Retrophin Reports First Quarter 2018 Financial Results

May 1, 2018

Company obtains FDA and EMA feedback on pathway to NDA and MAA filing for sparsentan in IgA nephropathy; single Phase 3 trial expected to initiate in the fourth quarter of 2018

Pivotal Phase 3 DUPLEX Study of sparsentan in FSGS underway to support Subpart H NDA filing

Revenue rose 14 percent to $38 million during the first quarter of 2018

SAN DIEGO, May 01, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its first quarter 2018 financial results and provided a corporate update.

- The Company recently obtained regulatory feedback from both the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) on the development pathway for sparsentan in IgA nephropathy (IgAN); a single registration-enabling Phase 3 clinical trial is expected to initiate in the fourth quarter of 2018
- In April 2018, the Company announced that the first patient had been enrolled in the DUPLEX Study, a pivotal Phase 3 clinical trial evaluating sparsentan in focal segmental glomerulosclerosis (FSGS); top-line data from the interim endpoint efficacy analysis are expected in the second half of 2020
- Net product sales for the first quarter of 2018 were $38.4 million, compared to $33.6 million for the same period in 2017
- Cash, cash equivalents and marketable securities, as of March 31, 2018, totaled $264.1 million

"We made significant development advancements to start the year with our recent regulatory feedback on sparsentan's development path in IgA nephropathy and the initiation of our pivotal Phase 3 DUPLEX Study in FSGS," said Stephen Aselage, chief executive officer of Retrophin. "The prospect of running parallel registrational trials is exciting not just for us, but also for patients with FSGS and IgA nephropathy worldwide. We look forward to building upon our momentum in the clinic, as well as the continued growth of our commercial products to further our efforts in delivering life-changing therapies to patients living with rare diseases."

Quarter Ended March 31, 2018

Net product sales for the first quarter of 2018 were $38.4 million, compared to $33.6 million for the same period in 2017. The increase in net product sales is attributable to growth across the Company's commercial products: Chenodal ®, Cholbam® and Thiola®. The Company continues to expect full year 2018 net product sales to be in the range of $170.0 to $180.0 million.

Research and development (R&D) expenses for the first quarter of 2018 were $24.6 million, compared to $20.9 million for the same period in 2017. The difference is largely attributable to support of non-clinical and clinical efforts related to fosmetpantotenate and sparsentan, as well as development funding to support the advancement of CNSA-001. On a non-GAAP adjusted basis, R&D expenses were $23.1 million for the first quarter of 2018, compared to $18.1 million for the same period in 2017.

Selling, general and administrative (SG&A) expenses for the first quarter of 2018 were $26.5 million, compared to $23.1 million for the same period in 2017. The difference is largely attributable to an increase in headcount as a result of the Company's operational growth, as well as marketing initiatives to support its commercial portfolio. On a non-GAAP adjusted basis, SG&A expenses were $19.0 million for the first quarter of 2018, compared to $14.5 million for the same period in 2017.

Total other expense for the first quarter of 2018 was $0.2 million, compared to total other income of $1.3 million for the same period in 2017. The difference is largely attributable to a change in accounting guidelines in which adjustments to the fair value of derivative instruments no longer impacts the net income or loss of the Company.

Net loss for the first quarter of 2018 was $18.4 million, or $0.46 per basic share, compared to $11.1 million, or $0.29 per basic share for the same period in 2017. On a non-GAAP adjusted basis, net loss for the first quarter of 2018 was $5.6 million, or $0.14 per basic share, compared to net income of $0.3 million, or $0.01 per basic share for the same period in 2017.

As of March 31, 2018, the Company had cash, cash equivalents and marketable securities of $264.1 million.

Program Updates

Fosmetpantotenate

- The Company continues to enroll patients in the Phase 3 FORT Study, an international, registrational clinical trial assessing the safety and efficacy of fosmetpantotenate in approximately 82 patients with pantothenate kinase-associated neurodegeneration (PKAN) aged 6 to 65 years. The primary endpoint in the study is the change from baseline in the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale through 24 weeks of treatment. After completing the 24-week treatment period, all patients will be eligible to receive fosmetpantotenate as part of an open-label extension. The FORT Study is expected to be registration-enabling in the U.S. and Europe, and is being

conducted under a Special Protocol Assessment (SPA) agreement, which indicates concurrence by the FDA that the design of the trial can adequately support the filing of a New Drug Application (NDA). The Company anticipates enrollment of pediatric patients to commence in the FORT Study during the second quarter of 2018, and completion of patient enrollment around year-end 2018. Top-line data are expected in the second half of 2019.

- Four PKAN patients receiving fosmetpantotenate for more than three years under physician-initiated treatment outside of the U.S. continue to receive therapy and remain stable.

Sparsentan

- In April 2018, the Company announced the initiation of the pivotal Phase 3 DUPLEX Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 300 patients with FSGS aged 8 to 75 years. The DUPLEX Study protocol provides for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint – the proportion of patients achieving a modified partial remission of proteinuria [urine protein-to-creatinine ratio (Up/C) ≤1.5 g/g and a >40 percent reduction in Up/C from baseline] at Week 36. While the confirmatory primary endpoint of the study is the change in slope of estimated glomerular filtration rate (eGFR) after 108 weeks of treatment, successful achievement of the 36-week interim efficacy endpoint is expected to serve as the basis for Subpart H accelerated approval in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe. Top-line data from the 36-week interim endpoint efficacy analysis are expected in the second half of 2020.

- The Company recently received feedback from both the FDA and EMA indicating a single Phase 3 trial of sparsentan in IgAN could support registration in the U.S. and Europe. Study start-up activities are underway in anticipation of initiating a pivotal study in the fourth quarter of 2018.

CNSA-001

- In the first quarter of 2018, the single ascending dose portion of the CNSA-001 Phase 1 study completed and the program advanced into the multiple ascending dose portion of the study. A Phase 2 proof-of-concept study in patients with phenylketonuria (PKU) remains on track to commence in mid-2018, with results expected to be available in early 2019.

Thiola

- In 2018, the Company expects an NDA to be filed for the new formulation of Thiola for the treatment of cystinuria. Pending approval, the Company expects to begin marketing the new formulation in 2019.

Conference Call Information

Retrophin will host a conference call and webcast today, Tuesday, May 1, 2018 at 4:30 p.m. ET to discuss development updates as well as first quarter 2018 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 1169697 shortly before 4:30 p.m. ET. The webcast can be accessed at 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 from 7:30 p.m. ET, May 1, 2018 to 7:30 p.m. ET, May 8, 2018. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 1169697.

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, stock-based compensation expense, amortization and depreciation expense, revaluation of acquisition related contingent consideration, change in fair value of derivative instruments and income tax; (ii) the historical non-GAAP SG&A expense measures exclude from GAAP SG&A expenses, as applicable, stock-based compensation expense, and amortization and depreciation 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 biopharmaceutical company specializing in identifying, developing and delivering life-changing therapies to people living with rare diseases. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal ®, Cholbam® and Thiola®.

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 current or future clinical trials will not proceed as planned. Specifically, the Company faces the risk that the Phase 3 clinical trial of sparsentan in FSGS will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan as planned; risk that the planned Phase 3 clinical trial of sparsentan in IgAN will not proceed as planned or will not demonstrate that sparsentan is safe or effective or serve as the basis for an NDA filing as planned; risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate 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 exclusivity periods and intellectual property rights of first 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, 2018	December 31, 2017
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 61,117	$ 99,394
Marketable securities	202,939	201,236
Accounts receivable, net	12,981	13,872
Inventory, net	5,142	5,351
Prepaid expenses and other current assets	2,011	3,112
Prepaid taxes	2,613	2,842
Total current assets	286,803	325,807
Property and equipment, net	3,042	3,230
Other assets	6,457	5,556
Investment-equity	15,000	—
Intangible assets, net	188,556	184,817
Goodwill	936	936
Total assets	$ 500,794	$ 520,346

Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,423	$ 18,938
Accrued expenses	31,644	36,018
Other current liabilities	3,958	3,902
Guaranteed minimum royalty	2,000	2,000
Business combination-related contingent consideration	9,500	9,100
Derivative financial instruments, warrants	—	15,710
Total current liabilities	56,525	85,668
Convertible debt	45,238	45,077
Other non-current liabilities	4,617	2,472
Guaranteed minimum royalty, less current portion	12,939	13,095
Business combination-related contingent consideration, less current portion	82,000	80,900
Total liabilities	201,319	227,212
Stockholders' Equity:		
Preferred stock $0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2018 and December 31, 2017	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 39,873,285 and 39,373,745 issued and outstanding as of March 31, 2018 and December 31, 2017, respectively	4	4
Additional paid-in capital	486,717	471,800
Accumulated deficit	(185,717)	(177,655)
Accumulated other comprehensive loss	(1,529)	(1,015)
Total stockholders' equity	299,475	293,134
Total liabilities and stockholders' equity	$ 500,794	$ 520,346

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

	Three Months Ended March 31,	
	2018	**2017**
Net product sales:		
Bile acid products	$ 18,508	$ 15,736
Thiola	19,924	17,884
Total net product sales	38,432	33,620
Operating expenses:		
Cost of goods sold	1,613	709
Research and development	24,636	20,860
Selling, general and administrative	26,468	23,115
Change in fair value of contingent consideration	3,627	3,344
Total operating expenses	56,344	48,028
Operating loss	(17,912)	(14,408)
Other income (expenses), net:		

Other income, net	121	126
Interest expense, net	(358)	(132)
Change in fair value of derivative instruments	—	1,260
Total other income (expense), net	(237)	1,254
Loss before provision for income taxes	(18,149)	(13,154)
Income tax benefit (expense)	(229)	2,064
Net loss	$ (18,378)	$ (11,090)
Net loss per common share:		
Basic	$ (0.46)	$ (0.29)
Diluted	$ (0.46)	$ (0.32)
Weighted average common shares outstanding:		
Basic	39,657,418	38,045,317
Diluted	39,657,418	39,158,922

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,	
	2018	**2017**
GAAP operating loss	**$ (17,912)**	**$ (14,408)**
R&D operating expense	(24,636)	(20,860)
Stock compensation	1,407	2,688
Amortization & depreciation	103	81
Subtotal non-GAAP items	1,510	2,769
Non-GAAP R&D expense	(23,126)	(18,091)
SG&A operating expense	(26,468)	(23,115)
Stock compensation	3,202	4,405
Amortization & depreciation	4,245	4,203
Subtotal non-GAAP items	7,447	8,608
Non-GAAP SG&A expense	(19,021)	(14,507)
Change in valuation of contingent consideration	3,627	3,344
Subtotal non-GAAP items	12,584	14,721
Non-GAAP operating income (loss)	**$ (5,328)**	**$ 313**
GAAP net loss	**$ (18,378)**	**$ (11,090)**
Non-GAAP operating loss adjustments	12,584	14,721
Change in fair value of derivative instruments	—	(1,260)

Income tax benefit (expense)	229	(2,064)
Non-GAAP net income (loss)	**$ (5,565)**	**$ 307**
Per share data:		
Net earnings (loss) per common share, basic	$ (0.14)	$ 0.01
Weighted average common shares outstanding, basic	39,657,418	38,045,317

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Source: Retrophin, Inc.