Retrophin and Censa Pharmaceuticals Enter into Strategic Collaboration to Advance CNSA-001 for the Treatment of Phenylketonuria (PKU)

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Retrophin obtains exclusive option to purchase Censa post proof-of-concept study

Phase 2 proof-of-concept study in PKU patients expected to commence in mid-2018

SAN DIEGO, Jan. 05, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today announced a joint development agreement with Censa Pharmaceuticals, a privately held biotechnology company focused on developing therapies for orphan metabolic diseases, to evaluate CNSA-001 for the treatment of phenylketonuria (PKU). CNSA-001 is an orally bioavailable form of a natural precursor of tetrahydrobiopterin (BH4) with the potential to provide improved phenylalanine (Phe) reduction in patients with PKU when compared to BH4.

Under the terms of the agreement, Retrophin is making a $10 million upfront payment to Censa and will provide funding for development of CNSA-001 in PKU. Censa will run the development program, which will be conducted under the oversight of a joint steering committee. As part of the agreement, Retrophin will pay certain milestone payments and obtains the exclusive option to acquire Censa upon conclusion of a specified option period, pending clinical proof of concept of CNSA-001 in PKU.

“This transaction represents a significant opportunity to further strengthen our product pipeline and expands Retrophin’s strategy of delivering life-changing therapies to individuals living with rare diseases who have limited treatment options,” said Stephen Aselage, chief executive officer of Retrophin. “Importantly, the data generated by the program to date suggest that CNSA-001 has both the potential to offer a meaningful improvement in care for PKU patients, and the ability to move rapidly through the clinic upon positive proof of concept data.”

“We believe Retrophin’s specialized capabilities and focus on developing and delivering therapies for rare diseases are highly differentiated and well-suited to this important program,” said Jonathan Reis, M.D., president and chief executive officer of Censa. “The pre-clinical data supporting this program are very encouraging, and suggest that treatment with CNSA-001 may lead to higher intracellular BH4 levels and improved phenylalanine reduction, which may offer a significant benefit for the large subset of PKU patients who do not achieve an optimal response with the current standard of care. We look forward to working with Retrophin to advance CNSA-001 through clinical proof of concept as expeditiously as possible.”

About CNSA-001

CNSA-001 is an orally bioavailable proprietary form of sepiapterin, a natural precursor of tetrahydrobiopterin (BH4) that is converted by an endogenous enzymatic pathway to BH4. Preclinical research has suggested CNSA-001 may provide improved bioavailability, plasma stability and tissue exposure, leading to higher intracellular BH4 levels and subsequent greater phenylalanine (Phe) reduction when compared to the current standard of care in PKU. In pre-clinical models, sepiapterin has also shown an ability to cross the blood-brain barrier which, if supported by clinical data, may lead to broader utility in additional indications such as primary BH4 deficiency (PBD) and Segawa syndrome. CNSA-001 is currently being evaluated in a single ascending dose (SAD) study and a Phase 2 proof of concept study in PKU is expected to commence in mid-2018.

About Phenylketonuria (PKU)

PKU is a rare, genetic metabolic condition in which the body cannot breakdown Phe due to a missing or defective phenylalanine hydroxylase (PAH) enzyme. High Phe levels can lead to developmental and physical growth delay, executive function impairment, seizures, and microcephaly caused by toxic Phe accumulation in the brain. PKU is typically diagnosed at birth and has an estimated prevalence of 45,000 to 50,000 patients worldwide.

About Retrophin

Retrophin is a biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. The Company’s approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease and glomerulonephritis, respectively. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin’s R&D efforts are supported by revenues from the Company’s commercial products Chenodal®, Cholbam® and Thiola®.

Retrophin.com

About Censa

Censa Pharmaceuticals Inc., is a Boston based, privately held biotechnology company dedicated to developing and commercializing novel therapies for people living with orphan diseases associated with defects in the tetrahydrobiopterin bio-chemical pathways.

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", “suggests”, "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, success of its commercial products as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing

therapies. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that planned clinical trials will not proceed as planned. With respect to the Company's strategic collaboration with Censa that is the subject of this press release, the Company faces the risk that the ongoing CNSA-001 SAD study will not be successful, risk that the planned Phase 2 clinical trial of CNSA-001 will not proceed as planned and/or will not demonstrate proof of concept in PKU during the specified option period, risk that the differentiating attributes of CNSA-001 seen in preclinical models will not be replicated in clinical studies, risk of manufacturing challenges due to the early stage of the program, risk that the regulatory authorities will impose requirements that preclude the ability to move rapidly through the clinic despite positive proof of concept data, risk that the joint development relationship with Censa will not be successful, risk that the pre-negotiated financial terms of the acquisition may not prove to be economically viable for the Company to exercise the option, and risk that the Company will elect not to exercise the option to acquire Censa and therefore will not gain ownership rights to CNSA-001. More generally, the Company faces risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. In addition, the Company faces risk that it will be unable to raise additional funding that may be required to complete development of any or all of its product candidates; risk relating to dependence on contractors for clinical drug supply and commercial manufacturing; uncertainties relating to patent protection and exclusivity periods and intellectual property rights of third parties; and risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products and/or CNSA-001. 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 any 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 most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

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