



## Retrophin Reports First Quarter 2016 Financial Results

May 3, 2016

*First quarter revenues increased 67 percent year-over-year*

*Enrollment of the Phase 2 DUET study of sparsentan completed; top-line results expected third quarter 2016*

SAN DIEGO, May 03, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its first quarter 2016 financial results.

- Net product sales for the first quarter 2016 were \$29.0 million, compared to net product sales of \$17.4 million for the same period in 2015
- Full-year 2016 revenue guidance of \$130.0 to \$140.0 million reiterated
- Cash, cash equivalents, marketable securities, and notes receivable as of March 31, 2016 totaled \$315.4 million
- Phase 2 DUET study of sparsentan completes enrollment, top-line data expected in third quarter of 2016

"In the first quarter, we made tangible progress on the strategic initiatives enabling 2016 to be a transformational year for Retrophin," said Stephen Aselage, chief executive officer of Retrophin. "Completing enrollment of the DUET study of sparsentan was a great accomplishment, and we expect top-line results in the third quarter. We also plan to initiate a trial evaluating the efficacy of RE-024 in PKAN patients during the second half of 2016. Additionally, our recently launched commercial efforts are gaining traction across all products and should yield increased growth through the remainder of the year."

### **Quarter Ended March 31, 2016**

Net product sales for the first quarter of 2016 were \$29.0 million, compared to \$17.4 million for the same period in 2015. The increase was primarily due to new patients initiating treatment with Thiola<sup>®</sup> and Chenodal<sup>®</sup>, and contributions from Cholbam<sup>®</sup>, which was acquired in March 2015. The Company expects sales growth to accelerate through the balance of 2016 due to increasing net patient additions to all three products, and reiterates its full-year guidance of \$130.0 to \$140.0 million.

Selling, general and administrative expenses for the first quarter of 2016 were \$19.1 million, compared to \$14.9 million for the same period in 2015. The increase is attributable to additional headcount supporting expanded commercial efforts and amortization related to the addition of Cholbam.

SG&A experienced a one-time benefit of \$3.0 million in the first quarter of 2016 due to the reversal of disputed legal fees as a result of a settlement with the Company's former external legal counsel. On a non-GAAP adjusted basis, selling, general and administrative expenses were \$11.0 million for the first quarter of 2016, compared to \$9.9 million for the same period in 2015.

Research and development expenses for the first quarter of 2016 were \$14.7 million, compared to \$10.3 million for the same period in 2015. The increase is largely due to clinical trial expense supporting the Phase 2 DUET study of sparsentan in patients with focal segmental glomerulosclerosis (FSGS), and the preparation of an efficacy trial of RE-024 in patients with pantothenate kinase-associated neurodegeneration (PKAN). On a non-GAAP adjusted basis, research and development expenses were \$12.1 million for the first quarter of 2016, compared to \$7.9 million for the same period in 2015.

Total other income for the first quarter of 2016 was \$14.4 million, compared to \$7.7 million for the same period in 2015. The difference is largely attributable to a gain resulting from a decrease in the Company's derivative liability due to share price fluctuation and a reduction in interest expense after the prepayment of the Company's credit facility in July 2015. This was partially offset by a bargain purchase gain in the first quarter of 2015 related to the acquisition of Cholbam.

Tax benefit of \$5.1 million for the first quarter of 2016 was primarily due to a favorable effective tax rate as a result of orphan drug and research and development tax credits.

Net Income for the first quarter of 2016 was \$11.2 million, or \$0.31 per basic share, compared to \$39.7 million, or \$1.46 per basic share for the same period in 2015. Non-GAAP adjusted net income for the first quarter of 2016 was \$5.2 million, or \$0.14 per basic share, compared to a net loss of \$4.8 million, or \$0.18 per basic share for the same period in 2015.

As of March 31, 2016, the Company had cash, cash equivalents, marketable securities and notes receivable of \$315.4 million.

### **Commercial Product Updates**

Thiola<sup>®</sup> (tiopronin)

- New patients continued to initiate treatment with Thiola on a weekly basis during the first quarter of 2016.
- In April 2016, the Company was advised that Thiola had been removed from the U.S. Food and Drug Administration's (FDA) drug shortage list. The previous inclusion of Thiola on the list dated from the period before Retrophin acquired distribution rights to the product. Since then, the Company has more than doubled access to the medication and has significantly invested in building supply to meet projected demand.

#### Cholbam® (cholic acid)

- New patients initiating treatment with Cholbam accelerated in the first quarter of 2016.
- During the first quarter of 2016, Retrophin began providing access to the Neonatal and Adult Cholestasis Sequencing Panel through a leading genetics laboratory. This screening panel covering 57 genetic mutations is available free of charge to patients and physicians in the U.S. Use of this panel is gaining traction and may enhance the identification of new patients who could benefit from Cholbam therapy.

#### Chenodal® (chenodeoxycholic acid)

- The rate of cerebrotendinous xanthomatosis (CTX) patients initiating Chenodal treatment increased during the first quarter of 2016.
- Following a constructive meeting with the FDA in April 2016, the Company is assessing clinical efforts that could enable the addition of CTX to the Chenodal label.
- Approximately 20 sites have been activated in the CTX prevalence study. The Company anticipates activating approximately 40 sites and enrolling up to 500 subjects in this multi-year study.

#### **Pipeline Updates**

##### *Sparsentan*

- In the first quarter of 2016, Retrophin completed enrollment of the Phase 2 DUET study of sparsentan for the treatment of FSGS. Top-line results are expected to be available in the third quarter of 2016.
- The Company is collaborating with academic research networks and patient advocates to further develop a data package supporting the use of proteinuria as a surrogate endpoint for clinical outcomes in FSGS. These data may ultimately support an application for accelerated approval of sparsentan if the DUET data demonstrate a robust reduction of proteinuria relative to the active control.

##### *RE-024*

- Preparations for an efficacy trial of RE-024 in patients with PKAN are ongoing. The study is expected to initiate in the second half of 2016. Constructive interactions continue with the FDA and European Medicines Agency on protocol design.
- In the first quarter of 2016, Retrophin and collaborators presented new data at the American College of Medical Genetics and Genomics (ACMG) Annual Clinical Genetics Meeting. These data included preclinical research describing initial insights into the pharmacokinetic profile and mechanism of action of RE-024, results from a Phase 1 study of healthy volunteers, and a patient case report, all of which support further clinical development of RE-024.
- An abstract summarizing new data from two of the four PKAN patients receiving RE-024 as part of physician-initiated treatment has been accepted for presentation at the 20th International Congress of Parkinson's Disease and Movement Disorders. These case reports will be shared in a poster session on June 23, 2016 in Berlin.
- All four PKAN patients receiving RE-024 under physician-initiated treatment outside of the U.S. continue on therapy and remain stable.

##### *RE-034*

- The Company is pursuing *in vivo* proof of concept data in non-clinical models for RE-034 that could support continued development in a specific orphan indication.

#### **Share Repurchase Program**

The Company's Board of Directors has approved the repurchase of up to an aggregate \$40.0 million of its common stock. Repurchases may be made from time-to-time via purchases on the open market, privately negotiated transactions, accelerated stock repurchases, pre-planned trading programs or other means subject to market conditions as management may deem appropriate. The timing of any repurchases and the actual number of shares repurchased will depend on a variety of factors, including stock price, corporate and regulatory requirements and other market and economic conditions. The Company may suspend or discontinue this repurchase program at any time and makes no representations, warranties or guarantees that such a buy-back will ultimately occur.

#### **Conference Call Information**

Retrophin will host a conference call and webcast today, Tuesday, May 3, 2016 at 4:30 p.m. ET to discuss first quarter 2016 financial results. To

participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 92067921 shortly before 4:30 p.m. ET. The webcast can be accessed at [www.retrophin.com](http://www.retrophin.com), in the Events and Presentations section. A replay of the call will be available 7:30 p.m. ET, May 3, 2016 to 7:30 p.m. ET, May 10, 2016. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 92067921.

### **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 clinical-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, including RE-034, in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's marketed products Chenodal<sup>®</sup>, Cholbam<sup>®</sup> and Thiola<sup>®</sup>.

[Retrophin.com](http://www.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, regarding the research, development and commercialization of pharmaceutical products. 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 research programs will not identify preclinical candidates for further development and risk that the Company's clinical candidates will not be found to be safe or effective. Specifically, the Company faces risk that the sparsentan Phase 2 clinical trials will fail to demonstrate that sparsentan is safe or effective; risk that the sparsentan Phase 2 program will be delayed for regulatory or other reasons, risk that RE-024 will not progress to Phase 2 or later clinical trials for safety, regulatory or other reasons; risk that the Company will be unable to file an IND for RE-034 or initiate Phase 1 clinical trials for regulatory or other reasons, and for each of the programs risk associated with enrollment of clinical trials for rare diseases. The Company faces risk that it will be unable to raise additional funding 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; 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 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 filings with the Securities and Exchange Commission.

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONSOLIDATED BALANCE SHEETS**  
**March 31, 2016**  
**(in thousands)**

	<b>March 31, 2016</b>	<b>December 31, 2015</b>
	(unaudited)	
<b>Assets</b>		
<b>Current assets:</b>		
Cash	\$ 23,262	\$ 37,805
Marketable securities	199,083	191,799
Accounts receivable, net	13,400	12,458
Inventory, net	3,230	2,536
Prepaid expenses and other current assets	3,093	2,378
Prepaid taxes	8,498	8,107
Note receivable, current	47,173	46,849
<b>Total current assets</b>	<u>297,739</u>	<u>301,932</u>
Property and equipment, net	417	428
Other asset	1,859	1,859
Intangible assets, net	160,260	161,536
Goodwill	936	936
Note receivable, long term	45,889	45,573
<b>Total assets</b>	<u>\$ 507,100</u>	<u>\$ 512,264</u>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 4,647	\$ 7,639
Accrued expenses	20,392	23,820
Other current liabilities	1,235	958
Guaranteed minimum royalty, short term	2,000	2,000
Business combination-related contingent consideration	13,873	13,754
Derivative financial instruments, warrants	24,470	38,810
<b>Total current liabilities</b>	<u>66,617</u>	<u>86,981</u>
Convertible debt	43,929	43,766
Other non-current liabilities	2,889	3,066
Guaranteed minimum royalty, long term	8,689	8,885
Business combination-related contingent consideration, less current portion	46,426	45,267
Deferred income tax liability, net	19,318	24,328
<b>Total liabilities</b>	<u>187,868</u>	<u>212,293</u>
<b>Stockholders' Equity:</b>		
Preferred stock Series A \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding	-	-
Common stock \$0.0001 par value; 100,000,000 shares authorized; 36,592,435 and 36,465,853 issued and 36,592,435 and 36,465,853 outstanding, respectively	4	4
Additional paid-in capital	373,327	365,802
Accumulated deficit	(53,937)	(65,153)
Accumulated other comprehensive loss	(162)	(682)
<b>Total stockholders' equity</b>	<u>319,232</u>	<u>299,971</u>
<b>Total liabilities and stockholders' equity</b>	<u>\$ 507,100</u>	<u>\$ 512,264</u>

**RETROPHIN, INC. AND SUBSIDIARIES**  
**CONSOLIDATED STATEMENTS OF OPERATIONS**  
**March 31, 2016**  
*(in thousands, except share and per share data)*  
**(unaudited)**

	<b>Three months ended March 31,</b>	
	<b>2016</b>	<b>2015</b>
Net product sales	\$ 29,008	\$ 17,372
Operating expenses:		
Cost of goods sold	757	274
Research and development	14,672	10,347
Selling, general and administrative	19,125	14,855
Change in valuation of contingent consideration	2,695	-
<b>Total operating expenses</b>	<b>37,249</b>	<b>25,476</b>
<b>Operating loss</b>	<b>(8,241)</b>	<b>(8,104)</b>
<b>Other income (expenses):</b>		
Other income, net	210	311
Interest expense, net	(163)	(3,798)
Finance expense	-	(600)
Change in fair value of derivative instruments	14,340	(36,753)
Bargain purchase gain	-	48,578
Total other income, net	14,387	7,738
<b>Income (loss) before provision for income taxes</b>	<b>6,146</b>	<b>(366)</b>
Income tax benefit	5,070	40,021
<b>Net income</b>	<b>\$ 11,216</b>	<b>\$ 39,655</b>
<b>Per share data:</b>		
Net earnings per common share, basic	\$ 0.31	\$ 1.46
Net earnings (loss) per common share, diluted	\$ (0.08)	\$ 1.32
Weighted average common shares outstanding, basic	36,520,186	27,157,883
Weighted average common shares outstanding, diluted	37,947,479	30,380,694

**RETROPHIN, INC. AND SUBSIDIARIES**  
**RECONCILIATION OF GAAP REPORTED TO NON-GAAP ADJUSTED INFORMATION**  
**March 31, 2016**  
*(in thousands, except share and per share data)*  
**(unaudited)**

	Three months ended March 31,	
	2016	2015
<b>GAAP Operating Loss</b>	<b>\$ (8,241)</b>	<b>\$ (8,104)</b>
R&D Operating Expense	(14,672)	(10,347)
Stock Compensation	2,486	2,220
Amortization & Depreciation	82	221
Subtotal non-GAAP items	<u>2,568</u>	<u>2,441</u>
Non-GAAP R&D Expense	<u>(12,104)</u>	<u>(7,906)</u>
SG&A Operating Expense	(19,125)	(14,855)
Stock Compensation	4,307	3,354
Amortization & Depreciation	3,810	1,558
Subtotal non-GAAP items	<u>8,117</u>	<u>4,912</u>
Non-GAAP SG&A Expense	<u>(11,008)</u>	<u>(9,943)</u>
Change in valuation of contingent consideration	2,695	-
Subtotal non-GAAP items	<u>13,380</u>	<u>7,353</u>
<b>Non-GAAP Operating Income (Loss)</b>	<b><u>5,139</u></b>	<b><u>(751)</u></b>
<b>GAAP Net Income</b>	<b>11,216</b>	<b>39,655</b>
Non-GAAP Operating Expense Adjustments	13,380	7,353
Change in fair value of derivative instruments	(14,340)	36,753
Bargain purchase gain	-	(48,578)
Income Tax benefit	(5,070)	(40,021)
<b>Non-GAAP Net Income (Loss)</b>	<b><u>\$ 5,186</u></b>	<b><u>\$ (4,838)</u></b>
Per Share Data:		
Net earnings (loss) per common share, basic	<u>\$ 0.14</u>	<u>\$ (0.18)</u>
Weighted average common shares outstanding, basic	<u>36,520,186</u>	<u>27,157,883</u>

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

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