Retrophin Announces Cooperative Research and Development Agreement with NCATS and NGLY1.org to Identify Potential Small Molecule Therapeutics for NGLY1 Deficiency

September 5, 2017

SAN DIEGO, Sept. 05, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced that it has entered into a three-way Cooperative Research and Development Agreement (CRADA) with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 Deficiency. This rare and debilitating monogenic disease is characterized by developmental delays, seizures and an inability to produce tears; there are currently no approved therapeutic options for NGLY1 Deficiency.

"This collaboration with Retrophin and NCATS represents an exciting step forward and a major milestone for the NGLY1 community," said Cristina Casanova Might, president of NGLY1.org. "We are grateful to both NCATS and Retrophin for working with our community to establish this partnership which enables us to accelerate drug discovery efforts, and gives hope to our patients and their families."

The research collaboration will focus on the development of assays for small molecule high-throughput screening in an effort to better understand the biology of the disorder, and identify potential small molecules to be developed as a therapeutic for patients living with NGLY1 Deficiency. Retrophin will seek to continue development of compounds through pre-clinical and clinical phases, with the ultimate goal of delivering a treatment option for the NGLY1 community.

"This CRADA is a great example of how we can strive to make advancements through collaborative research on new therapeutic approaches for people living with rare diseases, such as NGLY1 deficiency," said Bill Rote, PhD, senior vice president and head of research and development for Retrophin. "This synergy between industry, public research, and advocacy gives us a great opportunity to share resources with the common goal of making a difference in the lives of patients and families affected by NGLY1 deficiency, and Retrophin is proud to work alongside NCATS and NGLY1.org in this endeavor."

About NGLY1 Deficiency

N-glycanase deficiency, or NGLY1 deficiency, is an extremely rare genetic disorder believed to be caused by a deficiency in an enzyme called N-glycanase-1, which is encoded by the gene NGLY1. The condition has been characterized by symptoms such as developmental delays, seizures, complex hyperkinetic movement disorders, diminished reflexes and an inability to produce tears. There are no approved therapeutic options for NGLY1 deficiency, and current therapeutic strategies are limited to symptom management.

About NGLY1.org

NGLY1.org is a nonprofit 501(c)(3) patient support, advocacy and research organization for N-Glycanase (NGLY1) Deficiency. NGLY1.org's mission is to eliminate the challenges of NGLY1 through research, awareness and support. NGLY1.org improves the lives of patients through family support, diagnostic access, clinical and patient education, conferences, and grants. For more information, visit NGLY1.org

About Retrophin

Retrophin is a fully integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research exploring the potential of early-stage assets in additional rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola®, Cholbam® and Chenodal®. 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. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "seeks", "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. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the Company's business and finances in general, success of its commercial products, as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. The risks and uncertainties the Company faces with respect to the collaboration described in this press release include risk that the collaboration will not be successful and will not result in the identification of potential small molecule therapeutics for NGLY1 Deficiency. 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 most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

Contact:
Chris Cline, CFA
Vice President, Investor Relations & Corporate Communications
760-260-8600
IR@retrophin.com

Primary Logo

Retrophin, Inc.