



Retrophin Reports Third Quarter 2016 Financial Results

November 3, 2016

Positive top-line results from DUET announced in September; additional data to be presented at ASN Kidney Week 2016

Third quarter revenues increased 21 percent year-over-year

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

- Net product sales for the third quarter of 2016 were \$33.9 million, compared to net product sales of \$28.0 million for the same period in 2015
- Cash, cash equivalents, marketable securities, and note receivable as of September 30, 2016 totaled \$320.8 million
- Positive top-line results from the Phase 2 DUET study of sparsentan in focal segmental glomerulosclerosis (FSGS) announced in the third quarter; additional data from DUET to be featured as a late-breaking oral presentation at American Society of Nephrology (ASN) Kidney Week 2016
- Trial evaluating the efficacy of RE-024 in pantothenate kinase-associated neurodegeneration (PKAN) remains on track to initiate before year-end 2016

"Positive top-line data from the DUET study demonstrating sparsentan's potential to be a significant advancement in the treatment of FSGS enabled a pivotal third quarter for Retrophin," said Stephen Aselage, chief executive officer of Retrophin. "With the RE-024 efficacy study in PKAN patients on track to initiate by year-end and further top-line revenue growth expected, we are in position to deliver on all of our transformational milestones in 2016."

Quarter Ended September 30, 2016

Net product sales for the third quarter of 2016 were \$33.9 million, compared to \$28.0 million for the same period in 2015. For the nine months ended September 30, 2016, net product sales were \$96.3 million, compared to \$69.4 million for the same period in 2015. The increase is attributable to growth in the number of patients receiving the Company's commercial products: Thiola[®], Cholbam[®], and Chenodal[®]. The Company reiterates its full-year guidance of \$130.0 to \$140.0 million in net product sales for 2016.

Selling, general and administrative (SG&A) expenses for the third quarter of 2016 were \$23.8 million, compared to \$22.3 million for the same period in 2015. For the nine months ended September 30, 2016, SG&A expenses were \$66.2 million, compared to \$56.9 million for the same period in 2015. The increase is largely due to expanded sales and marketing efforts in support of the Company's commercial products. On a non-GAAP adjusted basis, SG&A expenses were \$15.0 million for the third quarter of 2016, compared to \$13.1 million for the same period in 2015.

Research and development (R&D) expenses for the third quarter of 2016 were \$18.4 million, compared to \$14.1 million for the same period in 2015. For the nine months ended September 30, 2016, R&D expenses were \$50.8 million, compared to \$35.0 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 \$15.4 million for the third quarter of 2016, compared to \$11.2 million for the same period in 2015.

During the third quarter of 2016, the Company recorded a non-recurring legal fee settlement charge of \$5.2 million related to the advancement of legal fees.

Total other expense for the third quarter of 2016 was \$10.3 million, compared to other income of \$164.8 million for the same period in 2015. For the nine months ended September 30, 2016, total other expense was \$5.3 million, compared to total other income of \$154.0 million for the same period in 2015. The difference is largely attributable to the sale of the Company's Priority Review Voucher and corresponding credit facility prepayment in July 2015, which resulted in a gain on sale of assets and loss on extinguishment of debt, as well as a greater derivative liability resulting from share price appreciation during the third quarter of 2016.

Tax expense of \$6.5 million for the third quarter of 2016 was primarily due to an increase in valuation allowance against deferred tax assets.

Net loss for the third quarter of 2016 was \$37.1 million, or \$1.00 per basic share, compared to net income of \$105.6 million, or \$2.95 per basic share for the same period in 2015. For the nine months ended September 30, 2016, net loss was \$39.3 million, compared to a net income of \$119.7 million for the same period in 2015. Non-GAAP adjusted net loss for the third quarter of 2016 was \$3.4 million, or \$0.09 per basic share, compared to a net loss of \$1.9 million, or \$0.05 per basic share for the same period in 2015.

As of September 30, 2016, the Company had cash, cash equivalents, marketable securities and note receivable of \$320.8 million.

Commercial Product Updates

Thiola[®] (tiopronin)

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

Cholbam[®] (cholic acid)

- The number of patients receiving treatment with Cholbam remained stable during the third quarter of 2016.
- Physician use of the Retrophin-sponsored Neonatal and Adult Cholestasis Sequencing Panel continued to gain traction during the third quarter of 2016.

Chenodal® (chenodeoxycholic acid)

- The number of patients receiving treatment with Chenodal remained stable during the third quarter of 2016.
- Approximately 30 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 third quarter of 2016, the Company reported positive top-line data from the Phase 2 DUET study of sparsentan in FSGS. A meeting with the U.S. Food and Drug Administration (FDA) to determine the most expeditious regulatory path forward is planned for January 2017.
- Additional data from the DUET study will be presented on November 19, during the late-breaking High-Impact Clinical Trials oral session at ASN Kidney Week 2016.
- Further supportive data from the sparsentan program, as well as observational cohort data characterizing the association between proteinuria reduction and improved clinical outcomes in FSGS patients will also be presented during ASN Kidney Week 2016.

RE-024

- The Company remains on track to initiate its efficacy trial evaluating RE-024 in PKAN before year-end 2016.
- 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)

- The Company remains on track to file a New Drug Application with the FDA for the treatment of primary biliary cholangitis (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, November 3, 2016 at 4:30 p.m. ET to discuss third 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 1738305 shortly before 4:30 p.m. ET. The webcast can be accessed at www.retrophin.com, in the Events and Presentations section. A replay of the call will be available starting at 7:30 p.m. ET, November 3, 2016 until 11:59 p.m. ET, November 10, 2016. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 1738305.

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[®], 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, 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 the risk that additional clinical trials will be required for regulatory approvals, risk that additional clinical trials, if any, will fail to demonstrate that sparsentan is safe or effective and risk that the sparsentan 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, 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 (in thousands, except share amounts)

	September 30, 2016	December 31, 2015
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 23,895	\$ 37,805
Marketable securities	250,408	191,799
Accounts receivable, net	14,955	12,458
Inventory, net	3,253	2,536
Prepaid expenses and other current assets	3,553	2,378
Prepaid taxes	—	8,107
Note receivable, current	46,526	46,849
Total current assets	342,590	301,932
Property and equipment, net	382	428
Other assets	1,974	1,859
Prepaid tax asset	3,240	—
Intangible assets, net	183,298	161,536
Goodwill	936	936
Note receivable, long term	—	45,573

Total assets	<u>\$ 532,420</u>	<u>\$ 512,264</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 3,066	\$ 7,639
Accrued expenses	40,376	23,820
Other current liabilities	1,143	958
Guaranteed minimum royalty	2,000	2,000
Tax payable	3,879	—
Business combination-related contingent consideration	6,570	13,754
Derivative financial instruments, warrants	28,960	38,810
Total current liabilities	<u>85,994</u>	<u>86,981</u>
Convertible debt	44,257	43,766
Other non-current liabilities	2,461	3,066
Guaranteed minimum royalty, less current portion	8,281	8,885
Business combination-related contingent consideration, less current portion	75,370	45,267
Deferred income tax liability, net	8,462	24,328
Total liabilities	<u>224,825</u>	<u>212,293</u>
Stockholders' Equity:		
Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2016 and December 31, 2015	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 37,703,996 and 36,465,853 issued and outstanding as of September 30, 2016 and December 31, 2015, respectively	4	4
Additional paid-in capital	412,348	365,802
Accumulated deficit	(104,453)	(65,153)
Accumulated other comprehensive loss	(304)	(682)
Total stockholders' equity	<u>307,595</u>	<u>299,971</u>
Total liabilities and stockholders' equity	<u>\$ 532,420</u>	<u>\$ 512,264</u>

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,	
	2016	2015	2016	2015
Net product sales	\$ 33,945	\$ 28,005	\$ 96,265	\$ 69,444
Operating expenses:				
Cost of goods sold	1,573	513	3,351	1,424
Research and development	18,428	14,064	50,775	34,974
Selling, general and administrative	23,848	22,308	66,178	56,856
Legal Fee Settlement	5,212	—	5,212	—
Change in fair value of contingent consideration	5,256	6,906	10,741	7,026
Impairment of intangible assets	—	4,710	—	4,710
Total operating expenses	<u>54,317</u>	<u>48,501</u>	<u>136,257</u>	<u>104,990</u>
Operating loss	<u>(20,372)</u>	<u>(20,496)</u>	<u>(39,992)</u>	<u>(35,546)</u>

Other income (expenses), net:				
Other income (expenses), net	151	(314)	156	35
Interest expense, net	(299)	(695)	(609)	(7,415)
Debt early payment penalty	—	—	—	(2,250)
Finance expense	—	—	—	(600)
Change in fair value of derivative instruments	(10,126)	29,991	(4,849)	(36,180)
Gain on sale of assets	—	140,004	—	140,004
Loss on extinguishment of debt	—	(4,151)	—	(4,151)
Litigation settlement gain	—	—	—	15,500
Bargain purchase gain	—	—	—	49,063
Total other income (loss), net	<u>(10,274)</u>	<u>164,835</u>	<u>(5,302)</u>	<u>154,006</u>
Income (loss) before provision for income taxes	(30,646)	144,339	(45,294)	118,460
Income tax benefit (expense)	<u>(6,467)</u>	<u>(38,761)</u>	<u>5,994</u>	<u>1,246</u>
Net income (loss)	<u>\$ (37,113)</u>	<u>\$ 105,578</u>	<u>\$ (39,300)</u>	<u>\$ 119,706</u>
Net earnings (loss) per common share, basic	<u>\$ (1.00)</u>	<u>\$ 2.95</u>	<u>\$ (1.07)</u>	<u>\$ 3.67</u>
Net earnings (loss) per common share, diluted	<u>\$ (1.00)</u>	<u>\$ 1.78</u>	<u>\$ (1.07)</u>	<u>\$ 3.30</u>
Weighted average common shares outstanding, basic	<u>36,980,356</u>	<u>35,741,877</u>	<u>36,728,911</u>	<u>32,650,408</u>
Weighted average common shares outstanding, diluted	<u>36,980,356</u>	<u>42,752,859</u>	<u>36,728,911</u>	<u>36,800,536</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 September 30,		Nine Months Ended September 30,	
	2016	2015	2016	2015
GAAP operating loss	\$ (20,372)	\$ (20,496)	\$ (39,992)	\$ (35,546)
R&D operating expense	(18,428)	(14,064)	(50,775)	(34,974)
Stock compensation	2,935	2,692	8,061	6,661
Amortization & depreciation	82	200	246	614
Subtotal non-GAAP items	<u>3,017</u>	<u>2,892</u>	<u>8,307</u>	<u>7,275</u>
Non-GAAP R&D expense	<u>(15,411)</u>	<u>(11,172)</u>	<u>(42,468)</u>	<u>(27,699)</u>
SG&A operating expense	(23,848)	(22,308)	(66,178)	(56,856)
Stock compensation	4,814	5,489	13,973	12,088
Amortization & depreciation	4,013	3,712	11,708	8,918
Subtotal non-GAAP items	<u>8,827</u>	<u>9,201</u>	<u>25,681</u>	<u>21,006</u>
Non-GAAP SG&A expense	<u>(15,021)</u>	<u>(13,107)</u>	<u>(40,497)</u>	<u>(35,850)</u>
Change in valuation of contingent consideration	5,256	6,906	10,741	7,026
Impairment of intangible assets	—	4,710	—	4,710

Subtotal non-GAAP items	17,100	23,709	44,729	40,017
Non-GAAP operating income (loss)	\$ (3,272)	\$ 3,213	\$ 4,737	\$ 4,471
GAAP net income (loss)	\$ (37,113)	\$ 105,578	\$ (39,300)	\$ 119,706
Non-GAAP operating loss adjustments	17,100	23,709	44,729	40,017
Change in fair value of derivative instruments	10,126	(29,991)	4,849	36,180
Bargain purchase gain	—	—	—	(49,063)
Gain on sale of assets	—	(140,004)	—	(140,004)
Income tax benefit (expense)	6,467	38,761	(5,994)	(1,246)
Non-GAAP net income (loss)	\$ (3,420)	\$ (1,947)	\$ 4,284	\$ 5,590
Per share data:				
Net gain (loss) per common share, basic	<u>\$ (0.09)</u>	<u>\$ (0.05)</u>	<u>\$ 0.12</u>	<u>\$ 0.17</u>
Weighted average common shares outstanding, basic	<u>36,980,356</u>	<u>35,741,877</u>	<u>36,728,911</u>	<u>32,650,408</u>

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Source: Retrophin, Inc.

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