Retrophin Completes Acquisition of Orphan Technologies

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Addition of OT-58 strengthens pipeline of potential first-in-class therapies targeting rare diseases

SAN DIEGO, Nov. 12, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced the completion of its previously announced acquisition of Orphan Technologies Limited, a privately held, clinical-stage biopharmaceutical company focused on the development of product candidate OT-58 for the treatment of classical homocystinuria (HCU). OT-58 is a novel investigational human enzyme replacement therapy being evaluated in Phase 1/2 development for the treatment of classical HCU, a rare metabolic disorder characterized by elevated levels of plasma homocysteine that can lead to life-threatening thrombotic events such as stroke and heart attacks, ophthalmologic and skeletal complications, as well as developmental delay.

“We are excited to begin working with the HCU community to develop a deeper understanding of how we can continue to integrate their perspectives into the development of OT-58, and help address their unmet needs,” said Eric Dube, Ph.D., chief executive officer of Retrophin. “We look forward to building upon the promising potential of OT-58 with the goal of developing and ultimately delivering the first disease modifying therapy for people living with HCU.”

Under the terms of the agreement, Retrophin made an upfront payment of $90 million in cash at closing of the transaction. Orphan Technologies shareholders will remain eligible to receive up to $427 million in additional cash payments contingent upon the achievement of key milestones in the development and commercialization of OT-58. Retrophin will also pay a tiered mid-single digit royalty on future net sales of OT-58 in the US and Europe, and potentially make a milestone payment in the event a rare pediatric disease priority review voucher is granted.

Barclays acted as financial advisor, and Cooley LLP acted as legal counsel to Retrophin. Cantor Fitzgerald & Co. acted as financial advisor, and Hogan Lovells US LLP acted as legal counsel to Orphan Technologies.

About Classical Homocystinuria

Classical homocystinuria (HCU) is a rare genetic metabolic disorder caused by a deficiency in the enzyme cystathionine beta synthase (CBS). CBS is a pivotal enzyme that is essential for the management of methionine and cysteine in the body. Classical HCU leads to toxic levels of homocysteine that can result in life-threatening thrombotic events such as stroke and heart attacks, ophthalmologic and skeletal complications, as well as developmental delay. Current treatment options are limited to protein-restricted diet and supplemental use of vitamin B6 and betaine.

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 sparsentan, a product candidate in late-stage development for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), rare disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including partnerships with leaders in patient advocacy and government research to identify potential therapeutics for NGLY1 deficiency and Alagille syndrome, conditions with no approved treatment options. Retrophin’s R&D efforts are supported by revenues from the Company’s commercial products Chenodal ®, Cholbam®, Thiola® and Thiola EC®.

Retrophin.com

About Orphan Technologies

Orphan Technologies is a clinical-stage biopharmaceutical company focused on the development of OT-58. OT-58 is an investigational human enzyme replacement therapy being evaluated in Phase 1/2 development for the treatment of classical homocystinuria (HCU). HCU is a rare metabolic disorder characterized by elevated levels of plasma homocysteine that can lead to life-threatening thrombotic events such as stroke and heart attacks, ophthalmologic and skeletal complications, as well as developmental delay.

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 include, but are not limited to, references related to; the potential impact upon and benefits to Retrophin from the acquisition of Orphan Technologies; the potential for OT-58 to ultimately become the first disease modifying therapy for HCU; and references to the achievement of future potential development and commercialization milestones for the OT-58 program, including, without limitation, the potential future issuance of a rare pediatric disease priority review voucher. Such forward-looking statements are based on current information available to Retrophin and involve inherent risks and uncertainties, including factors that could delay, divert or change any such forward-looking statements, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Retrophin faces risks associated with, but not limited to: Retrophin’s ability to realize the anticipated benefits of the proposed transaction, including the potential developmental and commercial success of the OT-58 product candidate; significant and unknown transaction costs; actual or contingent liabilities; the risk of litigation and/or regulatory actions related to the transaction; other business effects outside of Retrophin’s control, including the effects of industry, market, economic, political or regulatory conditions or the ongoing COVID-19 pandemic; as well as negative impacts that could result from changes in tax and other laws, regulations, rates and policies. In addition, such risks and uncertainties may include those described in Retrophin’s annual, quarterly and current reports (i.e., Form 10-K, Form 10-Q and Form 8-K) as filed or furnished with the Securities and Exchange Commission, which are available at

Retrophin's website (www.retrophin.com) under "Investors & Media". You are cautioned not to place undue reliance on any forward-looking statements as there are important factors that could cause actual results to differ materially from those in any forward-looking statements, many of which are beyond our control. Except to the extent required by law, Retrophin undertakes no obligation to publicly update any forward-looking statement.

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