



## Retrophin Reports Third Quarter 2017 Financial Results

November 7, 2017

### Phase 3 FORT Study of fosmetpantotenate continues to enroll PKAN patients Third quarter revenues increased 19 percent to \$40 million

SAN DIEGO, CALIFORNIA -- (Marketwired) -- 11/07/17 -- Retrophin, Inc. (NASDAQ:RTRX) today reported its third quarter 2017 financial results and provided a corporate update.

- The Phase 3 FORT Study of fosmetpantotenate in pantothenate kinase-associated neurodegeneration (PKAN) remains on-track to complete enrollment in the second half of 2018
- The Company received regulatory feedback on its Phase 3 protocol for sparsentan in FSGS; additional statistical analyses to be provided while trial start-up activities continue
- Net product sales for the third quarter of 2017 were \$40.3 million, compared to \$33.9 million for the same period in 2016
- Cash, cash equivalents and marketable securities, as of September 30, 2017, totaled \$303.9 million

"We delivered strong development, commercial, and operational results in the third quarter and continue to build upon our momentum as we enter the end of 2017," said Stephen Aselage, chief executive officer of Retrophin. "Retrophin is poised to make a meaningful impact in the rare disease community and accelerate our growth as we advance our pivotal programs for fosmetpantotenate, which continues to enroll PKAN patients in the FORT Study, and sparsentan, where clinical site preparations continue in parallel with our discussions with the FDA on the design of our Phase 3 study in FSGS."

#### **Quarter Ended September 30, 2017**

Net product sales for the third quarter of 2017 were \$40.3 million, compared to \$33.9 million for the same period in 2016. For the nine months ended September 30, 2017, net product sales were \$112.8 million, compared to \$96.3 million for the same period in 2016. The increase in net product sales is attributable to growth across the Company's commercial products: Chenoda<sup>®</sup>, Cholbam<sup>®</sup> and Thiola<sup>®</sup>. The Company reiterates its full-year 2017 guidance of \$150.0 to \$160.0 million in net product sales.

Research and development (R&D) expenses for the third quarter of 2017 were \$19.6 million, compared to \$18.4 million for the same period in 2016. For the nine months ended September 30, 2017, R&D expenses were \$58.6 million, compared to \$50.8 million for the same period in 2016. The difference is largely attributable to increased support of non-clinical and clinical efforts related to fosmetpantotenate and sparsentan. On a non-GAAP adjusted basis, R&D expenses were \$17.5 million for the third quarter of 2017, compared to \$15.4 million for the same period in 2016.

Selling, general and administrative (SG&A) expenses for the third quarter of 2017 were \$24.9 million, compared to \$23.5 million for the same period in 2016. For the nine months ended September 30, 2017, SG&A expenses were \$74.7 million, compared to \$65.7 million for the same period in 2016. The difference is largely attributable to an increase in headcount to support the Company's commercial and operational growth. On a non-GAAP adjusted basis, SG&A expenses were \$15.4 million for the third quarter of 2017, compared to \$14.6 million for the same period in 2016.

Total other expense for the third quarter of 2017 was \$8.4 million, compared to \$10.3 million for the same period in 2016. For the nine months ended September 30, 2017, total other expense was \$8.7 million, compared to \$5.3 million for the same period in 2016. The decrease in the third quarter resulted from a lower adjustment in the fair value of derivative instruments due to changes in the Company's stock price.

Net loss for the third quarter of 2017 was \$17.8 million, or \$0.46 per basic share, compared to \$37.1 million, or \$1.00 per basic share for the same period in 2016. For the nine months ended September 30, 2017, net loss was \$42.1 million, compared to \$39.3 million for the same period in 2016. On a non-GAAP adjusted basis, net income for the third quarter of 2017 was \$5.9 million, or \$0.15 per basic share, compared to a net loss of \$3.4 million, or \$0.09 per basic share for the same period in 2016.

As of September 30, 2017, the Company had cash, cash equivalents and marketable securities of \$303.9 million.

#### **Program Updates**

##### *Fosmetpantotenate (RE-024)*

- The Company continues to enroll patients with PKAN in the FORT Study, an international, registrational Phase 3 clinical trial assessing the safety and efficacy of fosmetpantotenate in approximately 82 patients with PKAN aged 6 to 65 years. 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 will be 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 the design of the trial can adequately support the filing of a New Drug Application (NDA). Enrollment in the study is expected to complete in the second half of 2018.
- Four patients with PKAN receiving fosmetpantotenate under physician-initiated treatment outside of the U.S. continue to

receive therapy and remain stable.

- In October 2017, the Company presented new data from physician-initiated treatment at the Child Neurology Society's 26th Annual Meeting. Key findings showed that 30-month treatment with fosmetpantotenate in a single patient with PKAN was associated with persistent improvement of the patient's functioning.

#### *Sparsentan*

- Following an End of Phase 2 meeting with the FDA in the first quarter of 2017, the Company announced plans to initiate a pivotal Phase 3 clinical trial of sparsentan in FSGS. The study is expected to include an interim analysis of proteinuria to serve as the basis for an NDA filing for Subpart H accelerated approval of sparsentan. The confirmatory endpoint of the study is expected to compare changes from baseline in estimated glomerular filtration rate (eGFR), which is widely regarded as the best overall measure of kidney function. In the third quarter of 2017, the Company submitted its Phase 3 protocol for review to the FDA, and on November 6, 2017, received feedback from the Agency requesting additional statistical analyses to support the trial design's eligibility for the Subpart H pathway. Study start-up activities continue in anticipation of initiating the pivotal trial in 2018.
- In November 2017, the Company presented new positive data from the ongoing open-label extension of the Phase 2 DUET study of sparsentan at ASN Kidney Week 2017. Key findings suggested FSGS patients treated with sparsentan over 48 weeks achieved progressive reduction in proteinuria combined with stable eGFR. Sparsentan also continued to be generally safe and well-tolerated in the open-label period. In addition, the Company presented results of pharmacokinetics and pharmacodynamics analyses from the DUET study which support the use of 800 mg of sparsentan as a target dose for reduction of proteinuria in FSGS.

#### *NGLY1 Deficiency*

- In the third quarter of 2017, 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 NGLY1.org to collaborate on research efforts aimed at the identification of potential small molecule therapeutics for NGLY1 Deficiency. The research collaboration will focus on the development of assays for small molecule high-throughput screening in an effort to better understand the biology of the disorder and identify potential small molecules to be developed as a therapeutic for patients living with NGLY1 deficiency.

#### *Thiola*

- On November 3, 2017, the Company amended its agreement with the manufacturer of Thiola to extend the term of the current exclusive U.S. and Canada licensing agreement by an additional five years to 2029.

#### **Conference Call Information**

Retrophin will host a conference call and webcast today, Tuesday, November 7, 2017 at 4:30 p.m. ET to discuss development updates and third quarter 2017 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 3889648 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, November 7, 2017 to 7:30 p.m. ET, November 14, 2017. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 3889648.

#### **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, revaluation of acquisition related contingent consideration, stock-based compensation expense, depreciation and amortization expense, change in fair value of derivative instruments; income tax benefit; (ii) the historical non-GAAP SG&A expense measures exclude from GAAP SG&A expenses, as applicable,

stock-based compensation expense, and depreciation and amortization 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 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), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research exploring 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 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 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; and risk that the Company's 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.

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

	<b>September 30, 2017</b>	<b>December 31, 2016</b>
	(unaudited)	
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 98,991	\$ 41,002
Marketable securities	204,882	214,871
Accounts receivable, net	14,543	18,510
Inventory, net	4,318	2,826
Prepaid expenses and other current assets	2,605	4,831
Prepaid taxes	-	3,463
Note receivable, current	-	46,849
<b>Total current assets</b>	<b>325,339</b>	<b>332,352</b>
Property and equipment, net	2,717	2,587
Other assets	7,101	7,364
Intangible assets, net	179,569	182,043
Goodwill	936	936
Long term deferred tax asset	4,848	-
<b>Total assets</b>	<b>\$ 520,510</b>	<b>\$ 525,282</b>

**Liabilities and Stockholders' Equity****Current liabilities:**

Accounts payable	\$	7,370	\$	7,522
Accrued expenses		32,253		33,308
Other current liabilities		4,048		1,842
Guaranteed minimum royalty		2,000		2,000
Tax payable		1,152		-
Business combination-related contingent consideration		16,941		16,150
Derivative financial instruments, warrants		20,140		22,440
<b>Total current liabilities</b>		<b>83,904</b>		<b>83,262</b>

Convertible debt		44,911		44,422
Other non-current liabilities		3,808		4,010
Guaranteed minimum royalty, less current portion		7,393		8,068
Business combination-related contingent consideration, less current portion		75,974		71,328
Deferred income tax liability, net		-		6,425
<b>Total liabilities</b>		<b>215,990</b>		<b>217,515</b>

**Stockholders' Equity:**

Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2017 and December 31, 2016		-		-
Common stock \$0.0001 par value; 100,000,000 shares authorized; 39,280,702 and 37,906,669 issued and outstanding as of September 30, 2017 and December 31, 2016, respectively		4		4
Additional paid-in capital		465,148		421,309
Accumulated deficit		(160,037)		(113,056)
Accumulated other comprehensive loss		(595)		(490)
<b>Total stockholders' equity</b>		<b>304,520</b>		<b>307,767</b>
<b>Total liabilities and stockholders' equity</b>	<b>\$</b>	<b>520,510</b>	<b>\$</b>	<b>525,282</b>

**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,	
	2017	2016	2017	2016
Net product sales	\$ 40,340	\$ 33,945	\$ 112,760	\$ 96,265
Operating expenses:				
Cost of goods sold	925	1,573	2,431	3,351
Research and development	19,610	18,414	58,592	50,758
Selling, general and administrative	24,852	23,466	74,683	65,714
Legal fee settlement	-	5,212	2,000	5,212
Change in fair value of contingent consideration	4,429	5,256	11,057	10,741
Restructuring	1,132	396	2,611	481
Total operating expenses	50,948	54,317	151,374	136,257
Operating loss	(10,608)	(20,372)	(38,614)	(39,992)
Other income (expenses), net:				
Other income, net	557	151	1,065	156
Interest expense, net	(65)	(299)	(855)	(609)

Change in fair value of derivative instruments	(8,901 )	(10,126 )	(8,921 )	(4,849 )
Total other expense, net	(8,409 )	(10,274 )	(8,711 )	(5,302 )
Loss before provision for income taxes	(19,017 )	(30,646 )	(47,325 )	(45,294 )
Income tax benefit (expense)	1,223	(6,467 )	5,212	5,994
Net loss	\$ (17,794 )	\$ (37,113 )	\$ (42,113 )	\$ (39,300 )
Net loss per common share:				
Basic	\$ (0.46 )	\$ (1.00 )	\$ (1.10 )	\$ (1.07 )
Diluted	\$ (0.46 )	\$ (1.00 )	\$ (1.10 )	\$ (1.07 )
Weighted average common shares outstanding:				
Basic	38,654,086	36,980,356	38,301,893	36,728,911
Diluted	38,654,086	36,980,356	38,301,893	36,728,911

**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,	
	2017	2016	2017	2016
<b>GAAP operating loss</b>	<b>\$ (10,608 )</b>	<b>\$ (20,372 )</b>	<b>\$ (38,614 )</b>	<b>\$ (39,992 )</b>
R&D operating expense	(19,610 )	(18,414 )	(58,592 )	(50,758 )
Stock compensation	1,998	2,935	7,113	8,061
Amortization & depreciation	83	82	245	246
Subtotal non-GAAP items	2,081	3,017	7,358	8,307
Non-GAAP R&D expense	(17,529 )	(15,397 )	(51,234 )	(42,451 )
SG&A operating expense	(24,852 )	(23,466 )	(74,683 )	(65,714 )
Stock compensation	4,962	4,814	14,179	13,973
Amortization & depreciation	4,533	4,013	13,092	11,708
Subtotal non-GAAP items	9,495	8,827	27,271	25,681
Non-GAAP SG&A expense	(15,357 )	(14,639 )	(47,412 )	(40,033 )
Change in valuation of contingent consideration	4,429	5,256	11,057	10,741
Subtotal non-GAAP items	16,005	17,100	45,686	44,729
<b>Non-GAAP operating income (loss)</b>	<b>\$ 5,397</b>	<b>\$ (3,272 )</b>	<b>\$ 7,072</b>	<b>\$ 4,737</b>
<b>GAAP net loss</b>	<b>\$ (17,794 )</b>	<b>\$ (37,113 )</b>	<b>\$ (42,113 )</b>	<b>\$ (39,300 )</b>
Non-GAAP operating loss adjustments	16,005	17,100	45,686	44,729
Change in fair value of derivative instruments	8,901	10,126	8,921	4,849
Income tax benefit (expense)	(1,223 )	6,467	(5,212 )	(5,994 )
<b>Non-GAAP net income (loss)</b>	<b>\$ 5,889</b>	<b>\$ (3,420 )</b>	<b>\$ 7,282</b>	<b>\$ 4,284</b>
<b>Per share data:</b>				
Net earnings per common share, basic	\$ 0.15	\$ (0.09 )	\$ 0.19	\$ 0.12

Weighted average common shares outstanding,  
basic

38,654,086

36,980,356

38,301,893

36,728,911

Contact:

Chris Cline, CFA

Vice President,

Investor Relations & Corporate Communications

646-564-3680

[IR@retrophin.com](mailto:IR@retrophin.com)

Source: Retrophin, Inc.