Retrophin Reports First Quarter 2019 Financial Results

May 7, 2019

Top-line results from pivotal Phase 3 FORT Study in PKAN on-track for 3Q 2019

Enrollment ongoing in Phase 3 clinical trials of sparsentan for patients with rare nephropathies FSGS and IgAN

Continued quarter over quarter growth in net product sales

SAN DIEGO, May 07, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its first quarter 2019 financial results and provided a corporate update.

- Top-line data from the pivotal Phase 3 FORT Study of fosmetpantotenate in pantothenate kinase-associated neurodegeneration (PKAN) are anticipated in the third quarter of 2019, followed by anticipated submissions for a New Drug Application (NDA) in the U.S. and Marketing Authorization Application (MAA) in the EU in 2020
- The Company expects to make a decision on its option to acquire Censa Pharmaceuticals during the third quarter of 2019, following an evaluation of the complete Phase 2 proof-of-concept study results evaluating CNSA-001 in patients with phenylketonuria (PKU)
- The two pivotal Phase 3 studies underway to support potential registration of sparsentan continue to enroll patients with focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN)
- Net product sales for the first quarter of 2019 were $39.6 million, compared to $38.4 million for the same period in 2018
- Cash, cash equivalents and marketable securities, as of March 31, 2019, totaled $447.6 million

"We continue to advance our pipeline to address significant unmet needs for people living with rare disease, and our execution in the first quarter has positioned us to reach the next key milestones this year for each of our late-stage programs," said Eric Dube, Ph.D., chief executive officer of Retrophin. "Fosmetpantotenate and sparsentan remain central to our growth over the next several years and I am pleased with our progress in these pivotal programs. We remain on-track to report top-line results from our pivotal FORT Study in the third quarter, positioning us to potentially deliver the first approved treatment option for patients with PKAN. The ongoing studies to support sparsentan's potential approval as a first-in-class treatment for FSGS and IgAN continue to enroll patients and advance toward the first readout next year. We look forward to building upon our momentum as we work toward our goal of generating multiple NDA and MAA submissions in the coming years and making a difference in the lives of the rare disease patients we serve."

Quarter Ended March 31, 2019

Net product sales for the first quarter of 2019 were $39.6 million, compared to $38.4 million for the same period in 2018. The increase in net product sales is primarily attributable to growth of the Company's commercial product Thiola®. The Company expects full year 2019 net product sales to continue on a similar growth rate compared to 2018.

Research and development (R&D) expenses for the first quarter of 2019 were $33.4 million, compared to $24.6 million for the same period in 2018. The difference is largely attributable to support of clinical and product development efforts related to fosmetpantotenate and sparsentan. On a non-GAAP adjusted basis, R&D expenses were $31.5 million for the first quarter of 2019, compared to $23.1 million for the same period in 2018.

Selling, general and administrative (SG&A) expenses for the first quarter of 2019 were $32.7 million, compared to $26.5 million for the same period in 2018. The difference is largely attributable to increased headcount as a result of the Company's operational growth, and legal expenses. On a non-GAAP adjusted basis, SG&A expenses were $23.2 million for the first quarter of 2019, compared to $19.0 million for the same period in 2018.

During the first quarter of 2019, the Company recorded a loss on impairment of $25.5 million related to the portfolio decision to discontinue the development program for liquid ursodeoxycholic acid (L-UDCA). This was offset by the corresponding write-off of the L-UDCA related contingent consideration of $18.0 million, for a net non-cash expense of $7.5 million. The Company acquired the rights to L-UDCA in 2016 for $0.5 million.

Total other expense for the first quarter of 2019 was $2.3 million, compared to $0.2 million for the same period in 2018. The difference is largely attributable to higher interest expense related to the Company's 2025 convertible notes issued in September 2018.

Net loss for the first quarter of 2019 was $41.0 million, or $0.99 per basic share, compared to $18.4 million, or $0.46 per basic share for the same period in 2018. On a non-GAAP adjusted basis, net loss for the first quarter of 2019 was $26.0 million, or $0.63 per basic share, compared to a net loss of $5.6 million, or $0.14 per basic share for the same period in 2018.

As of March 31, 2019, the Company had cash, cash equivalents and marketable securities of $447.6 million.

Program Updates

Fosmetpantotenate

- Top-line data from the pivotal Phase 3 FORT Study are expected in the third quarter of 2019. The FORT Study is an international, pivotal clinical trial assessing the safety and efficacy of fosmetpantotenate in patients with PKAN. 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 are 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 an NDA.

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

Sparsentan

- The Company continues to enroll patients with FSGS in the pivotal Phase 3 DUPLEX Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled clinical trial evaluating the safety and efficacy of sparsentan in approximately 300 patients. 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 FSGS partial remission of proteinuria endpoint (FPRE), which is defined as 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 endpoint of the study is the change in slope of estimated glomerular filtration rate (eGFR) after 108 weeks of treatment, successful achievement of the interim 36-week proteinuria endpoint is expected to serve as the basis for submission of an NDA under the Subpart H accelerated approval pathway in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe. Top-line efficacy data from the 36-week proteinuria endpoint analysis are expected in the second half of 2020.

- The PROTECT Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled pivotal Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 280 patients with IgAN, continues to enroll patients. The primary efficacy endpoint in the PROTECT Study is the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment. Successful achievement of this endpoint is expected to support submission of an NDA under the Subpart H accelerated approval pathway in the U.S., as well as an application for CMA consideration in Europe. Secondary efficacy endpoints include change in eGFR from baseline to four weeks post-cessation of randomized treatment, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment. Top-line efficacy data from the 36-week proteinuria endpoint analysis are expected in the first half of 2022.

CNSA-001

- CNSA-001 is advancing in a Phase 2 proof-of-concept study under a joint development and option agreement with Censa Pharmaceuticals. The Phase 2 study being conducted in patients with PKU, is a randomized, double-crossover, open-label, active-controlled study of multiple doses of CNSA-001 compared to the maximum recommended dose of sapropterin, the current standard of care. The Company anticipates receiving top-line data in the second quarter of 2019 and making a decision on its option to acquire Censa during the third quarter of 2019, following an evaluation of the complete Phase 2 study results.

Thiola

- The Company continues to expect a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2019 for the new formulation of Thiola. Pending approval, the Company expects to begin marketing the new formulation in the third quarter of 2019.

Conference Call Information

Retrophin will host a conference call and webcast today, Tuesday, May 7, 2019 at 4:30 p.m. ET to discuss company updates as well as first quarter 2019 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 6465286 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 7, 2019 to 7:30 p.m. ET, May 14, 2019. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 6465286.

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 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 disease. 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 commercial 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 demonstrate that sparsentan is safe or effective or serve as the basis for accelerated approval of sparsentan 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 for each of its development programs and for its partner's CNSA-001 program, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials may not proceed on expected timelines or may be delayed for safety, regulatory or other reasons and risk that the product candidates will not be approved for efficacy, 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 third parties; risks associated with its option to acquire Censa Pharmaceuticals and the CNSA-001 program; risk that the NDA for the new formulation of Thiola will not be approved by the FDA; 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-Q, Form 10-K and other filings with the Securities and Exchange Commission.

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RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS
(in thousands, except share amounts)

	March 31, 2019	December 31, 2018
Assets	(unaudited)	

Current assets:		
Cash and cash equivalents	$ 69,838	$ 102,873
Marketable securities	377,808	368,668
Accounts receivable, net	12,713	12,662
Inventory, net	5,571	5,619
Prepaid expenses and other current assets	8,171	4,140
Prepaid taxes	1,412	1,716
Total current assets	475,513	495,678
Property and equipment, net	2,995	3,146
Other non-current assets	13,471	7,709
Investment-equity	15,000	15,000
Intangible assets, net	159,753	186,691
Goodwill	936	936
Total assets	$ 667,668	$ 709,160
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 7,627	$ 6,954
Accrued expenses	46,619	49,695
Other current liabilities	8,292	6,165
Business combination-related contingent consideration	19,000	19,350
2019 Convertible debt	22,537	22,457
Total current liabilities	104,075	104,621
2025 Convertible debt	197,470	195,091
Other non-current liabilities	23,412	17,545
Business combination-related contingent consideration, less current portion	57,000	73,650
Total liabilities	381,957	390,907
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2019 and December 31, 2018	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 41,438,020 and 41,389,524 issued and outstanding as of March 31, 2019 and December 31, 2018, respectively	4	4
Additional paid-in capital	596,644	589,795
Accumulated deficit	(310,994)	(270,017)
Accumulated other comprehensive income (loss)	57	(1,529)
Total stockholders' equity	285,711	318,253
Total liabilities and stockholders' equity	$ 667,668	$ 709,160

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

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

	Three Months Ended March 31,	
	2019	**2018**
	(unaudited)	
Net product sales:		
Thiola	$ 21,180	$ 19,924
Bile acid products	18,390	18,508

Total net product sales	39,570	38,432
Operating expenses:		
Cost of goods sold	1,017	1,613
Research and development	33,443	24,636
Selling, general and administrative	32,669	26,468
Change in fair value of contingent consideration	3,169	3,627
Impairment of L-UDCA IPR&D intangible asset	25,500	—
Write off of L-UDCA contingent consideration	(18,000)	—
Total operating expenses	77,798	56,344
Operating loss	(38,228)	(17,912)
Other income (expenses), net:		
Other income (expense), net	(302)	121
Interest income	2,819	797
Interest expense	(4,865)	(1,155)
Total other expense, net	(2,348)	(237)
Loss before income taxes	(40,576)	(18,149)
Income tax expense	(401)	(229)
Net loss	$ (40,977)	$ (18,378)
Per share data:		
Net loss per common share, basic	$ (0.99)	$ (0.46)
Weighted average common shares outstanding, basic	41,410,314	39,657,418

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

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,	
	2019	2018
GAAP operating loss	**$ (38,228)**	**$ (17,912)**
R&D operating expense	(33,443)	(24,636)
Stock compensation	1,670	1,407
Amortization & depreciation	286	103
Subtotal non-GAAP items	1,956	1,510
Non-GAAP R&D expense	(31,487)	(23,126)
SG&A operating expense	(32,669)	(26,468)
Stock compensation	4,850	3,202
Amortization & depreciation	4,615	4,245
Subtotal non-GAAP items	9,465	7,447
Non-GAAP SG&A expense	(23,204)	(19,021)

Change in fair value of contingent consideration	3,169	3,627
Subtotal non-GAAP items	14,590	12,584
Non-GAAP operating loss	**$ (23,638)**	**$ (5,328)**
GAAP net loss	**$ (40,977)**	**$ (18,378)**
Non-GAAP operating loss adjustments	14,590	12,584
Income tax provision	401	229
Non-GAAP net loss	**$ (25,986)**	**$ (5,565)**
Per share data:		
Net loss per common share, basic	$ (0.63)	$ (0.14)
Weighted average common shares outstanding, basic	41,410,314	39,657,418

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

Source: Retrophin, Inc.