Retrophin Announces U.S. FDA Acceptance of NDA Filing for the New Formulation of Thiola® (tiopronin) in the Treatment of Cystinuria

November 12, 2018

PDUFA date set for June 30, 2019

SAN DIEGO, Nov. 12, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the U.S. Food and Drug Administration (FDA) has accepted for review the New Drug Application (NDA) for a new formulation of Thiola (tiopronin) in the treatment of cystinuria. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2019.

"We know that living with a rare kidney disorder like cystinuria can be a challenge so we have worked with the patient, caregiver and medical communities over the past several years to identify ways we can further enhance Thiola," said Stephen Aselage, chief executive officer of Retrophin. "The acceptance of this NDA for review is an important milestone as we continue efforts to fulfill our ongoing commitment to support patients with cystinuria and deliver a new, more patient-friendly formulation of Thiola."

The NDA was filed by the Company's partner, Mission Pharmacal Company, through the 505(b)(2) regulatory pathway which allows the FDA to reference previous findings of safety and efficacy for an already-approved product, in addition to reviewing findings from further studies of the product.

About Thiola® (tiopronin)

Thiola (tiopronin) tablets are indicated for the prevention of cystine (kidney) stone formation in patients with severe homozygous cystinuria with urinary cystine >500 mg/day, who are resistant to treatment with conservative measures of high fluid intake, alkali and diet modification, or who have adverse reactions to d-penicillamine.

Important Safety Information

Warnings & Precautions:

While no deaths have been reported with Thiola treatment, and despite the apparent reduced toxicity of Thiola relative to d-penicillamine, Thiola can potentially cause all the serious adverse reactions reported for d-penicillamine, including death. Hematologic abnormalities requiring drug discontinuance may occur. Proteinuria, may sometimes occur, severe enough to cause nephrotic syndrome. Thiola therapy should be discontinued if there are findings suggestive of Goodpasture syndrome, myasthenic syndrome, or myasthenia gravis. To help reduce the risk of serious complications, the following tests are recommended: peripheral blood counts, direct platelet count, hemoglobin, serum albumin, liver function tests, 24-hour urinary protein and routine urinalysis. The safety and efficacy for Thiola in children under 9 years of age have not been established.

Contraindications:

Thiola is contraindicated during pregnancy (except where the benefits clearly outweigh the risks), in nursing mothers, and in patients who have previously developed agranulocytosis, aplastic anemia or thrombocytopenia while on this medication.

Adverse Reactions:

Some patients may develop drug fever, usually during the first month of therapy. Thiola treatment should be discontinued until the fever subsides. Other potential associated adverse reactions include: gastrointestinal side effects, impairment in taste and smell, hypersensitivity reactions, hematologic abnormalities, renal complications, pulmonary manifestations, wrinkling and friability of skin, and neurological complications. The most common adverse reactions in clinical trials include: diarrhea (2%), and the following at 1%: reflux esophagitis, malaise, skin lesion, nausea, abdominal pain, intestinal polyp, urinary tract infection, and peripheral neuropathy.

Please see the full prescribing information including the complete indications and usage, contraindications, warnings, precautions and adverse reactions at thiola.com.

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", "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. Specifically, the Company faces risks associated with market acceptance of its products (including the new formulation of Thiola if approved for marketing by the FDA), including efficacy, safety, price, reimbursement and benefit over competing therapies. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that current or future clinical trials will not proceed as planned. The Company faces risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates; risk relating to the Company's dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and exclusivity periods and intellectual property rights of third parties; risk that the NDA for the new formulation of Thiola will not be approved by the FDA; and risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products. 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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