



May 4, 2017

Retrophin Reports First Quarter 2017 Financial Results

Phase 3 FORT study of RE-024 in PKAN to begin dosing mid-2017

Protocol development underway for pivotal Phase 3 trial of sparsentan in FSGS

First quarter revenues rose 16 percent to \$34 million

SAN DIEGO, May 04, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its first quarter 2017 financial results and provided a corporate update.

- | End of Phase 2 meeting during the first quarter established regulatory path forward for sparsentan in focal segmental glomerulosclerosis (FSGS); protocol development for single pivotal Phase 3 trial ongoing
- | Pivotal FORT study evaluating RE-024 in pantothenate kinase-associated neurodegeneration (PKAN) remains on track to initiate patient dosing mid-year 2017
- | Net product sales for the first quarter of 2017 were \$33.6 million, compared to net product sales of \$29.0 million for the same period in 2016
- | Cash, cash equivalents, marketable securities, and note receivable as of March 31, 2017 totaled \$295.3 million

"I'm very pleased with our progress to start the year," said Stephen Aselage, chief executive officer of Retrophin. "Our meeting with the FDA in the first quarter helped define the path forward for sparsentan, and progress continues on the development of a protocol that will enable us to begin the pivotal trial in FSGS. In addition, our recent advancements with RE-024 have positioned us to begin dosing the first PKAN patients in our Phase 3 study mid-year. These clinical achievements, along with strong operational performance during the quarter, have built solid momentum for the remainder of 2017."

Quarter Ended March 31, 2017

Net product sales for the first quarter of 2017 were \$33.6 million, compared to \$29.0 million for the same period in 2016. The increase in net product sales is attributable to growth across the Company's commercial products: Thiola[®], Cholbam[®], and Chenodal[®]. The Company reiterates its full-year 2017 guidance of \$150.0 to \$160.0 million in net product sales.

Research and development (R&D) expenses for the first quarter of 2017 were \$20.9 million, compared to \$14.7 million for the same period in 2016. The increase is largely attributable to enhanced clinical efforts related to sparsentan and RE-024. On a non-GAAP adjusted basis, R&D expenses were \$18.1 million for the first quarter of 2017, compared to \$12.1 million for the same period in 2016.

Selling, general and administrative (SG&A) expenses for the first quarter of 2017 were \$23.1 million, compared to \$19.1 million for the same period in 2016. The difference is largely attributable to an increase in marketing expense related to the growth of the Company's commercial portfolio, and a one-time benefit of \$3.0 million in the first quarter of 2016 due to a settlement covering disputed legal fees. On a non-GAAP adjusted basis, SG&A expenses were \$14.5 million for the first quarter of 2017, compared to \$11.0 million for the same period in 2016.

Total other income for the first quarter of 2017 was \$1.3 million, compared to \$14.4 million for the same period in 2016. The decrease is largely due to a change in the fair value of derivative instruments as a result of changes in the Company's stock price.

Net loss for the first quarter of 2017 was \$11.1 million, or \$0.29 per basic share, compared to net income of \$11.2 million, or \$0.31 per basic share for the same period in 2016. On a non-GAAP adjusted basis, net income for the first quarter of 2017 was \$0.3 million, or \$0.01 per basic share, compared to \$5.2 million, or \$0.14 per basic share for the same period in 2016.

As of March 31, 2017, the Company had cash, cash equivalents, marketable securities and note receivable of \$295.3 million.

Program Updates

Sparsentan

- | Following an End of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) during the first quarter of 2017, the Company announced plans to initiate a pivotal Phase 3 clinical trial of sparsentan in FSGS. Notably, the study will 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, or eGFR, which is widely regarded as the best overall measure of kidney function.
- | The Company expects to finalize the Phase 3 protocol and gain alignment with the FDA in the second half of 2017, with the pivotal trial expected to initiate thereafter.

RE-024

- | Recruitment activities for the Phase 3 FORT study of RE-024 have begun, with the first PKAN patient expected to begin dosing mid-year 2017.
- | The four PKAN patients receiving RE-024 under physician-initiated treatment outside of the U.S. continue to receive therapy and remain stable.

Conference Call Information

Retrophin will host a conference call and webcast today, Thursday, May 4, 2017 at 4:30 p.m. ET to discuss development updates and first quarter 2017 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 10209780 shortly before 4:30 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 7:30 p.m. ET, May 4, 2017 until 7:30 p.m. ET, May 11, 2017. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 10209780.

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 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)

| | March 31, 2017 | December 31, 2016 |
|---|-------------------|----------------------|
| | (unaudited) | |
| Assets | | |
| Current assets: | | |
| Cash and cash equivalents | \$ 41,871 | \$ 41,002 |
| Marketable securities | 206,290 | 214,871 |
| Accounts receivable, net | 15,427 | 18,510 |
| Inventory, net | 4,751 | 2,826 |
| Prepaid expenses and other current assets | 3,241 | 4,831 |
| Prepaid taxes | 3,645 | 3,463 |
| Note receivable, current | 47,173 | 46,849 |
| Total current assets | 322,398 | 332,352 |
| Property and equipment, net | 2,428 | 2,587 |
| Other assets | 3,661 | 7,364 |
| Intangible assets, net | 180,958 | 182,043 |

| | | | |
|---|--|-------------------|-------------------|
| Goodwill | | 936 | 936 |
| Total assets | | <u>\$ 510,381</u> | <u>\$ 525,282</u> |
| Liabilities and Stockholders' Equity | | | |
| Current liabilities: | | | |
| Accounts payable | | \$ 4,671 | \$ 7,522 |
| Accrued expenses | | 30,192 | 33,308 |
| Other current liabilities | | 1,671 | 1,842 |
| Guaranteed minimum royalty | | 2,000 | 2,000 |
| Business combination-related contingent consideration | | 16,841 | 16,150 |
| Derivative financial instruments, warrants | | 21,180 | 22,440 |
| Total current liabilities | | <u>76,555</u> | <u>83,262</u> |
| Convertible debt | | 44,583 | 44,422 |
| Other non-current liabilities | | 3,857 | 4,010 |
| Guaranteed minimum royalty, less current portion | | 7,849 | 8,068 |
| Business combination-related contingent consideration, less current portion | | 72,241 | 71,328 |
| Deferred income tax liability, net | | 4,361 | 6,425 |
| Total liabilities | | <u>209,446</u> | <u>217,515</u> |
| Stockholders' Equity: | | | |
| Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2017 and December 31, 2016 | | — | — |
| Common stock \$0.0001 par value; 100,000,000 shares authorized; 38,161,424 and 37,906,669 issued and outstanding as of March 31, 2017 and December 31, 2016, respectively | | 4 | 4 |
| Additional paid-in capital | | 430,366 | 421,309 |
| Accumulated deficit | | (129,014) | (113,056) |
| Accumulated other comprehensive loss | | (421) | (490) |
| Total stockholders' equity | | <u>300,935</u> | <u>307,767</u> |
| Total liabilities and stockholders' equity | | <u>\$ 510,381</u> | <u>\$ 525,282</u> |

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

| | <u>Three Months Ended March 31,</u> | |
|--|-------------------------------------|----------------|
| | <u>2017</u> | <u>2016</u> |
| Net product sales | \$ 33,620 | \$ 29,008 |
| Operating expenses: | | |
| Cost of goods sold | 709 | 757 |
| Research and development | 20,860 | 14,672 |
| Selling, general and administrative | 23,115 | 19,125 |
| Change in fair value of contingent consideration | 3,344 | 2,695 |
| Total operating expenses | <u>48,028</u> | <u>37,249</u> |
| Operating loss | <u>(14,408)</u> | <u>(8,241)</u> |
| Other income (expenses), net: | | |
| Other income, net | 126 | 210 |
| Interest expense, net | (132) | (163) |
| Change in fair value of derivative instruments | 1,260 | 14,340 |
| Total other income, net | <u>1,254</u> | <u>14,387</u> |
| Income (loss) before provision for income taxes | <u>(13,154)</u> | <u>6,146</u> |
| Income tax benefit | <u>2,064</u> | <u>5,070</u> |

| | | |
|---|-------------|------------|
| Net income (loss) | \$ (11,090) | \$ 11,216 |
| Net earnings (loss) per common share, basic | \$ (0.29) | \$ 0.31 |
| Net loss per common share, diluted | \$ (0.32) | \$ (0.08) |
| Weighted average common shares outstanding, basic | 38,045,317 | 36,520,186 |
| Weighted average common shares outstanding, diluted | 39,158,922 | 37,947,479 |

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 March 31, | |
|---|------------------------------|-------------------|
| | 2017 | 2016 |
| GAAP operating loss | \$ (14,408) | \$ (8,241) |
| R&D operating expense | (20,860) | (14,672) |
| Stock compensation | 2,688 | 2,486 |
| Amortization & depreciation | 81 | 82 |
| Subtotal non-GAAP items | 2,769 | 2,568 |
| Non-GAAP R&D expense | (18,091) | (12,104) |
| SG&A operating expense | (23,115) | (19,125) |
| Stock compensation | 4,405 | 4,307 |
| Amortization & depreciation | 4,203 | 3,810 |
| Subtotal non-GAAP items | 8,608 | 8,117 |
| Non-GAAP SG&A expense | (14,507) | (11,008) |
| Change in valuation of contingent consideration | 3,344 | 2,695 |
| Subtotal non-GAAP items | 14,721 | 13,380 |
| Non-GAAP operating income | \$ 313 | \$ 5,139 |
| GAAP net income (loss) | \$ (11,090) | \$ 11,216 |
| Non-GAAP operating adjustments | 14,721 | 13,380 |
| Change in fair value of derivative instruments | (1,260) | (14,340) |
| Income tax benefit | (2,064) | (5,070) |
| Non-GAAP net income | \$ 307 | \$ 5,186 |
| Per share data: | | |
| Net earnings per common share, basic | \$ 0.01 | \$ 0.14 |
| Weighted average common shares outstanding, basic | 38,045,317 | 36,520,186 |

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