Retrophin Acquires Liquid Formulation of Ursodeoxycholic Acid

June 20, 2016

SAN DIEGO, June 20, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced the signing of a definitive agreement to purchase the rights, titles, and ownership of a liquid formulation of ursodeoxycholic acid from Asklepion Pharmaceuticals, LLC. Retrophin intends to file a New Drug Application with the U.S. Food and Drug Administration for the liquid formulation of ursodeoxycholic acid for the treatment of primary biliary cholangitis (PBC) in 2017.

"Liquid ursodeoxycholic acid is highly complementary to our portfolio of bile acid therapies, allowing us to leverage our current commercial infrastructure and further diversify our marketed products," said Steve Aselage, chief executive officer of Retrophin. "By commercializing a liquid formulation of ursodeoxycholic acid, we will aim to fill a gap in care for PBC patients who have difficulty swallowing pills."

Under the terms of the agreement, Retrophin made an upfront payment of $0.5 million and will make future payments of up to $23.5 million based on cumulative sales milestones of liquid ursodeoxycholic acid in PBC. Asklepion will be eligible to receive incremental milestone payments of up to $35.0 million in aggregate upon successful development and commercialization, including cumulative sales milestones, of liquid ursodeoxycholic acid in additional indications. Retrophin will assume all future program costs and pay Asklepion tiered royalties on future product sales.

About Ursodeoxycholic Acid

Also known as ursodiol or UDCA, ursodeoxycholic acid is a naturally occurring hydrophilic bile acid derived from cholesterol, which is indicated for the treatment of PBC and currently prescribed only in solid forms. Introducing a liquid formulation of ursodeoxycholic acid would provide healthcare professionals with a dosing alternative for patients who have difficulty swallowing tablets or capsules, and may facilitate increased compliance.

About Primary Biliary Cholangitis

Primary biliary cholangitis, also known as primary biliary cirrhosis, is a rare and chronic autoimmune disease that causes the small bile ducts in the liver to become inflamed, damaged and ultimately destroyed. This results in the buildup of bile in the liver and damage to the liver cells over time, resulting in cirrhosis, or scarring of the liver. As cirrhosis progresses, and the amount of scar tissue in the liver increases, the liver loses its ability to function.

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 clinical-stage assets targeting rare diseases with significant unmet medical needs, including sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease, and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood. Research exploring the potential of early-stage assets in several 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. 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, as well as risks and uncertainties associated with the Company's research preclinical and clinical stage pipeline. Specifically, the risks and uncertainties the Company faces with respect to its liquid formulation of ursodeoxycholic acid program are: the inability to file a New Drug Application, the ability able to file a New Drug Application in 2017, that a New Drug Application will not be approved for safety, regulatory or other reasons, and that the liquid formulation of ursodeoxycholic acid will not be effective for the treatment of primary biliary cholangitis or other indications. 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 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 filings with the Securities and Exchange Commission.

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Source: Retrophin, Inc.