a hereditary disease characterized by buildup of iron in the brain and progressive degeneration of the nervous system. Symptoms of PKAN vary greatly but may include dystonia, muscular rigidity, and sudden involuntary muscle spasms. 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 cure for PKAN.^{1, 2}

Suzanne Jackowski, PhD, principal investigator of the study for St. Jude, has conducted extensive research on coenzyme A, which is regulated by pantothenate kinase. She has been investigating the four active pantothenate kinases to understand the metabolic basis for PKAN.

"This is a great opportunity for me to contribute to the development of a promising treatment for a catastrophic pediatric disease," said Jackowski.

"This collaboration reinforces our commitment to discovering treatments for rare diseases, especially those that have a devastating effect on children," said Martin Shkreli, Chief Executive Officer of Retrophin. "We're thrilled to be working with Dr. Jackowski and St. Jude, and look forward to reporting progress on the research program in the coming year. Our hope is to get a viable candidate into clinical research as soon as possible. The nature of replacement therapy is intuitive and we believe has a high probability of success compared to other treatment modalities. By replacing the underlining defect in these patients, we hope to directly restore their health."

Under terms of the agreement, Retrophin will own the intellectual property for the compounds it delivers to St. Jude, while St. Jude will own and have the ability to publish results of the biological tests it performs. Financial terms of the agreement are not being disclosed.

About St. Jude Children's Research Hospital

Since opening 50 years ago, St. Jude Children's Research Hospital has changed the way the world treats childhood cancer and other life-threatening diseases. No family ever pays St. Jude for the care their child receives and, for every child treated here, thousands more have been saved worldwide through St. Jude discoveries. The hospital has played a pivotal role in pushing U.S. pediatric cancer survival rates from 20 to 80 percent overall, and is the first and only National Cancer Institute-designated Comprehensive Cancer Center devoted solely to children. It is also a leader in the research and treatment of blood disorders and infectious diseases in children. St. Jude was founded by the late entertainer Danny Thomas, who believed that no child should die in the dawn of life. To learn more, visit www.stjude.org. Follow us on Twitter @StJudeResearch.

About Retrophin, LLC

Retrophin, LLC is a privately-held New York-based, biotechnology company focused on discovering and developing treatments for rare and life-threatening diseases. Retrophin is currently developing treatments for focal segmental glomerulosclerosis (FSGS), Duchenne Muscular Dystrophy and other catastrophic diseases. The company's lead compound, RE-021, formerly known as DARA, is scheduled to begin enrollment in a Phase II clinical trial for FSGS in the near future. Retrophin's Series A financing was led by MSMB Capital and several current and former senior executives at global pharmaceutical and healthcare companies.

¹ National Institute of Neurological Disorders and Stroke: <http://www.ninds.nih.gov/disorders/nbia/nbia.htm>

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