



## Retrophin Provides Corporate Update and 2018 Outlook

January 8, 2018

### **Preliminary full-year 2017 revenue of approximately \$155 million**

SAN DIEGO, Jan. 08, 2018 (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 2017 to be approximately \$42 million. For the fiscal year 2017, the Company expects total net product sales of approximately \$155 million. The Company also provided a general update on its development programs, including anticipated milestones for 2018.

"2018 will be an important year for Retrophin as we continue to build upon our recent progress, including the addition of CNSA-001 for the treatment of PKU to our development efforts and the expansion of sparsentan's clinical footprint into IgA nephropathy," said Stephen Aselage, chief executive officer of Retrophin. "We are also pleased to have closed out 2017 with a strong financial performance, providing additional resources as we continue to advance our key clinical programs towards NDA filing and potential commercialization. We look forward to executing on our clinical and business objectives throughout 2018 and beyond."

### **Program Updates and Anticipated 2018 Milestones**

- The Phase 3 FORT Study of fosmetpantotenate continues to enroll patients with pantothenate kinase-associated neurodegeneration (PKAN) and is on-track to complete enrollment in the second half of 2018.
- In response to a U.S. Food and Drug Administration (FDA) request, the Company has conducted additional statistical analyses to support its Phase 3 trial design and eligibility for the Subpart H accelerated approval pathway for sparsentan in focal segmental glomerulosclerosis (FSGS). The Company will resubmit its Phase 3 protocol for FSGS during the first quarter of 2018.
- Feasibility analyses and regulatory interaction for sparsentan in IgA nephropathy are underway with the expectation of initiating a clinical trial in 2018.
- CNSA-001 is expected to begin clinical development for phenylketonuria (PKU) in 2018 with results from a Phase 2 proof-of-concept study expected to be available in early 2019.
- The Company expects a New Drug Application filing in 2018 for its new formulation of Thiola<sup>®</sup> for the treatment of cystinuria, and anticipates marketing efforts to commence upon potential approval in 2019.

In late February, the Company expects to announce final financial results from the fourth quarter and full-year 2017, as well as provide a detailed corporate update.

### **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<sup>®</sup>, Cholbam<sup>®</sup> and Thiola<sup>®</sup>.

[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 planned clinical trials will not proceed as planned. Specifically, the Company faces the risk that the planned 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 fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; risk that the planned Phase 2 clinical trial of CNSA-001 will not demonstrate proof of concept in PKU; and risk that the product candidates will not be approved for efficacy, safety, regulatory or other reasons, and for each of the programs, 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. 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; 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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Source: Retrophin, Inc.