Retrophin Reports Fourth Quarter and Full Year 2018 Financial Results

February 26, 2019

Top-line results from pivotal Phase 3 FORT Study in PKAN expected in 3Q 2019

Two pivotal Phase 3 studies of sparsentan progressing on-track to enable potential first-in-class treatment for both FSGS and IgAN

SAN DIEGO, Feb. 26, 2019 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its fourth quarter and full year 2018 financial results and provided a corporate update.

- The Phase 3 FORT Study of fosmetpantotenate in pantothenate kinase-associated neurodegeneration (PKAN) completed enrollment in December 2018; top-line data are expected in the third quarter of 2019, followed by expected submissions for a New Drug Application (NDA) in the U.S. and Marketing Authorization Application (MAA) in the EU in 2020
- Patient enrollment nears completion in the Phase 2 proof-of-concept study evaluating CNSA-001 in phenylketonuria (PKU); the Company expects to receive top-line data from the study in the second quarter of 2019
- Enrollment in the pivotal Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) continues; top-line data from the interim efficacy analysis are expected in the second half of 2020
- In December 2018, the Company announced that the first patient had been dosed in the pivotal Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN); top-line data are expected in the first half of 2022
- Net product sales for the fourth quarter of 2018 were $43.8 million, compared to $42.2 million for the same period in 2017
- Net product sales for the full year 2018 were $164.2 million, compared to $154.9 million for the same period in 2017
- Cash, cash equivalents and marketable securities, as of December 31, 2018, totaled $471.5 million

“Our teams demonstrated strong execution in the fourth quarter by delivering on key clinical objectives in our fosmetpantotenate and sparsentan development programs, as well as by continuing the period over period growth of our commercial portfolio,” said Eric Dube, Ph.D., chief executive officer of Retrophin. “This is an exciting year for Retrophin as we are positioned to start realizing the value of our pipeline that has the potential to generate multiple NDA and MAA submissions in the coming years, each of which is designed to address significant unmet needs for patients living with rare disease. In 2019, we will be focused on successfully executing the pivotal trials of fosmetpantotenate and sparsentan to fulfill their potential as first-in-class therapies, while continuing the growth of our commercial portfolio.”

Fourth Quarter and Full Year 2018 Financial Results

Net product sales for the fourth quarter of 2018 were $43.8 million, compared to $42.2 million for the same period in 2017. For the full year 2018, net product sales were $164.2 million, compared to $154.9 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 expects 2019 net product sales to continue on a similar growth rate compared to 2018.

Research and development (R&D) expenses for the fourth quarter of 2018 were $32.0 million, compared to $19.6 million for the same period in 2017. For the full year 2018, R&D expenses were $123.8 million, compared to $78.2 million for the same period in 2017. The difference is largely attributable to support of clinical and product development efforts related to fosmetpantotenate and sparsentan, as well as funding to support the advancement of CNSA-001. On a non-GAAP adjusted basis, R&D expenses were $30.1 million for the fourth quarter of 2018, compared to $17.7 million for the same period in 2017. For the full year 2018, non-GAAP adjusted R&D expenses were $116.6 million, compared to $68.9 million in 2017.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2018 were $26.0 million, compared to $26.7 million for the same period in 2017. For the full year 2018, SG&A expenses were $103.7 million, compared to $101.3 million for the same period in 2017. On a non-GAAP adjusted basis, SG&A expenses were $18.1 million for the fourth quarter of 2018, compared to $18.5 million for the same period in 2017. For the full year 2018, non-GAAP adjusted SG&A expenses were $72.4 million, compared to $65.9 million in 2017.

Total other expense for the fourth quarter of 2018 was $2.5 million, compared to other income of $4.1 million for the same period in 2017. For the full year 2018, total other expense was $21.8 million, compared to $4.6 million for the same period in 2017. The difference is largely attributable to a loss on early extinguishment of debt related to the repurchase of approximately half of the Company's outstanding 2019 convertible notes effected in September 2018, as well as higher interest expense related to the Company's 2025 convertible notes issued in September 2018.

Net loss for the fourth quarter of 2018 was $7.5 million, or $0.18 per basic share, compared to $17.6 million, or $0.45 per basic share for the same period in 2017. For the full year 2018, net loss was $102.7 million or $2.54 per basic share, compared to $59.7 million or $1.54 per basic share for the same period in 2017. On a non-GAAP adjusted basis, net loss for the fourth quarter of 2018 was $8.5 million, or $0.21 per basic share, compared to net income of $2.9 million, or $0.07 per basic share for the same period in 2017. For the full year 2018, non-GAAP adjusted net loss was $51.8 million, or $1.28 per basic share, compared to a non-GAAP adjusted net income of $10.2 million, or $0.26 per basic share for the same period in 2017.

As of December 31, 2018, the Company had cash, cash equivalents and marketable securities of $471.5 million.

Program Updates

Fosmetpantotenate

- In December 2018, the Company completed enrollment in the Phase 3 FORT Study, 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 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 an NDA. Top-line data are expected in the third quarter of 2019.

- 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 in 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 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 efficacy 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 data from the 36-week proteinuria endpoint efficacy analysis are expected in the second half of 2020.

- In December 2018, the Company announced that the first patient had been dosed in the pivotal PROTECT Study. The PROTECT Study is a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 280 patients with IgAN aged 18 years or older. 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 data from the 36-week proteinuria endpoint efficacy analysis are expected in the first half of 2022.

CNSA-001

- In February 2019, *Molecular Genetics and Metabolism* published online the results of a first-in-humans, randomized, double-blind, placebo-controlled, dose-ranging, Phase 1 clinical trial in 83 healthy volunteers of CNSA-001, a novel formulation of sepiapterin. These data indicate that CNSA-001 is rapidly and efficiently converted to BH4 in humans and support the continued clinical evaluation of CNSA-001 for the management of PKU. CNSA-001 is advancing under a joint development and option agreement with Censa Pharmaceuticals; the CNSA-001 development program is being run by Censa under the oversight of a joint steering committee.

- Enrollment in the Phase 2 proof-of-concept study evaluating CNSA-001 for the treatment of PKU is nearing completion. The Phase 2 study 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. Top-line data are expected to become available to the Company in the second quarter of 2019. A subsequent decision on exercising the option to acquire Censa is expected to be made following an evaluation of the complete Phase 2 study results.

Thiola

- In the fourth quarter of 2018, the NDA for a new formulation of Thiola for the treatment of cystinuria was accepted for review by the FDA and assigned a Prescription Drug User Fee Act (PDUFA) target action date of June 30, 2019. Pending approval, the Company expects to begin marketing the new formulation in the second half of 2019.

Conference Call Information

Retrophin will host a conference call and webcast today, Tuesday, February 26, 2019 at 4:30 p.m. ET to discuss company updates as well as fourth quarter and full year 2018 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 3878749 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, February 26, 2019 to 7:30 p.m. ET, March 5, 2019. The

replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 3878749.

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 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-K and other filings with the Securities and Exchange Commission.

RETROPHIN, INC. AND SUBSIDIARIES
CONSOLIDATED BALANCE SHEETS

(in thousands, except share amounts)

	December 31, 2018	December 31, 2017
Assets		
Current assets:		
Cash and cash equivalents	$ 102,873	$ 99,394
Marketable securities	368,668	201,236
Accounts receivable, net	14,490	13,872
Inventory, net	5,619	5,351
Prepaid expenses and other current assets	2,312	3,112
Prepaid taxes	1,716	2,842
Total current assets	495,678	325,807
Property and equipment, net	3,146	3,230
Other assets	7,709	5,556
Investment-equity	15,000	—
Intangible assets, net	186,691	184,817
Goodwill	936	936
Total assets	$ 709,160	$ 520,346
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 6,954	$ 18,938
Accrued expenses	49,695	36,018
Guaranteed minimum royalty, short term	2,100	2,000
Other current liabilities	4,065	3,902
Business combination-related contingent consideration	19,350	9,100
Convertible debt	22,457	—
Derivative financial instruments, warrants	—	15,710
Total current liabilities	104,621	85,668
Convertible debt	195,091	45,077
Other noncurrent liabilities	4,496	2,472
Guaranteed minimum royalty, long term	13,049	13,095
Business combination-related contingent consideration, less current portion	73,650	80,900
Total liabilities	390,907	227,212
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2018 and 2017, respectively	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 41,389,524 and 39,373,745 issued and outstanding as of December 31, 2018 and 2017, respectively	4	4
Additional paid-in capital	589,795	471,800
Accumulated deficit	(270,017)	(177,655)
Accumulated other comprehensive loss	(1,529)	(1,015)
Total stockholders' equity	318,253	293,134
Total liabilities and stockholders' equity	$ 709,160	$ 520,346

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)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
	(unaudited)			
Net product sales:				
Thiola	$ 23,855	$ 22,213	$ 89,176	$ 82,311
Bile acid products	19,916	19,964	75,070	72,626
Total net product sales	43,771	42,177	164,246	154,937
Operating expenses:				
Cost of goods sold	1,603	1,174	5,527	3,605
Research and development	31,995	19,576	123,757	78,168
Selling, general and administrative	25,955	26,650	103,654	101,333
Change in fair value of contingent consideration	(10,797)	8,332	11,590	19,389
Restructuring	—	997	(242)	3,608
Legal fee settlement	—	625	—	2,625
Total operating expenses	48,756	57,354	244,286	208,728
Operating loss	(4,985)	(15,177)	(80,040)	(53,791)
Other Income (expense), net:				
Other income (expense), net	(102)	42	(474)	1,107
Interest income	2,697	736	5,499	3,234
Interest expense	(5,065)	(1,069)	(9,810)	(4,422)
Loss on extinguishment of debt	—	—	(17,042)	—
Change in fair value of derivative instruments	—	4,430	—	(4,491)
Total other income (expense), net	(2,470)	4,139	(21,827)	(4,572)
Loss before benefit (provision) for income taxes	(7,455)	(11,038)	(101,867)	(58,363)
Income tax provision	—	(6,580)	(811)	(1,368)
Net loss	$ (7,455)	$ (17,618)	$ (102,678)	$ (59,731)
Net earnings (loss) per common share, basic	$ (0.18)	$ (0.45)	$ (2.54)	$ (1.54)
Net earnings (loss) per common share, diluted	$ (0.18)	$ (0.55)	$ (2.54)	$ (1.54)
Weighted average common shares outstanding, basic	41,275,872	39,325,913	40,433,171	38,769,816
Weighted average common shares outstanding, diluted	41,275,872	40,089,779	40,433,171	38,769,816

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 December 31,		Twelve Months Ended December 31,	
	2018	2017	2018	2017
GAAP operating loss	**$ (4,985)**	**$ (15,177)**	**$ (80,040)**	**$ (53,791)**
R&D operating expense	(31,995)	(19,576)	(123,757)	(78,168)
Stock compensation	1,632	1,837	6,224	8,950
Amortization & depreciation	292	82	976	327
Subtotal non-GAAP items	1,924	1,919	7,200	9,277
Non-GAAP R&D expense	(30,071)	(17,657)	(116,557)	(68,891)
SG&A operating expense	(25,955)	(26,650)	(103,654)	(101,333)
Stock compensation	3,222	3,745	13,550	17,924
Amortization & depreciation	4,587	4,385	17,692	17,477
Subtotal non-GAAP items	7,809	8,130	31,242	35,401
Non-GAAP SG&A expense	(18,146)	(18,520)	(72,412)	(65,932)
Change in fair value of contingent consideration	(10,797)	8,332	11,590	19,389
Subtotal non-GAAP items	(1,064)	18,381	50,032	64,067
Non-GAAP operating income (loss)	**$ (6,049)**	**$ 3,204**	**$ (30,008)**	**$ 10,276**
GAAP net loss	**$ (7,455)**	**$ (17,618)**	**$ (102,678)**	**$ (59,731)**
Non-GAAP operating expense adjustments	(1,064)	18,381	50,032	64,067
Change in fair value of derivative instruments	—	(4,430)	—	4,491
Income tax provision	—	6,580	811	1,368
Non-GAAP net income (loss)	**$ (8,519)**	**$ 2,913**	**$ (51,835)**	**$ 10,195**
Per share data:				
Net earnings per common share, basic	$ (0.21)	$ 0.07	$ (1.28)	$ 0.26
Weighted average common shares outstanding, basic	41,275,872	39,325,913	40,433,171	38,769,816

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

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