



## Retrophin Reports Third Quarter 2019 Financial Results

October 30, 2019

Enrollment on-track in pivotal Phase 3 clinical trials to support registration of sparsentan for patients with FSGS and IgAN

Third quarter revenues rose nine percent to \$44 million

SAN DIEGO, Oct. 30, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its third quarter 2019 financial results and provided a corporate update.

- Enrollment in the pivotal Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) remains on track to enable topline data from the 36-week proteinuria analysis in the first half of 2021
- The pivotal Phase 3 PROTECT Study of sparsentan continues to enroll patients with IgA nephropathy (IgAN) and is on schedule to deliver topline data from the 36-week proteinuria analysis in the first half of 2022
- Peter Heerma appointed chief commercial officer; Mr. Heerma brings more than 20 years of global experience launching best-in-class therapies, managing pipeline and on-market product portfolios, and leading commercial and cross-functional teams
- Net product sales for the third quarter of 2019 were \$44.4 million, compared to \$40.7 million for the same period in 2018
- Cash, cash equivalents and marketable securities, as of September 30, 2019, totaled \$407.0 million

"In the third quarter our team delivered a strong performance with continued enrollment of our two pivotal studies of sparsentan, and a well-executed start to the THIOLA<sup>®</sup> EC launch, underscoring our commercial team's capabilities and our commitment to serving patients living with rare disease," said Eric Dube, Ph.D., chief executive officer of Retrophin. "We also recently announced the appointment of Peter Heerma as our first chief commercial officer. Peter's extensive experience in nephrology and depth in developing successful commercialization strategies in the U.S. and Europe will be instrumental as we focus on maximizing sparsentan's future potential to become a new treatment standard in rare kidney disorders and continuing the organic growth of our commercial portfolio."

### Quarter Ended September 30, 2019

Net product sales for the third quarter of 2019 were \$44.4 million, compared to \$40.7 million for the same period in 2018. For the nine months ended September 30, 2019, net product sales were \$128.7 million, compared to \$120.5 million for the same period in 2018. The increase in net product sales is attributable to growth across the Company's commercial products including the ongoing launch of THIOLA EC. 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 third quarter of 2019 were \$33.2 million, compared to \$32.4 million for the same period in 2018. For the nine months ended September 30, 2019, R&D expenses were \$104.6 million, compared to \$91.5 million for the same period in 2018. The difference is largely attributable to increased support of clinical and product development efforts. On a non-GAAP adjusted basis, R&D expenses were \$31.2 million for the third quarter of 2019, compared to \$30.6 million for the same period in 2018.

Selling, general and administrative (SG&A) expenses for the third quarter of 2019 were \$29.8 million, compared to \$26.1 million for the same period in 2018. For the nine months ended September 30, 2019, SG&A expenses were \$101.4 million, compared to \$77.7 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 \$22.3 million for the third quarter of 2019, compared to \$18.3 million for the same period in 2018.

In the third quarter of 2019, the Company incurred a \$15.0 million impairment of long-term investment related to the previously announced decision to discontinue the joint development program for CNSA-001 with Censa Pharmaceuticals.

Total other expense for the third quarter of 2019 was \$2.6 million, compared to \$18.5 million for the same period in 2018. The difference is largely attributable to a loss on early extinguishment of debt related to the repurchase of outstanding convertible notes due 2019 effected in September 2018.

Net loss for the third quarter of 2019 was \$36.5 million, or \$0.85 per basic share, compared to \$54.5 million, or \$1.34 per basic share for the same period in 2018. For the nine months ended September 30, 2019, net loss was \$116.2 million, compared to \$95.2 million for the same period in 2018. On a non-GAAP adjusted basis, net loss for the third quarter of 2019 was \$28.2 million, or \$0.66 per basic share, compared to a net loss of \$27.8 million, or \$0.68 per basic share for the same period in 2018.

As of September 30, 2019, the Company had cash, cash equivalents and marketable securities of \$407.0 million.

### Program Updates

#### *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 a New Drug Application (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.
- At the upcoming American Society of Nephrology (ASN) Kidney Week 2019, the Company will present new data from the Phase 2 DUET Study examining the impact of sparsentan on quality of life in patients with FSGS, as well as preclinical findings exploring the potential effect of sparsentan in Alport syndrome. ASN Kidney Week 2019 is being held November 5–10, 2019, in Washington DC.

#### *Fosmetpantotenate*

- In the third quarter of 2019, the Company reported that the Phase 3 FORT Study evaluating the safety and efficacy of fosmetpantotenate compared to placebo in patients with pantothenate kinase-associated neurodegeneration (PKAN) did not meet its primary or secondary endpoints. Based upon the results of the trial, Retrophin has discontinued future development and commercial preparation activities for the fosmetpantotenate program.

#### **Conference Call Information**

Retrophin will host a conference call and webcast today, Wednesday, October 30, 2019 at 4:30 p.m. ET to discuss company updates as well as third 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 4153049 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, October 30, 2019 to 6:30 p.m. ET, November 6, 2019. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 4153049.

#### **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 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<sup>®</sup>, Cholbam<sup>®</sup>, Thiola<sup>®</sup> and Thiola EC<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 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)

	September 30, 2019 (unaudited)	December 31, 2018
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 65,188	\$ 102,873
Marketable securities	341,835	368,668
Accounts receivable, net	16,781	12,662
Inventory, net	5,264	5,619
Prepaid expenses and other current assets	8,628	4,140
Prepaid taxes	2,022	1,716
<b>Total current assets</b>	<b>439,718</b>	<b>495,678</b>
Property and equipment, net	2,910	3,146
Other non-current assets	12,453	7,709
Investment-equity	—	15,000
Intangible assets, net	157,799	186,691
Goodwill	936	936
<b>Total assets</b>	<b>\$ 613,816</b>	<b>\$ 709,160</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 13,832	\$ 6,954

Accrued expenses	47,874	49,695
Other current liabilities	8,411	6,165
Business combination-related contingent consideration	17,900	19,350
2019 Convertible debt	—	22,457
<b>Total current liabilities</b>	<b>88,017</b>	<b>104,621</b>
2025 Convertible debt	202,355	195,091
Other non-current liabilities	21,487	17,545
Business combination-related contingent consideration, less current portion	56,000	73,650
<b>Total liabilities</b>	<b>367,859</b>	<b>390,907</b>

**Stockholders' Equity:**

Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2019 and December 31, 2018	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 42,958,401 and 41,389,524 issued and outstanding as of September 30, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	630,966	589,795
Accumulated deficit	(386,185)	(270,017)
Accumulated other comprehensive income (loss)	1,172	(1,529)
Total stockholders' equity	245,957	318,253
<b>Total liabilities and stockholders' equity</b>	<b>\$ 613,816</b>	<b>\$ 709,160</b>

**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)*

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
	<i>(unaudited)</i>			
Net product sales:				
Thiola	\$ 24,435	\$ 22,654	\$ 69,393	\$ 65,322
Bile acid products	19,938	18,052	59,258	55,153
Total net product sales	44,373	40,706	128,651	120,475
Operating expenses:				
Cost of goods sold	1,513	1,133	3,509	3,924
Research and development	33,220	32,448	104,597	91,544
Selling, general and administrative	29,779	26,107	101,418	77,675
Change in fair value of contingent consideration	(702)	16,601	5,820	22,387
Impairment of L-UDCA IPR&D intangible asset	—	—	25,500	—
Write off of L-UDCA contingent consideration	—	—	(18,000)	—
Impairment of long-term investment	15,000	—	15,000	—
Total operating expenses	78,810	76,289	237,844	195,530
Operating loss	(34,437)	(35,583)	(109,193)	(75,055)

Other income (expenses), net:				
Other expense, net	(496)	(90)	(673)	(372)
Interest income	2,467	1,147	7,875	2,905
Interest expense	(4,547)	(2,533)	(14,230)	(4,848)
Loss on early extinguishment of debt	—	(17,042)	—	(17,042)
Total other expense, net	<u>(2,576)</u>	<u>(18,518)</u>	<u>(7,028)</u>	<u>(19,357)</u>
Loss before income taxes	(37,013)	(54,101)	(116,221)	(94,412)
Income tax benefit (expense)	<u>523</u>	<u>(415)</u>	<u>53</u>	<u>(811)</u>
Net loss	<u>\$ (36,490)</u>	<u>\$ (54,516)</u>	<u>\$ (116,168)</u>	<u>\$ (95,223)</u>

**Per share data:**

Basic and diluted net loss per common share:	<u>\$ (0.85)</u>	<u>\$ (1.34)</u>	<u>\$ (2.76)</u>	<u>\$ (2.37)</u>
Basic and diluted weighted average common shares outstanding:	<u>42,943,828</u>	<u>40,717,440</u>	<u>42,109,618</u>	<u>40,149,184</u>

**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 September 30,		Nine Months Ended September 30,	
	2019	2018	2019	2018
<b>GAAP operating loss</b>	<b><u>(34,437)</u></b>	<b><u>(35,583)</u></b>	<b><u>(109,193)</u></b>	<b><u>(75,055)</u></b>
R&D operating expense	(33,220)	(32,448)	(104,597)	(91,544)
Stock compensation	1,735	1,603	5,301	4,592
Amortization & depreciation	293	292	867	684
Subtotal non-GAAP items	<u>2,028</u>	<u>1,895</u>	<u>6,168</u>	<u>5,276</u>
Non-GAAP R&D expense	<u>(31,192)</u>	<u>(30,553)</u>	<u>(98,429)</u>	<u>(86,268)</u>
SG&A operating expense	(29,779)	(26,107)	(101,418)	(77,675)
Stock compensation	2,605	3,282	11,307	10,328
Amortization & depreciation	4,896	4,506	14,251	13,105
Subtotal non-GAAP items	<u>7,501</u>	<u>7,788</u>	<u>25,558</u>	<u>23,433</u>
Non-GAAP SG&A expense	<u>(22,278)</u>	<u>(18,319)</u>	<u>(75,860)</u>	<u>(54,242)</u>
Change in fair value of contingent consideration	<u>(702)</u>	<u>16,601</u>	<u>5,820</u>	<u>22,387</u>
Subtotal non-GAAP items	<u>8,827</u>	<u>26,284</u>	<u>37,546</u>	<u>51,096</u>
<b>Non-GAAP operating loss</b>	<b><u>\$ (25,610)</u></b>	<b><u>\$ (9,299)</u></b>	<b><u>\$ (71,647)</u></b>	<b><u>\$ (23,959)</u></b>
<b>GAAP net loss</b>	<b><u>\$ (36,490)</u></b>	<b><u>\$ (54,516)</u></b>	<b><u>\$ (116,168)</u></b>	<b><u>\$ (95,223)</u></b>
Non-GAAP operating loss adjustments	8,827	26,284	37,546	51,096

Income tax provision	(523)	415	(53)	811
<b>Non-GAAP net loss</b>	<b>\$ (28,186)</b>	<b>\$ (27,817)</b>	<b>\$ (78,675)</b>	<b>\$ (43,316)</b>

**Per share data:**

Net loss per common share, basic	\$ (0.66)	\$ (0.68)	\$ (1.87)	\$ (1.08)
Weighted average common shares outstanding, basic	42,943,828	40,717,440	42,109,618	40,149,184

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

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Source: Retrophin, Inc.