



## Retrophin Provides Corporate Update and 2019 Outlook

January 7, 2019

### Top-line readout of pivotal FORT Study in PKAN expected 3Q19

SAN DIEGO, Jan. 07, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today announced that, based on preliminary and unaudited financial data, the Company expects net product sales for the fourth quarter of 2018 to be approximately \$44 million. For the fiscal year 2018, the Company expects total net product sales of approximately \$164 million. The Company also provided a general update on its development programs, including anticipated milestones for 2019.

"With key data readouts from the FORT Study of fosmetpantotenate in PKAN and the CNSA-001 Phase 2 proof-of-concept study expected in 2019, Retrophin is positioned to create significant value for patients and shareholders this year," said Stephen Aselage, former chief executive officer of Retrophin. "The Company closed 2018 with the initiation of the PROTECT Study of sparsentan in IgA nephropathy, our third pivotal development program, continued growth of the base commercial business and most recently hired its new president and chief executive officer, Eric Dube, Ph.D. These clinical and commercial achievements provide strong momentum entering the new year, and I look forward to working with Eric to ensure a seamless transition to drive our continued success."

### Program Updates and Anticipated 2019 Milestones

- In December 2018, the pivotal Phase 3 FORT Study of fosmetpantotenate in pantothenate kinase-associated neurodegeneration (PKAN) completed patient enrollment. The FORT Study is designed to be registration-enabling in the U.S. and Europe, and top-line data are expected to become available in the third quarter of 2019.
- The Phase 2 proof-of-concept study evaluating CNSA-001 in patients with phenylketonuria (PKU) continues to progress, and top-line data are expected to be available in the first half of 2019. CNSA-001 is advancing under a joint development and option agreement with Censa Pharmaceuticals, and the Company expects to make a determination on its option to acquire Censa following the data readout.
- The New Drug Application (NDA) for a new, more patient-friendly formulation of Thiola is currently under review by the U.S. Food and Drug Administration (FDA) with an assigned Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2019. Pending approval, the Company expects to begin marketing the new formulation in the second half of 2019.
- Enrollment in the Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) continues. The Company expects to reach enrollment of 190 patients with FSGS during the second half of 2019, which would enable a top-line readout of the 36-week interim efficacy analysis of proteinuria in the second half of 2020. Successful achievement of the interim efficacy endpoint is expected to serve as the basis for submission of an NDA under the Subpart H accelerated approval pathway in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe.
- In December 2018, the Company announced that the first patient had been dosed in the PROTECT Study, a global, pivotal Phase 3 clinical trial evaluating the long-term nephroprotective potential of sparsentan for the treatment of IgA nephropathy (IgAN). The Company expects that the study will complete enrollment of approximately 280 patients with IgAN in the first half of 2021. Top-line data from the 36-week primary endpoint efficacy analysis of proteinuria is expected to become available in the first half of 2022. Retrophin expects that successful achievement of this endpoint will support submission of an NDA under the Subpart H accelerated approval pathway in the U.S., as well as an application for CMA consideration in Europe.

In late February, the Company expects to announce final financial results from the fourth quarter and full-year 2018, as well as provide a corporate update.

### 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 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. 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<sup>®</sup>,

Cholbam® and Thiola®.

[Retrophin.com](http://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. 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, 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 current or future clinical trials will not proceed as planned. Specifically, the Company faces the risk that the Phase 3 clinical trial of sparsentan in FSGS will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the Phase 3 clinical trial of sparsentan in IgAN will not demonstrate that sparsentan is safe or effective or serve as the basis for accelerated approval of sparsentan as planned; risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; and for each of its development programs and for its partner's CNSA-001 program, 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 and risk that the product candidates will not be approved for efficacy, safety, regulatory or other reasons. 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 the Company's 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; risks associated with its option to acquire Censa Pharmaceuticals and the CNSA-001 program; risk that the NDA for the new formulation of Thiola will not be approved by the FDA; and 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 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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