Retrophin Reports Fourth Quarter and Full Year 2017 Financial Results

February 27, 2018

Company gains alignment to proceed on Subpart H accelerated approval pathway for sparsentan in FSGS; Study start-up activities ongoing to initiate Phase 3 pivotal trial in second quarter of 2018

Clinical development of sparsentan in IgAN and CNSA-001 in PKU expected to initiate in 2018

Revenue increased 16 percent to $155 million during 2017

SAN DIEGO, Feb. 27, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its fourth quarter and full year 2017 financial results and provided a corporate update.

- The Company recently obtained regulatory feedback on its Phase 3 protocol for sparsentan in focal segmental glomerulosclerosis (FSGS); the U.S. Food and Drug Administration (FDA) has concurred that the Company's protocol and statistical modeling support proceeding with the planned Phase 3 pivotal trial that is designed for a Subpart H accelerated approval pathway
- In January 2018, the Company entered into a joint development arrangement to evaluate CNSA-001 for the treatment of phenylketonuria (PKU); results from a planned Phase 2 proof-of-concept study in PKU are expected to be available in early 2019
- Net product sales for the fourth quarter of 2017 were $42.2 million, compared to $37.3 million for the same period in 2016
- Net product sales for the full year 2017 were $154.9 million, compared to $133.6 million for the same period in 2016
- Cash, cash equivalents and marketable securities, as of December 31, 2017, totaled $300.6 million
- The Company expects full year 2018 net product sales to be in the range of $170.0 to $180.0 million

"2017 was an important year of execution for Retrophin, marked by meaningful progress across our clinical development efforts, as well as continued strong commercial and operational performance," said Stephen Aselage, chief executive officer of Retrophin. "We are also very pleased with our recent communications with the FDA regarding our sparsentan program for FSGS, for which we have reached alignment to pursue Subpart H accelerated approval with our planned Phase 3 trial. With four programs advancing in the clinic, 2018 is poised to be an exceptional year during which we expect to drive significant value for patients and shareholders alike."

Fourth Quarter and Full Year 2017 Financial Results

Net product sales for the fourth quarter of 2017 were $42.2 million, compared to $37.3 million for the same period in 2016. For the full year 2017, net product sales were $154.9 million, compared to $133.6 million for the same period in 2016. The increase in net product sales is attributable to growth across the Company's commercial products: Chenodal ®, Cholbam® and Thiola®. The Company expects full year 2018 net product sales to be in the range of $170.0 to $180.0 million.

Research and development (R&D) expenses for the fourth quarter of 2017 were $19.6 million, compared to $20.1 million for the same period in 2016. For the full year 2017, R&D expenses were $78.2 million, compared to $70.8 million for the same period in 2016. The difference is largely attributable to support of non-clinical and clinical efforts related to fosmetpantotenate and sparsentan. On a non-GAAP adjusted basis, R&D expenses were $17.7 million for the fourth quarter of 2017, compared to $17.6 million for the same period in 2016. For the full year 2017, non-GAAP adjusted R&D expenses were $68.9 million, compared to $60.0 million in 2016.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2017 were $26.7 million, compared to $26.2 million for the same period in 2016. For the full year 2017, SG&A expenses were $101.3 million, compared to $91.9 million for the same period in 2016. The difference is largely attributable to an increase in headcount as a result of the Company's commercial and operational growth, as well as marketing initiatives to support its commercial portfolio. On a non-GAAP adjusted basis, SG&A expenses were $18.5 million for the fourth quarter of 2017, compared to $17.5 million for the same period in 2016. For the full year 2017, non-GAAP adjusted SG&A expenses were $65.9 million, compared to $57.5 million in 2016.

Total other income for the fourth quarter of 2017 was $4.1 million, compared to $5.9 million for the same period in 2016. For the full year 2017, total other expense was $4.6 million, compared to other income of $0.6 million for the same period in 2016. The differences are largely attributable to adjustments in the fair value of derivative instruments due to changes in the Company's stock price.

Net loss for the fourth quarter of 2017 was $17.6 million, or $0.45 per basic share, compared to $8.6 million, or $0.23 per basic share for the same period in 2016. For the full year 2017, net loss was $59.7 million, or $1.54 per basic share, compared to $47.9 million, or $1.29 per basic share, for the same period in 2016. On a non-GAAP adjusted basis, net income for the fourth quarter of 2017 was $2.9 million, or $0.07 per basic share, compared to $0.1 million, or $0.00 per basic share for the same period in 2016. For the full year 2017, non-GAAP net income was $10.2 million, or $0.26 per basic share, compared to $4.4 million, or $0.12 per basic share, for the same period in 2016.

As of December 31, 2017, the Company had cash, cash equivalents and marketable securities of $300.6 million.

Program Updates

Fosmetpantotenate

- The Company continues to enroll patients in the Phase 3 FORT Study, an international, registrational clinical trial assessing the safety and efficacy of fosmetpantotenate in approximately 82 patients with pantothenate kinase-associated neurodegeneration (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 around year-end 2018.

- Four PKAN patients receiving fosmetpantotenate for more than three years under physician-initiated treatment outside of the U.S. continue to receive therapy and remain stable.

Sparsentan

- Following an End of Phase 2 meeting with the FDA, the Company announced plans to initiate a pivotal Phase 3 clinical trial of sparsentan in FSGS. The study is designed 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. The Company submitted its Phase 3 protocol to the Agency for review, and in late February 2018, the FDA communicated concurrence that the protocol design and statistical modeling completed by the Company were sufficient to support proceeding with the trial on the Subpart H accelerated approval pathway. Study start-up activities continue in anticipation of initiating the pivotal trial in the second quarter of 2018.

- The Company is also advancing the development of sparsentan in IgA nephropathy (IgAN), a rare, immune complex mediated glomerulonephritis characterized by proteinuria and variable rates of progressive renal failure. Regulatory interactions in the U.S. and Europe are underway in preparation for initiating a clinical trial in IgAN during the second half of 2018.

CNSA-001

- In January 2018, the Company announced a strategic collaboration with Censa Pharmaceuticals to advance CNSA-001 for the treatment of PKU. CNSA-001 is an orally bioavailable proprietary form of sepiapterin, a natural precursor of tetrahydrobiopterin (BH4) that is converted by an endogenous enzymatic pathway to BH4. CNSA-001 is currently being evaluated in a single ascending dose (SAD) study, and a Phase 2 proof-of-concept study in PKU is expected to commence in mid-2018, with results expected to be available in early 2019.

Thiola

- In 2018, the Company expects an NDA to be filed for its new formulation of Thiola for the treatment of cystinuria.

Conference Call Information

Retrophin will host a conference call and webcast today, Tuesday, February 27, 2018 at 4:30 p.m. ET to discuss development updates as well as fourth quarter and full year 2017 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 6589975 shortly before 4:30 p.m. ET. The webcast can be accessed at 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, February 27, 2018 to 7:30 p.m. ET, March 6, 2018. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 6589975.

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) 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®, Cholbam® and Thiola®.

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 fourth 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)

	December 31, 2017	December 31, 2016
Assets		
Current assets:		
Cash and cash equivalents	$ 99,394	$ 41,002
Marketable securities	201,236	214,871
Accounts receivable, net	13,872	18,510
Inventory, net	5,351	2,826
Prepaid expenses and other current assets	3,112	4,831
Prepaid taxes	2,842	3,463
Note receivable, current	—	46,849
Total current assets	325,807	332,352
Property and equipment, net	3,230	2,587
Other assets	5,556	7,364
Intangible assets, net	184,817	182,043
Goodwill	936	936
Total assets	$ 520,346	$ 525,282

Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 18,938	$ 7,522
Accrued expenses	36,018	33,308
Guaranteed minimum royalty, short term	2,000	2,000
Other current liabilities	3,902	1,842
Business combination-related contingent consideration	9,100	16,150
Derivative financial instruments, warrants	15,710	22,440
Total current liabilities	85,668	83,262
Convertible debt	45,077	44,422
Other noncurrent liabilities	2,472	4,010
Guaranteed minimum royalty, long term	13,095	8,068
Business combination-related contingent consideration, less current portion	80,900	71,328
Deferred income tax liability, net	—	6,425
Total liabilities	227,212	217,515
Stockholders' Equity:		
Preferred stock $0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2017 and 2016, respectively	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 39,373,745 and 37,906,669 issued and outstanding as of December 31, 2017 and 2016, respectively	4	4
Additional paid-in capital	471,800	421,309
Accumulated deficit	(177,655)	(113,056)
Accumulated other comprehensive loss	(1,015)	(490)
Total stockholders' equity	293,134	307,767
Total liabilities and stockholders' equity	$ 520,346	$ 525,282

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

	Three Months Ended December 31,		**Twelve Months Ended December 31,**	
	2017	**2016**	**2017**	**2016**
	(unaudited)			
Net product sales:				
Thiola	$ 22,213	$ 20,326	$ 82,311	$ 71,199
Bile acid products	19,964	17,001	72,626	62,392
Total net product sales	42,177	37,327	154,937	133,591
Operating expenses:				
Cost of goods sold	1,174	1,203	3,605	4,554
Research and development	19,576	20,064	78,168	70,822
Selling, general and administrative	26,650	26,227	101,333	91,941
Change in fair value of contingent consideration	8,332	7,643	19,389	18,383
Restructuring	997	412	3,608	893
Legal fee settlement	625	—	2,625	5,212
Total operating expenses	57,354	55,549	208,728	191,805
Operating loss	(15,177)	(18,222)	(53,791)	(58,214)
Other Income (expense), net:				
Other income (expense), net	42	(419)	1,107	(264)
Interest expense, net	(333)	(150)	(1,188)	(759)
Change in fair value of derivative instruments	4,430	6,504	(4,491)	1,655
Total other income (expense), net	4,139	5,935	(4,572)	632
Income (loss) before benefit (provision) for income taxes	(11,038)	(12,287)	(58,363)	(57,582)

Income tax benefit (provision)	(6,580)	3,684	(1,368)	9,679
Net income (loss)	$ (17,618)	$ (8,603)	$ (59,731)	$ (47,903)
Net earnings (loss) per common share, basic	$ (0.45)	$ (0.23)	$ (1.54)	$ (1.29)
Net earnings (loss) per common share, diluted	$ (0.55)	$ (0.39)	$ (1.54)	$ (1.29)
Weighted average common shares outstanding, basic	39,325,913	37,798,879	38,769,816	36,997,865
Weighted average common shares outstanding, diluted	40,089,779	38,940,193	38,769,816	38,288,012

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 December 31,		Twelve Months Ended December 31,	
	2017	2016	2017	2016
GAAP operating loss	**$ (15,177)**	**$ (18,222)**	**$ (53,791)**	**$ (58,214)**
R&D operating expense	(19,576)	(20,064)	(78,168)	(70,822)
Stock compensation	1,837	2,427	8,950	10,488
Amortization & depreciation	82	82	327	328
Subtotal non-GAAP items	1,919	2,509	9,277	10,816
Non-GAAP R&D expense	(17,657)	(17,555)	(68,891)	(60,006)
SG&A operating expense	(26,650)	(26,227)	(101,333)	(91,941)
Stock compensation	3,745	4,641	17,924	18,614
Amortization & depreciation	4,385	4,099	17,477	15,807
Subtotal non-GAAP items	8,130	8,740	35,401	34,421
Non-GAAP SG&A expense	(18,520)	(17,487)	(65,932)	(57,520)
Change in fair value of contingent consideration	8,332	7,643	19,389	18,383
Subtotal non-GAAP items	18,381	18,892	64,067	63,620
Non-GAAP operating income	**$ 3,204**	**$ 670**	**$ 10,276**	**$ 5,406**
GAAP net loss	**$ (17,618)**	**$ (8,603)**	**$ (59,731)**	**$ (47,903)**
Non-GAAP operating loss adjustments	18,381	18,892	64,067	63,620
Change in fair value of derivative instruments	(4,430)	(6,504)	4,491	(1,655)
Income tax benefit (provision)	6,580	(3,684)	1,368	(9,679)
Non-GAAP net income	**$ 2,913**	**$ 101**	**$ 10,195**	**$ 4,383**
Per share data:				
Net earnings per common share, basic	$ 0.07	$ —	$ 0.26	$ 0.12
Weighted average common shares outstanding, basic	39,325,913	37,798,879	38,769,816	36,997,865

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

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