



## Retrophin Reports Second Quarter 2019 Financial Results

August 6, 2019

*Top-line results from pivotal Phase 3 FORT Study in PKAN on-track for 3Q 2019*

*Commercial launch for THIOLA<sup>®</sup> EC underway following FDA-approval for the treatment of cystinuria*

*Second quarter revenues increased eight percent to \$45 million*

SAN DIEGO, Aug. 06, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its second quarter 2019 financial results and provided a corporate update.

- The Phase 3 FORT Study evaluating fosmetpantotenate for the treatment of pantothenate kinase-associated neurodegeneration (PKAN) remains on-track to report top-line results in the third quarter of 2019, followed by anticipated submissions for a New Drug Application (NDA) in the U.S. and Marketing Authorization Application (MAA) in the EU in 2020
- The two pivotal Phase 3 studies to support potential registration of sparsentan continue to enroll patients with focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN)
- Following a strategic review of the CNSA-001 program in patients with phenylketonuria (PKU), the Company made the decision to decline to exercise its option to acquire Censa Pharmaceuticals and accordingly discontinue its joint development program for CNSA-001
- Sandra E. Poole joins Board of Directors; Ms. Poole brings more than 25 years of biopharmaceutical product development and manufacturing experience to Retrophin
- Commercial launch for THIOLA<sup>®</sup> EC (tiopronin) is underway following approval by U.S. Food and Drug Administration (FDA) for the treatment of cystinuria
- Net product sales for the second quarter of 2019 were \$44.7 million, compared to \$41.3 million for the same period in 2018
- Cash, cash equivalents and marketable securities, as of June 30, 2019, totaled \$425.9 million

"We are looking forward to the upcoming data read-out from our Phase 3 FORT Study this quarter, which if successful would support NDA and MAA submissions to potentially make fosmetpantotenate the first approved treatment for PKAN," said Eric Dube, Ph.D., chief executive officer of Retrophin. "Beyond fosmetpantotenate, our teams remain focused on execution and the goal of delivering new treatment options through the continued advancement of our two pivotal studies evaluating sparsentan for FSGS and IgA nephropathy, and the recent approval and launch of THIOLA EC for the treatment of cystinuria."

### Quarter Ended June 30, 2019

Net product sales for the second quarter of 2019 were \$44.7 million, compared to \$41.3 million for the same period in 2018. For the six months ended June 30, 2019, net product sales were \$84.3 million, compared to \$79.8 million for the same period in 2018. The increase in net product sales is attributable to growth across the Company's commercial products. Growth of the Company's net product sales for the full year 2019 is expected to be in line with the growth rate seen for the full year 2018.

Research and development (R&D) expenses for the second quarter of 2019 were \$37.9 million, compared to \$34.5 million for the same period in 2018. For the six months ended June 30, 2019, R&D expenses were \$71.4 million, compared to \$59.1 million for the same period in 2018. The difference is largely attributable to support of clinical and product development efforts related to fosmetpantotenate and sparsentan. On a non-GAAP adjusted basis, R&D expenses were \$35.8 million for the second quarter of 2019, compared to \$32.6 million for the same period in 2018.

Selling, general and administrative (SG&A) expenses for the second quarter of 2019 were \$39.0 million, compared to \$25.1 million for the same period in 2018. For the six months ended June 30, 2019, SG&A expenses were \$71.6 million, compared to \$51.6 million for the same period in 2018. The difference is largely attributable to increased headcount as a result of the Company's operational growth, and professional fees. On a non-GAAP adjusted basis, SG&A expenses were \$30.4 million for the second quarter of 2019, compared to \$16.9 million for the same period in 2018.

Total other expense for the second quarter of 2019 was \$2.1 million, compared to \$0.6 million for the same period in 2018. The difference is largely attributable to higher interest expense related to the Company's 2025 convertible notes issued in September 2018.

Net loss for the second quarter of 2019 was \$38.7 million, or \$0.92 per basic share, compared to \$22.3 million, or \$0.56 per basic share for the same period in 2018. For the six months ended June 30, 2019, net loss was \$79.7 million, compared to \$40.7 million for the same period in 2018. On a non-GAAP adjusted basis, net loss for the second quarter of 2019 was \$24.5 million, or \$0.58 per basic share, compared to a net loss of \$9.9 million, or \$0.25 per basic share for the same period in 2018.

As of June 30, 2019, the Company had cash, cash equivalents and marketable securities of \$425.9 million.

### Program Updates

#### *Fosmetpantotenate*

- Top-line data from the pivotal Phase 3 FORT Study are expected in the third quarter of 2019. The FORT Study is an international, pivotal clinical trial assessing the safety and efficacy of fosmetpantotenate in patients with PKAN. The primary endpoint in the study is the change from baseline in the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale through 24 weeks of treatment. After completing the 24-week treatment period, all patients are eligible to receive fosmetpantotenate as part of an open-label extension. The FORT Study is expected to be registration-enabling in the U.S. and Europe and is being conducted under a Special Protocol Assessment (SPA) agreement, which indicates concurrence by the FDA that, if the data are positive, the design of the trial can adequately support the filing of an NDA.

#### *Sparsentan*

- The Company continues to enroll patients with FSGS in the pivotal Phase 3 DUPLEX Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of sparsentan in approximately 300 patients. The DUPLEX Study protocol provides for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint – the proportion of patients achieving a FSGS partial remission of proteinuria endpoint (FPRE), which is defined as urine protein-to-creatinine ratio (Up/C)  $\leq 1.5$  g/g and a  $>40$  percent reduction in Up/C from baseline, at Week 36. While the confirmatory endpoint of the study is the change in slope of estimated glomerular filtration rate (eGFR) after 108 weeks of treatment, successful achievement of the interim 36-week proteinuria 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. Top-line efficacy data from the 36-week proteinuria endpoint analysis are expected in the first half of 2021.
- The PROTECT Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled pivotal Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 280 patients with IgAN, continues to enroll. The primary efficacy endpoint in the PROTECT Study is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. Successful achievement of this endpoint is expected to 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. Secondary efficacy endpoints include change in eGFR from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Top-line efficacy data from the 36-week proteinuria endpoint analysis are expected in the first half of 2022.

#### *CNSA-001*

- Following a strategic review of the CNSA-001 program in patients with phenylketonuria (PKU), the Company made the decision to decline to exercise its option to acquire Censa Pharmaceuticals and accordingly discontinue its joint development program for CNSA-001.

#### *Alagille Syndrome CRADA*

- In the second quarter of 2019, the Company entered into a three-way Cooperative Research and Development Agreement (CRADA) with the National Institutes of Health's National Center for Advancing Translational Sciences (NCATS) and patient advocacy foundation Alagille Syndrome Alliance to collaborate on research efforts aimed at the identification and development of potential therapeutics for Alagille syndrome. This CRADA broadens Retrophin's innovative approach to multi-stakeholder partnerships in an effort to drive early research advancements for rare diseases.

#### *Thiola EC*

- On June 28, 2019 the Company announced that the FDA approved 100 mg and 300 mg tablets of THIOLA EC, a new enteric-coated formulation of THIOLA<sup>®</sup> (tiopronin), to be used for the treatment of cystinuria. THIOLA EC offers the potential for administration with or without food, and the ability to reduce the number of tablets necessary to manage cystinuria. THIOLA EC became available to patients in July of 2019.

#### **Conference Call Information**

Retrophin will host a conference call and webcast today, Tuesday, August 6, 2019 at 4:30 p.m. ET to discuss company updates as well as second quarter 2019 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 4587975 shortly before 4:30 p.m. ET. The webcast can be accessed at [retrophin.com](http://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 7:30 p.m. ET, August 6, 2019 to 7:30 p.m. ET, August 13, 2019. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 4587975.

#### **Use of Non-GAAP Financial Measures**

To supplement Retrophin's financial results and guidance presented in accordance with U.S. generally accepted accounting principles (GAAP), the Company uses certain non-GAAP adjusted financial measures in this press release and the accompanying tables. The Company believes that these non-GAAP financial measures are helpful in understanding its past financial performance and potential future results. They are not meant to be considered in isolation or as a substitute for comparable GAAP measures, and should be read in conjunction with the consolidated financial statements prepared in accordance with GAAP. Retrophin's management regularly uses these supplemental non-GAAP financial measures internally to understand, manage and evaluate its business and make operating decisions. In addition, Retrophin believes that the use of these non-GAAP measures enhances the ability of investors to compare its results from period to period and allows for greater transparency with respect to key financial metrics the Company uses in making operating decisions.

Investors should note that these non-GAAP financial measures are not prepared under any comprehensive set of accounting rules or principles and do not reflect all of the amounts associated with the Company's results of operations as determined in accordance with GAAP. Investors should also note that these non-GAAP financial measures have no standardized meaning prescribed by GAAP and, therefore, have limits in their usefulness to investors. In addition, from time to time in the future the Company may exclude other items, or cease to exclude items that it has historically excluded, for purposes of its non-GAAP financial measures; because of the non-standardized definitions, the non-GAAP financial measures as used by the Company in this press release and the accompanying tables may be calculated differently from, and therefore may not be directly comparable to, similarly titled measures used by the Company's competitors and other companies.

As used in this press release, (i) the historical non-GAAP net income (loss) measures exclude from GAAP net income (loss), as applicable, stock-based compensation expense, amortization and depreciation expense, revaluation of acquisition related contingent consideration and income tax; (ii) the historical non-GAAP SG&A expense measures exclude from GAAP SG&A expenses, as applicable, stock-based compensation expense, and amortization and depreciation expense; (iii) the historical non-GAAP R&D expense measures exclude from GAAP R&D expenses, as applicable, stock-based compensation expense, and depreciation and amortization expense.

### **About THIOLA<sup>®</sup> EC (tiopronin)**

THIOLA<sup>®</sup> EC (tiopronin) is indicated, in combination with high fluid intake, alkali, and diet modification for the prevention of cystine stone formation in adults and pediatric patients  $\geq 20$  kg with severe homozygous cystinuria, who are not responsive to these measures alone.

Patients and physicians can access additional information about THIOLA EC by visiting [thiola.com](http://thiola.com).

### **Important Safety Information**

#### *Contraindications:*

THIOLA EC is contraindicated in patients with hypersensitivity to tiopronin or any other components of THIOLA EC.

#### *Warnings and precautions:*

**Proteinuria:** Proteinuria, including nephrotic syndrome and membranous nephropathy, have been reported with tiopronin use. Pediatric patients receiving  $>50$  mg/kg of tiopronin per day may be at increased risk for proteinuria. Monitor patients for the development of proteinuria and discontinue therapy in patients who develop proteinuria.

**Hypersensitivity Reactions:** Hypersensitivity reactions (drug fever, rash, fever, arthralgia and lymphadenopathy) have been reported.

#### *Adverse Reactions:*

The most common adverse reactions ( $\geq 10\%$ ) are nausea, diarrhea or soft stools, oral ulcers, rash, fatigue, fever, arthralgia, proteinuria, and emesis.

#### *Drug interactions:*

Avoid alcohol consumption 2 hours before and 3 hours after taking THIOLA EC.

#### *Special populations:*

**Lactation:** Breastfeeding is not recommended during treatment with THIOLA EC.

**Geriatric Use:** Because elderly patients are more likely to have decreased renal function, care should be taken in dose selection, and it may be useful to monitor renal function.

You may report negative side effects to Retrophin Medical Information at 1-877-659-5518, or to the FDA at 1-800-FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch). Please see full Prescribing Information for Important Safety Information at [thiola.com](http://thiola.com).

### **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. 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 commercial 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 clinical trials will not proceed as planned. Specifically, the Company faces the risk that the upcoming data read-out from the Phase 3 FORT Study will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; 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; and for each of its development programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing clinical trials may not proceed on expected timelines 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 regulatory interactions; and risks and uncertainties relating to competitive products, including potential generic competition with certain of the Company's 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-Q, Form 10-K and other filings with the Securities and Exchange Commission.

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONSOLIDATED BALANCE SHEETS**  
*(in thousands, except share amounts)*

	<b>June 30, 2019</b>	<b>December 31, 2018</b>
	(unaudited)	
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 75,657	\$ 102,873
Marketable securities	350,243	368,668
Accounts receivable, net	15,451	12,662
Inventory, net	5,050	5,619
Prepaid expenses and other current assets	9,164	4,140
Prepaid taxes	1,450	1,716
<b>Total current assets</b>	<b>457,015</b>	<b>495,678</b>
Property and equipment, net	3,015	3,146
Other non-current assets	12,702	7,709
Investment-equity	15,000	15,000
Intangible assets, net	158,906	186,691
Goodwill	936	936
<b>Total assets</b>	<b>\$ 647,574</b>	<b>\$ 709,160</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 12,519	\$ 6,954
Accrued expenses	50,557	49,695
Other current liabilities	8,417	6,165
Business combination-related contingent consideration	19,094	19,350
2019 Convertible debt	—	22,457
<b>Total current liabilities</b>	<b>90,587</b>	<b>104,621</b>
2025 Convertible debt	199,891	195,091
Other non-current liabilities	22,102	17,545

Business combination-related contingent consideration, less current portion	57,905	73,650
<b>Total liabilities</b>	<u>370,485</u>	<u>390,907</u>
<b>Stockholders' Equity:</b>		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2019 and December 31, 2018	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 42,899,318 and 41,389,524 issued and outstanding as of June 30, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	625,999	589,795
Accumulated deficit	(349,695)	(270,017)
Accumulated other comprehensive income (loss)	781	(1,529)
Total stockholders' equity	<u>277,089</u>	<u>318,253</u>
<b>Total liabilities and stockholders' equity</b>	<u>\$ 647,574</u>	<u>\$ 709,160</u>

**Note:** Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONSOLIDATED STATEMENT OF OPERATIONS**  
*(in thousands, except share and per share data)*  
*(unaudited)*

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	<u>2019</u>	<u>2018</u>	<u>2019</u>	<u>2018</u>
Net product sales:				
Thiola	\$ 23,778	\$ 22,743	\$ 44,958	\$ 42,667
Bile acid products	20,929	18,594	39,319	37,102
Total net product sales	<u>44,707</u>	<u>41,337</u>	<u>84,277</u>	<u>79,769</u>
Operating expenses:				
Cost of goods sold	979	1,178	1,996	2,791
Research and development	37,934	34,460	71,377	59,096
Selling, general and administrative	38,970	25,100	71,639	51,568
Change in fair value of contingent consideration	3,353	2,159	6,522	5,786
Impairment of L-UDCA IPR&D intangible asset	—	—	25,500	—
Write off of L-UDCA contingent consideration	—	—	(18,000)	—
Total operating expenses	<u>81,236</u>	<u>62,897</u>	<u>159,034</u>	<u>119,241</u>
Operating loss	<u>(36,529)</u>	<u>(21,560)</u>	<u>(74,757)</u>	<u>(39,472)</u>
Other income (expenses), net:				
Other income (expense), net	125	(403)	(177)	(282)
Interest income	2,589	858	5,408	1,655
Interest expense	(4,817)	(1,057)	(9,682)	(2,212)
Total other expense, net	<u>(2,103)</u>	<u>(602)</u>	<u>(4,451)</u>	<u>(839)</u>
Loss before income taxes	<u>(38,632)</u>	<u>(22,162)</u>	<u>(79,208)</u>	<u>(40,311)</u>
Income tax expense	<u>(69)</u>	<u>(167)</u>	<u>(470)</u>	<u>(396)</u>
Net loss	<u>\$ (38,701)</u>	<u>\$ (22,329)</u>	<u>\$ (79,678)</u>	<u>\$ (40,707)</u>

**Per share data:**

Basic and diluted net loss per common share:	\$ (0.92)	\$ (0.56)	\$ (1.91)	\$ (1.03)
Basic and diluted weighted average common shares outstanding:	41,957,860	40,061,045	41,685,599	39,641,334

**Note: Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.**

**RETROPHIN, INC. AND SUBSIDIARIES**  
**RECONCILIATION OF GAAP REPORTED TO NON-GAAP ADJUSTED INFORMATION**  
*(in thousands, except share and per share data)*  
*(unaudited)*

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
<b>GAAP operating loss</b>	<b>(36,529)</b>	<b>(21,560)</b>	<b>(74,757)</b>	<b>(39,472)</b>
R&D operating expense	(37,934)	(34,460)	(71,377)	(59,096)
Stock compensation	1,896	1,582	3,566	2,989
Amortization & depreciation	288	289	574	392
Subtotal non-GAAP items	2,184	1,871	4,140	3,381
Non-GAAP R&D expense	(35,750)	(32,589)	(67,237)	(55,715)
SG&A operating expense	(38,970)	(25,100)	(71,639)	(51,568)
Stock compensation	3,852	3,844	8,702	7,046
Amortization & depreciation	4,740	4,354	9,355	8,599
Subtotal non-GAAP items	8,592	8,198	18,057	15,645
Non-GAAP SG&A expense	(30,378)	(16,902)	(53,582)	(35,923)
Change in fair value of contingent consideration	3,353	2,159	6,522	5,786
Subtotal non-GAAP items	14,129	12,228	28,719	24,812
<b>Non-GAAP operating loss</b>	<b>\$ (22,400)</b>	<b>\$ (9,332)</b>	<b>\$ (46,038)</b>	<b>\$ (14,660)</b>
<b>GAAP net loss</b>	<b>\$ (38,701)</b>	<b>\$ (22,329)</b>	<b>\$ (79,678)</b>	<b>\$ (40,707)</b>
Non-GAAP operating loss adjustments	14,129	12,228	28,719	24,812
Income tax provision	69	167	470	396
<b>Non-GAAP net loss</b>	<b>\$ (24,503)</b>	<b>\$ (9,934)</b>	<b>\$ (50,489)</b>	<b>\$ (15,499)</b>
<b>Per share data:</b>				
Net loss per common share, basic	\$ (0.58)	\$ (0.25)	\$ (1.21)	\$ (0.39)
Weighted average common shares outstanding, basic	41,957,860	40,061,045	41,685,599	39,641,334

**Note: Certain adjustments / reclassifications have been made to prior periods to conform to current year presentation.**

Contact:  
Chris Cline, CFA  
Vice President, Investor Relations & Corporate Communications  
888-969-7879  
[IR@retrophin.com](mailto:IR@retrophin.com)



Source: Retrophin, Inc.