# Retrophin Announces Agreement to Acquire Orphan Technologies

October 22, 2020

*Agreement adds OT-58, a novel enzyme replacement therapy in Phase 1/2 development for the treatment of classical homocystinuria*

*Compelling strategic fit that will expand pipeline of potential first-in-class therapies targeting rare diseases*

*Retrophin to host conference call and webcast today at 5:00 p.m. ET*

SAN DIEGO, Oct. 22, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that it has entered into a definitive agreement to acquire 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 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. Current treatment options, including heavy dietary restrictions and supplemental use of vitamin B6 and betaine, are often ineffective in managing homocysteine levels and a significant unmet need remains.

“Many people with HCU face a continuous risk of developing life-threatening complications because current treatment options are largely ineffective in managing homocysteine levels,” said Eric Dube, Ph.D., chief executive officer of Retrophin. “OT-58 has demonstrated an ability to meaningfully reduce homocysteine levels in preclinical models and has the potential to ultimately become the first disease modifying therapy for HCU. This promising, novel development candidate fits directly with our mission to identify, develop and deliver life-changing therapies to people living with rare disease and brings exciting growth potential to Retrophin.”

OT-58 is a PEGylated, recombinant enzyme replacement therapy designed to address the underlying cause of classical HCU — a deficiency in the naturally occurring enzyme cystathionine beta synthase (CBS). A deficiency in CBS prevents regular metabolism from occurring and results in elevated levels of homocysteine. In preclinical studies, OT-58 has demonstrated an ability to reduce total homocysteine levels and improve clinical parameters. Specifically, dosing of OT-58 in mouse models corrected metabolite levels, including up to 90% reduction in homocysteine levels in plasma and tissues, and appeared to prolong survival, prevent osteoporosis and rescue ocular structure. OT-58 is currently advancing in a Phase 1/2 dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. OT-58 has been granted Rare Pediatric Disease and Fast Track designations by the US Food and Drug Administration (FDA), as well as Orphan Drug designation in the US and Europe.

“Orphan Technologies’ longstanding mission has been to reduce the disease burden for people living with HCU, including debilitating complications of the skeletal, cardiovascular, ocular, and central nervous systems. Therefore, I am extremely proud of the Orphan Technologies team for advancing OT-58 from early stage research and preclinical studies into a Phase 1/2 trial,” said Frank Glavin, chief executive officer of Orphan Technologies. “We are now at an ideal juncture to pair the therapeutic promise of OT-58 with Retrophin’s late-stage development and commercial capabilities in rare diseases. I believe that with this new stewardship, we increase the potential for OT-58 to become an impactful new treatment option for patients.”

Under the terms of the agreement, Retrophin will make an upfront payment of $90 million in cash upon closing of the transaction. Orphan Technologies shareholders will also be 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 pediatric rare disease voucher is granted.

The transaction has been approved by the boards of directors of both companies. It is subject to customary closing conditions, including consummation of a spinout agreement for Orphan Technologies’ preclinical OT-15 product candidate, and is anticipated to close in the fourth quarter of 2020.

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.

**Conference Call Information**

Retrophin will host a conference call and webcast today, October 22, 2020 at 5:00 p.m. ET to discuss the acquisition. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 5086267 shortly before 5:00 p.m. ET. The webcast and slides can be accessed at retrophin.com, in the Events and Presentations section, and will be archived for at least 30 days. A replay of the call will be available from 8:00 p.m. ET, October 22, 2020 to 8:00 p.m. ET, October 29, 2020. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 5086267.

**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 dedicated to developing novel therapies to dramatically improve the lives of patients suffering from the rare disorder, classical homocystinuria. OT-58 has been optimized as an investigational enzyme replacement therapy for classical homocystinuria, a genetic disease characterized by debilitating cardiovascular, skeletal, neurologic, and ophthalmologic complications. OT-58 is designed to reduce homocysteine levels via a targeted mechanism of action and may have therapeutic applications in other diseases.

**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 Retrophin's expectations with respect to the closing of its planned acquisition of Orphan Technologies; the potential impact upon and benefits to Retrophin from the proposed acquisition; the potential for OT-58 to ultimately become the first disease modifying therapy for HCU; and references to future expectations, plans and prospects for Retrophin. 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: the parties' ability to complete the proposed transaction in a timely manner, if at all, considering the various closing conditions; if consummated, 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 proposed transaction; other business effects outside of either company'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.

Contact:
Chris Cline, CFA
Senior Vice President, Investor Relations & Corporate Communications
888-969-7879
IR@retrophin.com

Source: Retrophin, Inc.