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U.S. Food and Drug Administration Approves Cholbam for the Treatment of Rare Bile Acid Synthesis Disorders and Grants Rare Pediatric Disease Priority Review Voucher

Retrophin Will Acquire Worldwide Rights

SAN DIEGO--(BUSINESS WIRE)-- Retrophin, Inc. (NASDAQ:RTRX) announced today that the U.S. Food and Drug Administration (FDA) has approved Cholbam (cholic acid) capsules, the first FDA approved treatment for pediatric and adult patients with bile acid synthesis disorders due to single enzyme defects, and for patients with peroxisomal disorders (including Zellweger spectrum disorders).

As a result of the approval, Retrophin will exercise its right to purchase from Asklepion Pharmaceuticals, LLC all worldwide rights, titles, and ownership of Cholbam and related assets. Under the terms of the agreement announced on January 12, 2015, Retrophin will pay Asklepion a one-time cash payment of $27 million, in addition to approximately 661,278 shares of Retrophin common stock (initially valued at $9 million at the time of the agreement), which assumes Cholbam received an approval for a CTX indication. Asklepion will also be eligible to receive up to $37 million in cumulative sales milestones, as well as tiered royalties based on future net sales of Cholbam.

The FDA also granted Asklepion a Rare Pediatric Disease Priority Review Voucher ("Pediatric PRV"), a provision that encourages development of new drugs and biologics for the prevention and treatment of rare pediatric diseases. This voucher is designed to be transferable or sold and provides the bearer with an expedited FDA review for any new drug application. The Pediatric PRV will be transferred to Retrophin under the original terms of the agreement with Asklepion.

"With FDA approval, Cholbam will be available to patients suffering from several life-threatening diseases that until now, had no approved treatment. Cholbam complements Retrophin's existing bile acid therapy, Chenodal (chenodeoxycholic acid), and will position us as the leading provider of treatments for patients with these bile acid synthesis and peroxisomal disorders," said Stephen Aselage, Chief Executive Officer of Retrophin.

The effectiveness of Cholbam has been demonstrated in clinical trials for bile acid synthesis disorders and the adjunctive treatment of peroxisomal disorders. There are approximately 30 patients currently receiving Cholbam through an open label extension of these trials. The estimated incidence of bile acid synthesis disorders due to single enzyme defects is 1 to 9 per million live births. Peroxisomal disorders are believed to affect approximately 1 in 50,000 live births.

Cholbam will have seven years market exclusivity in the United States conferred by its designation as an orphan drug. Retrophin expects to close the acquisition and be able to begin distributing therapy in as few as two to four weeks, subject to the satisfaction of customary closing conditions.

About Retrophin

Retrophin is a pharmaceutical company focused on the development, acquisition and commercialization of drugs for the treatment of serious, catastrophic or rare diseases for which there are currently no viable options for patients. The Company's approved products include Chenodal® and Thiola®, and its pipeline includes compounds for several catastrophic diseases, including focal segmental glomerulosclerosis (FSGS), pantothenate kinase-associated neurodegeneration (PKAN), infantile spasms, nephrotic syndrome and others. For additional information, please visit www.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, regarding the research, development and commercialization of pharmaceutical products. 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 ability to consummate the acquisition of Cholbam or the Pediatric PRV, the effectiveness of Cholbam in treating bile acid synthesis disorders or peroxisomal disorders, the Company's ability to leverage Cholbam as a

complement to the Company's existing bile acid therapy, the Company's ability to position itself as the leading provider of treatments for patients with bile acid synthesis and peroxisomal disorders, the incidence rate of bile acid synthesis disorders and peroxisomal disorders, Cholbam receiving an approval for a CTX indication, the Company's business and finances in general, as well as risks and uncertainties associated with the Company's sales and marketing strategies. 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.

Retrophin, Inc.
Chris Cline, CFA, 646-564-3680
Manager, Investor Relations
IR@retrophin.com

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