



Retrophin Provides Sparsentan Regulatory Update; Reports Fourth Quarter and Full Year 2016 Financial Results

March 1, 2017

Phase 3 trial of sparsentan in FSGS to initiate in second half of 2017; Interim analysis of proteinuria to serve as basis for NDA filing for accelerated approval

Full year 2016 revenue grew 34% over 2015 to \$134 million

Conference call today at 5:00 p.m. ET

SAN DIEGO, March 01, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today provided an update on the regulatory pathway for its late-stage product candidate sparsentan, and announced fourth quarter and full year 2016 financial results. Following guidance received from the U.S. Food and Drug Administration (FDA) during the Company's End of Phase 2 meeting, the Company plans to initiate a single Phase 3 clinical trial to enable a New Drug Application (NDA) filing for sparsentan for the treatment of focal segmental glomerulosclerosis (FSGS). Notably, the trial will include an interim analysis of proteinuria as a surrogate endpoint to serve as the basis for an NDA filing for Subpart H accelerated approval of sparsentan. The confirmatory endpoint of the study will subsequently compare changes from baseline in estimated glomerular filtration rate, or eGFR. The Company is working with the FDA to finalize the study protocol and expects to initiate the trial in the second half of 2017.

"We are pleased to have received regulatory guidance from the FDA for sparsentan and look forward to working with them to finalize our Phase 3 study protocol and initiate this important trial later this year," said Stephen Aselage, chief executive officer of Retrophin. "We are particularly encouraged by the agency's alignment on the use of an interim analysis of proteinuria in this trial, which gives us an opportunity to expedite sparsentan's path to approval. We are eager to build on the strong data from the Phase 2 DUET study to further demonstrate the significant potential of sparsentan in the treatment of FSGS."

Fourth Quarter and Full Year 2016 Financial Results

- Net product sales for the fourth quarter of 2016 were \$37.3 million, compared to net product sales of \$30.4 million for the same period in 2015
- Net product sales for the full year 2016 were \$133.6 million, compared to net product sales of \$99.9 million for the same period in 2015
- Cash, cash equivalents, marketable securities, and note receivable as of December 31, 2016 totaled \$302.7 million
- Positive data from the Phase 2 DUET study of sparsentan presented at American Society of Nephrology (ASN) Kidney Week 2016
- Agreement reached under Special Protocol Assessment (SPA) for Phase 3 FORT study evaluating RE-024 in PKAN
- The Company expects full year 2017 net product sales to be in the range of \$150.0 to \$160.0 million

"From an operational perspective we finished 2016 with a strong fourth quarter, growing top-line revenue by more than 20 percent over the same period last year," said Neil McFarlane, chief operating officer of Retrophin. "Looking ahead, we are excited about our prospects for double-digit revenue growth in 2017 and supporting our strategic development efforts to help shape the promising future of the organization."

Net product sales for the fourth quarter of 2016 were \$37.3 million, compared to \$30.4 million for the same period in 2015. For the full year 2016, net product sales were \$133.6 million, compared to \$99.9 million for the same period in 2015. The increase in net product sales for the fourth quarter and full year 2016 is attributable to growth across the Company's commercial products: Thiola[®], Cholbam[®], and Chenodal[®].

Research and development (R&D) expenses for the fourth quarter of 2016 were \$20.1 million, compared to \$15.5 million for the same period in 2015. For the full year 2016, R&D expenses were \$70.9 million, compared to \$50.4 million for the same period in 2015. The increase is largely attributable to an increase in clinical efforts related to sparsentan and RE-024. On a non-GAAP adjusted basis, R&D expenses were \$17.6 million for the fourth quarter of 2016, compared to \$12.6 million for the same period in 2015. For the full year 2016, non-GAAP adjusted R&D expenses were \$60.0 million, compared to \$40.3 million in 2015.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2016 were \$26.6 million, compared to \$22.7 million for the same period in 2015. For the full year 2016, SG&A expenses were \$92.8 million, compared to \$79.5 million for the same period in 2015. The overall increase is largely due to expanded sales and marketing efforts to support the growth of the Company's commercial products. On a non-GAAP adjusted basis, SG&A expenses were \$17.9 million for the fourth quarter of 2016, compared to \$14.1 million for the same period in 2015. For the full year 2016, non-GAAP adjusted SG&A expenses were \$58.4 million, compared to \$50.4 million in 2015.

Total other income for the fourth quarter of 2016 was \$5.9 million, compared to \$2.2 million for the same period in 2015. For the full year 2016, total other income was \$0.6 million, compared to \$156.2 million for the same period in 2015. The increase in other income for the fourth quarter 2016 compared to the same period in 2015 is largely due to a change in the fair value of derivative instruments driven by changes in the Company's stock price. The \$155.6 million decrease in other income from the full year 2015 to 2016 is primarily attributable to one-time events in 2015 such as the gain on the sale of the Company's Priority Review Voucher, the bargain purchase gain as a result of the Cholbam acquisition and a litigation settlement gain, offset by the change in fair value of derivative instruments driven by changes in the Company's stock price.

Net loss for the fourth quarter of 2016 was \$8.6 million, or \$0.23 per basic share, compared to a net loss of \$2.5 million, or \$0.07 per basic share for the same period in 2015. For the full year 2016, net loss was \$47.9 million, or \$1.29 per basic share, compared to a net income of \$117.2 million, or \$3.49 per basic share for the same period in 2015. On a non-GAAP adjusted basis, net income for the fourth quarter of 2016 was \$0.1 million, or \$0.00 per basic share, compared to a net income of \$2.3 million, or \$0.06 per basic share for the same period in 2015. For the full year 2016, non-GAAP adjusted net income was \$4.4 million, or \$0.12 per basic share, compared to a net income of \$7.5 million, or \$0.22 per basic share in 2015.

As of December 31, 2016, the Company had cash, cash equivalents, marketable securities and note receivable of \$302.7 million.

Program Updates

Sparsentan

- The Company today announced plans to initiate a Phase 3 clinical trial of sparsentan in FSGS during the second half of 2017. The study will include an interim analysis of proteinuria to serve as the basis for an NDA filing for accelerated approval of sparsentan. The confirmatory endpoint of the study will compare changes from baseline in eGFR, which is widely regarded as the best overall measure of kidney function.
- In the fourth quarter of 2016, the Company presented positive data from the Phase 2 DUET study of sparsentan at ASN Kidney Week 2016. These data included an analysis of the secondary endpoint showing that a significantly greater proportion of patients receiving sparsentan achieved modified partial remission (mPR) of proteinuria, compared to irbesartan-treated patients. mPR of proteinuria is defined as proteinuria levels of less than or equal to 1.5 g/g and greater than 40 percent reduction of proteinuria from baseline, and is associated with long-term preservation of renal function in FSGS. In addition, four patients receiving sparsentan achieved complete remission, compared to zero irbesartan-treated patients. The data presented also showed sparsentan was generally safe and well-tolerated in the study.

RE-024

- During the fourth quarter of 2016, the Company reached a SPA agreement with the FDA on the design of the Phase 3 FORT study of RE-024. The agreement indicates concurrence by the FDA that the trial design can adequately support an NDA filing seeking U.S. approval of RE-024 for the treatment of PKAN.
- The Company expects to begin dosing the first PKAN patients in the Phase 3 FORT study of RE-024 in mid-2017.
- The four PKAN patients receiving RE-024 under physician-initiated treatment outside of the U.S. continue on therapy and remain stable.

Liquid ursodeoxycholic acid (L-UDCA)

- Development efforts continue with the intention of making L-UDCA commercially available to the subset of primary biliary cholangitis patients who have difficulty swallowing.

Chenodal[®] (chenodeoxycholic acid)

- The prevalence study of cerebrotendinous xanthomatosis (CTX) in patient populations diagnosed with early-onset idiopathic bilateral cataracts continues to enroll subjects and serve as a valuable approach to raise awareness of the disorder.

2017 Outlook

The Company expects full year 2017 net product sales to be in the range of \$150.0 to \$160.0 million. The anticipated double-digit percent increase over 2016 is expected to be primarily driven by growth across all three products.

Conference Call Information

Retrophin will host a conference call and webcast today, Wednesday, March 1, 2017 at 5:00 p.m. ET to discuss development updates as well as fourth quarter and full year 2016 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 70808962 shortly before 5:00 p.m. ET. The webcast can be accessed at www.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 starting at 8:00 p.m. ET, March 1, 2017 until 8:00 p.m. ET, March 8, 2017. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 70808962.

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 provision; bargain purchase gain (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 fully integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease, and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood. Research exploring the potential of early-stage assets in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola[®], Cholbam[®], and Chenodal[®].

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 RE-024 will not demonstrate that RE-024 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 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)

	<u>December 31,</u> <u>2016</u>	<u>December 31,</u> <u>2015</u>
Assets		
Current assets:		
Cash and cash equivalents	\$ 41,002	\$ 37,805
Marketable securities	214,871	191,799
Accounts receivable, net	18,510	12,458
Inventory, net	2,826	2,536
Prepaid expenses and other current assets	4,831	2,378

Prepaid taxes	3,463	8,107
Note receivable, current	46,849	46,849
Total current assets	332,352	301,932
Property and equipment, net	2,587	428
Other assets	7,364	1,859
Intangible assets, net	182,043	161,536
Goodwill	936	936
Note receivable, long-term	—	45,573
Total assets	\$ 525,282	\$ 512,264

Liabilities and Stockholders' Equity

Current liabilities:

Accounts payable	\$ 7,522	\$ 7,639
Accrued expenses	33,308	23,820
Guaranteed minimum royalty, short term	2,000	2,000
Other current liabilities	1,842	958
Business combination-related contingent consideration	16,150	13,754
Derivative financial instruments, warrants	22,440	38,810
Total current liabilities	83,262	86,981
Convertible debt	44,422	43,766
Other noncurrent liabilities	4,010	3,066
Guaranteed minimum royalty, long term	8,068	8,885
Business combination-related contingent consideration, less current portion	71,328	45,267
Deferred income tax liability, net	6,425	24,328
Total liabilities	217,515	212,293

Stockholders' Equity:

Preferred stock Series A \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2016 and 2015, respectively	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 37,906,669 and 36,465,853 issued and outstanding as of December 31, 2016 and 2015, respectively	4	4
Additional paid-in capital	421,309	365,802
Accumulated deficit	(113,056)	(65,153)
Accumulated other comprehensive loss	(490)	(682)
Total stockholders' equity	307,767	299,971
Total liabilities and stockholders' equity	\$ 525,282	\$ 512,264

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)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2016	2015	2016	2015
	(unaudited)			
Net product sales	\$ 37,327	\$ 30,447	\$ 133,591	\$ 99,892

Operating expenses:				
Cost of goods sold	1,203	761	4,554	2,185
Research and development	20,078	15,452	70,853	50,426
Selling, general and administrative	26,625	22,686	92,803	79,541
Legal fee settlement	—	—	5,212	—
Change in fair value of contingent consideration	7,643	6,752	18,383	13,778
Impairment of intangible assets	—	—	—	4,710
Total operating expenses	<u>55,549</u>	<u>45,651</u>	<u>191,805</u>	<u>150,640</u>
Operating loss	<u>(18,222)</u>	<u>(15,204)</u>	<u>(58,214)</u>	<u>(50,748)</u>
Other Income (expense), net:				
Litigation settlement gain	—	—	—	15,500
Other income (expense), net	(419)	(330)	(264)	(296)
Interest expense, net	(150)	(333)	(759)	(7,748)
Debt early payment penalty	—	—	—	(2,250)
Loss on extinguishment of debt	—	—	—	(4,151)
Finance expense	—	—	—	(600)
Change in fair value of derivative instruments	6,504	2,873	1,655	(33,307)
Gain on sale of assets	—	—	—	140,004
Bargain purchase gain	—	—	—	49,063
Total other income (expense), net	<u>5,935</u>	<u>2,210</u>	<u>632</u>	<u>156,215</u>
Income (loss) before benefit for income taxes	(12,287)	(12,994)	(57,582)	105,467
Income tax benefit	<u>3,684</u>	<u>10,525</u>	<u>9,679</u>	<u>11,770</u>
Net income (loss)	<u>\$ (8,603)</u>	<u>\$ (2,469)</u>	<u>\$ (47,903)</u>	<u>\$ 117,237</u>
Net earnings (loss) per common share, basic	<u>\$ (0.23)</u>	<u>\$ (0.07)</u>	<u>\$ (1.29)</u>	<u>\$ 3.49</u>
Net earnings (loss) per common share, diluted	<u>\$ (0.39)</u>	<u>\$ (0.14)</u>	<u>\$ (1.29)</u>	<u>\$ 3.17</u>
Weighted average common shares outstanding, basic	<u>37,798,879</u>	<u>36,260,106</u>	<u>36,997,865</u>	<u>33,560,249</u>
Weighted average common shares outstanding, diluted	<u>38,940,193</u>	<u>37,985,347</u>	<u>38,288,012</u>	<u>37,581,439</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 December 31 ,		Twelve Months Ended December 31 ,	
	2016	2015	2016	2015
GAAP operating loss	\$ (18,222)	\$ (15,204)	\$ (58,214)	\$ (50,748)
R&D operating expense	(20,078)	(15,452)	(70,853)	(50,426)
Stock compensation	2,427	2,756	10,488	9,417
Amortization & depreciation	<u>82</u>	<u>83</u>	<u>328</u>	<u>697</u>

Subtotal non-GAAP items	2,509	2,839	10,816	10,114
Non-GAAP R&D expense	(17,569)	(12,613)	(60,037)	(40,312)
SG&A operating expense	(26,625)	(22,686)	(92,803)	(79,541)
Stock compensation	4,641	4,395	18,614	16,483
Amortization & depreciation	4,099	4,148	15,807	12,693
Subtotal non-GAAP items	8,740	8,543	34,421	29,176
Non-GAAP SG&A expense	(17,885)	(14,143)	(58,382)	(50,365)
Change in fair value of contingent consideration	7,643	6,752	18,383	13,778
Impairment of intangible assets	—	—	—	4,710
Subtotal non-GAAP items	18,892	18,134	63,620	57,778
Non-GAAP operating income	\$ 670	\$ 2,930	\$ 5,406	\$ 7,030
GAAP net income (loss)	\$ (8,603)	\$ (2,469)	\$ (47,903)	\$ 117,237
Non-GAAP operating loss adjustments	18,892	18,134	63,620	57,778
Change in fair value of derivative instruments	(6,504)	(2,873)	(1,655)	33,307
Bargain purchase gain	—	—	—	(49,063)
Gain on sale of assets	—	—	—	(140,004)
Income tax benefit	(3,684)	(10,525)	(9,679)	(11,770)
Non-GAAP net income	\$ 101	\$ 2,267	\$ 4,383	\$ 7,485
Per share data:				
Net gain (loss) per common share, basic	0.00	\$ 0.06	\$ 0.12	\$ 0.22
Weighted average common shares outstanding, basic	37,798,879	36,260,106	36,997,865	33,560,249

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

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