



## Retrophin Reports Second Quarter 2016 Financial Results

August 4, 2016

*Second quarter revenues increased 38 percent year-over-year*

*Sparsentan top-line data expected in third quarter of 2016*

*RE-024 efficacy trial expected to initiate in second half of 2016*

SAN DIEGO, Aug. 04, 2016 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its second quarter 2016 financial results.

- Net product sales for the second quarter of 2016 were \$33.3 million, compared to net product sales of \$24.1 million for the same period in 2015
- Cash, cash equivalents, marketable securities, and notes receivable as of June 30, 2016 totaled \$315.2 million
- Top-line data from the Phase 2 DUET study of sparsentan in focal segmental glomerulosclerosis (FSGS) remains on track to read out in the third quarter of 2016
- Trial evaluating the efficacy of RE-024 in pantothenate kinase-associated neurodegeneration (PKAN) remains on track to initiate in the second half of 2016
- Liquid formulation of ursodeoxycholic acid (L-UDCA) acquired in the second quarter of 2016; NDA filing for primary biliary cholangitis (PBC) indication expected in 2017

"We are very pleased with our strong second quarter performance, which was led by growth across all of our commercial products, and included the expansion of our pipeline," said Stephen Aselage, chief executive officer of Retrophin. "We look forward to building upon the momentum from the first half of the year with upcoming clinical milestones for sparsentan and RE-024, which we believe will make 2016 a transformational year for Retrophin."

### **Quarter Ended June 30, 2016**

Net product sales for the second quarter of 2016 were \$33.3 million, compared to \$24.1 million for the same period in 2015. For the six months ended June 30, 2016, net product sales were \$62.3 million, compared to \$41.4 million for the same period in 2015. The increase was due to new patients initiating treatment with all of the Company's commercial products: Thiola<sup>®</sup>, Cholbam<sup>®</sup>, and Chenodal<sup>®</sup>. The Company expects further sales growth throughout the balance of 2016 and reiterates its full-year guidance of \$130.0 to \$140.0 million.

Selling, general and administrative (SG&A) expenses for the second quarter of 2016 were \$23.2 million, compared to \$19.7 million for the same period in 2015. For the six months ended June 30, 2016, SG&A expenses were \$42.3 million, compared to \$34.5 million for the same period in 2015. The increase is attributable to additional headcount and expanded efforts in support of the Company's commercial products. On a non-GAAP adjusted basis, SG&A expenses were \$14.5 million for the second quarter of 2016, compared to \$12.8 million for the same period in 2015.

Research and development (R&D) expenses for the second quarter of 2016 were \$17.7 million, compared to \$10.6 million for the same period in 2015. For the six months ended June 30, 2016, R&D expenses were \$32.3 million, compared to \$20.9 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 \$15.0 million for the second quarter of 2016, compared to \$8.6 million for the same period in 2015.

Total other expense for the second quarter of 2016 was \$9.4 million, compared to \$18.6 million for the same period in 2015. For the six months ended June 30, 2016, total other income was \$5.0 million, compared to total other expense of \$10.8 million for the same period in 2015. The difference is largely attributable to a decrease in the Company's derivative liability due to less share price fluctuation in the period, and the prepayment of the Company's credit facility in July 2015, which resulted in a reduction in interest expense and loss on extinguishment of debt.

Tax benefit of \$7.4 million for the second quarter of 2016 was primarily due to a favorable effective tax rate as a result of orphan drug and R&D tax credits.

Net loss for the second quarter of 2016 was \$13.4 million, or \$0.37 per basic share, compared to \$25.5 million, or \$0.73 per basic share for the same period in 2015. For the six months ended June 30, 2016, net loss was \$2.2 million, compared to a net income of \$14.1 million for the same period in 2015. Non-GAAP adjusted net income for the second quarter of 2016 was \$2.5 million, or \$0.07 per basic share, compared to \$12.9 million, or \$0.37 per basic share for the same period in 2015.

As of June 30, 2016, the Company had cash, cash equivalents, marketable securities and notes receivable of \$315.2 million.

### **Commercial Product Updates**

Thiola<sup>®</sup> (tiopronin)

- New patients continued to initiate treatment with Thiola during the second quarter of 2016.

Cholbam<sup>®</sup> (cholic acid)

- New patient growth with Cholbam continued during the second quarter of 2016.
- Uptake of the Retrophin-sponsored Neonatal and Adult Cholestasis Sequencing Panel remained strong during the second quarter of 2016. The free genetic panel is expected to drive further awareness of bile acid synthesis disorders and Zellweger spectrum disorders and may enhance identification of new patients who could benefit from Cholbam therapy.

Chenodal® (chenodeoxycholic acid)

- New cerebrotendinous xanthomatosis (CTX) patients continued to initiate Chenodal treatment during the second quarter of 2016.
- Approximately 25 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*

- Top-line data from the Phase 2 DUET study of sparsentan in FSGS are expected to read out in the third quarter of 2016, as previously reported.

*RE-024*

- The Company remains on track to initiate its efficacy trial evaluating RE-024 in PKAN during the second half of 2016.
- In the second quarter of 2016, Retrophin and collaborators presented new data at the 20th International Congress of Parkinson's Disease and Movement Disorders suggesting RE-024 was safe and well tolerated in two adults with PKAN. Both patients experienced clinically meaningful improvements, followed by stabilization of disease symptoms over 47 weeks of treatment.
- 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)*

- During the second quarter of 2016, the Company acquired the rights to L-UDCA. Retrophin intends to file a New Drug Application with the U.S. Food and Drug Administration for the treatment of PBC in 2017, with the goal of making L-UDCA commercially available to the subset of PBC patients who have difficulty swallowing.

### **Conference Call Information**

Retrophin will host a conference call and webcast today, Thursday, August 4, 2016 at 4:30 p.m. ET to discuss second 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 50760103 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 starting at 7:30 p.m. ET, August 4, 2016 until 11:59 p.m. ET, August 11, 2016. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 50760103.

### **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 in several rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola<sup>®</sup>, Cholbam<sup>®</sup>, and Chenodal<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, 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 a New Drug Application for liquid ursodeoxycholic acid in 2017 or ever, risk that a New Drug Application for liquid ursodeoxycholic acid will not be approved for efficacy, safety, regulatory or other reasons, the 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

June 30, 2016

(in thousands)

	June 30, 2016	December 31, 2015
	(unaudited)	
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 21,155	\$ 37,805
Marketable securities	200,359	191,799
Accounts receivable, net	16,566	12,458
Inventory, net	2,713	2,536
Prepaid expenses and other current assets	3,365	2,378
Prepaid taxes	8,499	8,107
Note receivable, current	47,500	46,849
<b>Total current assets</b>	<b>300,157</b>	<b>301,932</b>
Property and equipment, net	381	428
Other asset	1,859	1,859
Intangible assets, net	184,432	161,536
Goodwill	936	936
Note receivable, long term	46,206	45,573

<b>Total assets</b>	<u>\$ 533,971</u>	<u>\$ 512,264</u>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 6,285	\$ 7,639
Accrued expenses	23,159	23,820
Other current liabilities	1,187	958
Guaranteed minimum royalty	2,000	2,000
Business combination-related contingent consideration	14,077	13,754
Derivative financial instruments, warrants	33,360	38,810
<b>Total current liabilities</b>	<u>80,068</u>	<u>86,981</u>
Convertible debt	44,092	43,766
Other non-current liabilities	2,548	3,066
Guaranteed minimum royalty, less current portion	8,488	8,885
Business combination-related contingent consideration, less current portion	72,300	45,267
Deferred income tax liability, net	12,103	24,328
<b>Total liabilities</b>	<u>219,599</u>	<u>212,293</u>
<b>Stockholders' Equity:</b>		
Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2016 and December 31, 2015	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 36,725,130 and 36,465,853 issued and outstanding as of June 30, 2016 and December 31, 2015, respectively	4	4
Additional paid-in capital	381,687	365,802
Accumulated deficit	(67,340)	(65,153)
Accumulated other comprehensive income (loss)	21	(682)
<b>Total stockholders' equity</b>	<u>314,372</u>	<u>299,971</u>
<b>Total liabilities and stockholders' equity</b>	<u>\$ 533,971</u>	<u>\$ 512,264</u>

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

	<u>Three Months Ended June 30,</u>		<u>Six Months Ended June 30,</u>	
	2016	2015	2016	2015
Net product sales	\$ 33,311	\$ 24,068	\$ 62,319	\$ 41,440
Operating expenses:				
Cost of goods sold	1,021	637	1,778	912
Research and development	17,675	10,563	32,347	20,910
Selling, general and administrative	23,205	19,692	42,330	34,547
Change in valuation of contingent consideration	2,789	120	5,485	120
<b>Total operating expenses</b>	<u>44,690</u>	<u>31,012</u>	<u>81,940</u>	<u>56,489</u>
<b>Operating loss</b>	<u>(11,379)</u>	<u>(6,944)</u>	<u>(19,621)</u>	<u>(15,049)</u>
Other income (expenses), net:				
Other income (expenses), net	(206)	522	4	349
Interest expense, net	(147)	(2,922)	(309)	(6,720)

Finance expense	—	—	—	(600)
Change in fair value of derivative instruments	(9,063)	(29,418)	5,277	(66,171)
Loss on extinguishment of debt	—	(2,250)	—	(2,250)
Litigation settlement gain	—	15,500	—	15,500
Bargain purchase gain	—	—	—	49,063
Total other income (loss), net	<u>(9,416)</u>	<u>(18,568)</u>	<u>4,972</u>	<u>(10,829)</u>
Loss before provision for income taxes	(20,795)	(25,512)	(14,649)	(25,878)
Income tax benefit (expense)	<u>7,392</u>	<u>(15)</u>	<u>12,462</u>	<u>40,006</u>
Net income (loss)	<u>\$ (13,403)</u>	<u>\$ (25,527)</u>	<u>\$ (2,187)</u>	<u>\$ 14,128</u>
Net earnings (loss) per common share, basic	<u>\$ (0.37)</u>	<u>\$ (0.73)</u>	<u>\$ (0.06)</u>	<u>\$ 0.45</u>
Net earnings (loss) per common share, diluted	<u>\$ (0.37)</u>	<u>\$ (0.73)</u>	<u>\$ (0.20)</u>	<u>\$ 0.44</u>
Weighted average common shares outstanding, basic	<u>36,683,429</u>	<u>34,957,134</u>	<u>36,601,807</u>	<u>31,079,053</u>
Weighted average common shares outstanding, diluted	<u>36,683,429</u>	<u>34,957,134</u>	<u>38,063,285</u>	<u>34,827,405</u>

**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 June 30,		Six Months Ended June 30,	
	2016	2015	2016	2015
<b>GAAP Operating Loss</b>	<b>\$ (11,379)</b>	<b>\$ (6,944)</b>	<b>\$ (19,621)</b>	<b>\$ (15,049)</b>
R&D Operating Expense	(17,675)	(10,563)	(32,347)	(20,910)
Stock Compensation	2,641	1,749	5,127	3,968
Amortization & Depreciation	81	193	163	414
Subtotal non-GAAP items	<u>2,722</u>	<u>1,942</u>	<u>5,290</u>	<u>4,382</u>
Non-GAAP R&D Expense	<u>(14,953)</u>	<u>(8,621)</u>	<u>(27,057)</u>	<u>(16,528)</u>
SG&A Operating Expense	(23,205)	(19,692)	(42,330)	(34,547)
Stock Compensation	4,852	3,245	9,159	6,599
Amortization & Depreciation	3,885	3,648	7,695	5,206
Subtotal non-GAAP items	<u>8,737</u>	<u>6,893</u>	<u>16,854</u>	<u>11,805</u>
Non-GAAP SG&A Expense	<u>(14,468)</u>	<u>(12,799)</u>	<u>(25,476)</u>	<u>(22,742)</u>
Change in valuation of contingent consideration	2,789	120	5,485	120
Subtotal non-GAAP items	<u>14,248</u>	<u>8,955</u>	<u>27,629</u>	<u>16,307</u>
<b>Non-GAAP Operating Income</b>	<b>\$ 2,869</b>	<b>\$ 2,011</b>	<b>\$ 8,008</b>	<b>\$ 1,258</b>
<b>GAAP Net Income (Loss)</b>	<b>\$ (13,403)</b>	<b>\$ (25,527)</b>	<b>\$ (2,187)</b>	<b>\$ 14,128</b>
Non-GAAP Operating Loss Adjustments	14,248	8,955	27,629	16,307
Change in fair value of derivative instruments	9,063	29,418	(5,277)	66,171

Bargain purchase gain	—	—	—	(49,063)
Income tax benefit (expense)	(7,392)	15	(12,462)	(40,006)
<b>Non-GAAP Net Income</b>	<b>\$ 2,516</b>	<b>\$ 12,861</b>	<b>\$ 7,703</b>	<b>\$ 7,537</b>

**Per share data:**

Net gain (loss) per common share, basic	\$ 0.07	\$ 0.37	\$ 0.21	\$ 0.24
Weighted average common shares outstanding, basic	36,683,429	34,957,134	36,601,807	31,079,053

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