Retrophin Announces Cooperative Research and Development Agreement with NCATS and the Alagille Syndrome Alliance to Identify Potential Therapeutics for Alagille Syndrome

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SAN DIEGO, June 18, 2019 (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 Alagille Syndrome Alliance (ALGSA) to collaborate on research efforts aimed at the identification and development of potentially novel therapeutics for Alagille syndrome (ALGS). ALGS is a rare and debilitating disease characterized by severe liver and cardiovascular abnormalities. There are currently no approved therapies for ALGS.

"This partnership with Retrophin and NCATS offers new hope to our patients and their families," said Roberta Smith, president of ALGSA. "We're excited to be an active part of this collaboration aimed at advancing meaningful efforts to find potential therapies for ALGS."

The collaboration seeks to develop high-throughput screening assays and conduct hit validation in an effort to identify compounds that could be advanced in development, with the goal of delivering potentially novel therapeutic options to the ALGS patient community.

"This CRADA broadens our collaboration with leading scientists at NCATS and patient advocacy organizations to advance early research and development efforts," said Bill Rote, Ph.D., senior vice president of research and development for Retrophin. "Retrophin is proud to work with the NIH and ALGSA in continuing to shift the paradigm of early therapeutic identification and development in rare disease toward multi-stakeholder partnerships in pre-clinical research, with the common goal of making a difference in the lives of patients and families affected by ALGS."

About Alagille Syndrome

Alagille syndrome, or ALGS, is a rare genetic disorder that can affect the liver, heart, skeleton, eyes and kidneys. Symptoms and severity of ALGS can vary greatly from one person to another. Symptoms often develop during the first three months of life and include interrupted bile flow (cholestasis), jaundice, poor weight gain and growth, and severe itching (pruritis). ALGS is an autosomal dominant disorder, meaning that a child may be affected when one parent passes on a copy of the abnormal gene (JAG1 or NOTCH2). In other cases, the gene mutation can occur spontaneously, meaning it is not inherited from a parent. According to the National Organization for Rare Disorders (NORD), the estimated incidence of ALGS is between 1 in 30,000 and 1 in 45,000 births.

About Alagille Syndrome Alliance

The Alagille Syndrome Alliance is an international nonprofit started in 1993 representing the ALGS community. The mission of the organization is to mobilize resources, facilitate connections, promote unity and advocate for a cure to inspire, empower and enrich the lives of people affected by Alagille syndrome. The ALGSA staff and board includes ALGS patients and family members, all deeply understanding of the complex and difficult nature of ALGS and circumstances resulting from such difficulty.

Alagille.org

About National Center for Advancing Translational Sciences

The National Center for Advancing Translational Sciences (NCATS) — one of 27 Institutes and Centers at the National Institutes of Health (NIH) — was established to transform the translational process so that new treatments and cures for disease can be delivered to patients faster. The Center's organizational structure includes diverse representation, including those from disease advocacy organizations and private equity firms, along with renowned scholars in translational science and regulatory review.

ncats.nih.gov

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare disease. 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) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal ®, Cholbam® and Thiola®.

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 Alagille syndrome. 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.

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