Retrophin Announces Acquisition of Exclusive Right to Purchase Cholic Acid from Asklepion Pharmaceuticals

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NEW YORK & BALTIMORE--(BUSINESS WIRE)-- Retrophin, Inc. (NASDAQ:RTRX) and Asklepion Pharmaceuticals, LLC, a privately held rare disease pharmaceutical development company, today announced the signing of a definitive agreement under which Retrophin has acquired the exclusive right to purchase from Asklepion, all worldwide rights, titles, and ownership of cholic acid for the treatment of bile acid synthesis defects, if approved by the U.S. Food and Drug Administration (FDA). Under the terms of the agreement, Retrophin will pay Asklepion an upfront payment of $5 million and up to $73 million in milestones based on approval and net product sales, plus tiered royalties on future net sales of cholic acid. Retrophin has secured a line of credit from current lenders to cover necessary payments.

"We are very pleased with this agreement, which has the potential to significantly accelerate Retrophin's growth in 2015 and beyond," said Stephen Aselage, Chief Executive Officer of Retrophin. "Cholic acid is a natural complement to our current commercial portfolio and upon its approval, Retrophin would be positioned to become the leading provider of bile acid treatments for a number of rare diseases."

Mr. Aselage continued, "The effort by Asklepion and its investigators is a testament to their dedication and hard work in bringing an important medication closer to the marketplace. The disorders treated in their clinical trial are devastating and we are grateful to have an opportunity to address those patients' needs."

Cholic acid is being considered for approval in a late-stage review by the FDA and is currently approved in Europe for the treatment of inborn errors in primary bile acid synthesis. It restores endogenous bile acid pool levels while inhibiting abnormal bile acid synthesis. The clinical use of cholic acid has been documented in medical literature for several single enzyme bile acid synthesis defects since the mid-1990s.

"This agreement could represent a significant milestone for both our company and patients suffering from bile acid disorders," said Gary Pasternack, Chief Executive Officer of Asklepion. "If cholic acid is approved, we are confident that Retrophin's expertise in this area would allow for the greatest number of patients to benefit from this treatment.

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®, Thiola® and Vecamyl®, and its pipeline includes compounds for several catastrophic diseases, including focal segmental glomerulosclerosis (FSGS), pantothenate kinase-associated neurodegeneration (PKAN), schizophrenia, infantile spasms, nephrotic syndrome and others. For additional information, please visit www.retrophin.com.

About Asklepion

Asklepion is a pharmaceutical company focused on the clinical research and development through regulatory approval of innovative therapies for rare pediatric diseases. Asklepion's pipeline products address other liver diseases and critical care applications in children.

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 business and finances in general, as well as risks and uncertainties associated with the Company's pre-clinical and clinical stage pipeline as well as its sales and marketing strategies. Specifically, the Company faces risk that cholic acid may not be approved by the FDA, that certain conditions to the purchase of cholic acid by the Company will not be met and that the Company will not be able to successfully launch and commercialize cholic acid in the United States following FDA approval. The Company faces risk that it will be unable to raise additional funding; risk relating to the dependence on contractors for commercial manufacturing; uncertainties relating to patent protection and intellectual property rights of third parties; 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 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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