Retrophin Announces FDA Approval of THIOLA® EC (tiopronin) 100mg and 300mg Tablets for the Treatment of Cystinuria

June 28, 2019

Enteric-coated formulation offers flexible dosing options, including administration with or without food

THIOLA EC expected to be available to patients next month

SAN DIEGO, June 28, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that the U.S. Food and Drug Administration (FDA) has approved 100 mg and 300 mg tablets of THIOLA® EC (tiopronin), a new enteric-coated formulation of THIOLA® (tiopronin), to be used for the treatment of cystinuria, a rare inherited disorder that causes a buildup of cystine levels in the urine resulting in the formation of recurring cystine kidney stones. THIOLA EC is expected to be available in July 2019.

"The approval of THIOLA EC marks another step in our continued commitment to helping patients with cystinuria manage the threat of recurring cystine stones," said Eric Dube, Ph.D., chief executive officer of Retrophin. "This new formulation provides patients with the freedom to administer THIOLA EC with or without food, an advancement over the original formulation which has limiting food restrictions, and also provides the potential to reduce the number of tablets necessary to manage cystinuria. We look forward to working with the cystinuria community as we make the new formulation available next month."

The recommended initial dosage of THIOLA in adult patients is 800 mg per day and in clinical studies the average dose of THIOLA was approximately 1,000 mg, or 10 pills per day. The original formulation of THIOLA 100 mg is recommended to be administered at least one hour before or two hours after meals. THIOLA EC 100 mg and 300 mg tablets are recommended to be administered with or without food.

"THIOLA's utility as the treatment of choice for cystinuria is well established. However, for certain patients, the challenges of administration one hour before or two hours after meals three times a day, coupled with a high pill burden, have been challenging," said Dr. David S. Goldfarb, Clinical Chief, Division of Nephrology at NYU Langone Health. "Having a new treatment option with the flexibility of dosing with or without food, as well as one that provides an opportunity for patients to take fewer pills, should meaningfully improve convenience and compliance."

THIOLA EC tablets were approved through the 505(b)(2) regulatory pathway which allows the FDA to reference previous findings of safety and efficacy for an already-approved product, combined with reviewing findings from further studies of the product.

About THIOLA® EC (tiopronin)

THIOLA® EC (tiopronin) is indicated, in combination with high fluid intake, alkali, and diet modification for the prevention of cystine stone formation in adults and pediatric patients ≥20 kg with severe homozygous cystinuria, who are not responsive to these measures alone.

Patients and physicians can access additional information about THIOLA EC by visiting thiola.com.

Important Safety Information

Contraindications:

THIOLA EC is contraindicated in patients with hypersensitivity to tiopronin or any other components of THIOLA EC.

Warnings and precautions:

Proteinuria: Proteinuria, including nephrotic syndrome and membranous nephropathy, have been reported with tiopronin use. Pediatric patients receiving >50 mg/kg of tiopronin per day may be at increased risk for proteinuria. Monitor patients for the development of proteinuria and discontinue therapy in patients who develop proteinuria.

Hypersensitivity Reactions: Hypersensitivity reactions (drug fever, rash, fever, arthralgia and lymphadenopathy) have been reported.

Adverse Reactions:

The most common adverse reactions (≥10%) are nausea, diarrhea or soft stools, oral ulcers, rash, fatigue, fever, arthralgia, proteinuria, and emesis.

Drug interactions:

Avoid alcohol consumption 2 hours before and 3 hours after taking THIOLA EC.

Special populations:

Lactation: Breastfeeding is not recommended during treatment with THIOLA EC.

Geriatric Use: Because elderly patients are more likely to have decreased renal function, care should be taken in dose selection, and it may be useful to monitor renal function.

You may report negative side effects to Retrophin Medical Information at 1-877-659-5518, or to the FDA at 1-800-FDA-1088 or

www.fda.gov/medwatch. Please see full Prescribing Information for Important Safety Information at thiola.com.

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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), 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; 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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