Retrophin Reports Third Quarter 2018 Financial Results

November 1, 2018

Pivotal programs enrolling towards first data readout in 2019

Phase 2 study of CNSA-001 in PKU underway; top-line results expected in first half 2019

Completed offering of $276 million convertible senior notes due 2025

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

- Phase 3 FORT Study of fosmetpantotenate in pantothenate kinase-associated neurodegeneration (PKAN) continues toward completion of enrollment; top-line data expected in second half of 2019
- Patient enrollment in the 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
- Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) set to initiate in the fourth quarter of 2018
- Phase 2 proof-of-concept study evaluating CNSA-001 in phenylketonuria (PKU) commenced patient dosing; top-line data expected in first half 2019
- New Drug Application (NDA) to approve the new formulation of Thiola has been submitted to the U.S. Food and Drug Administration (FDA)
- Net product sales for the third quarter of 2018 were $40.7 million, compared to $40.3 million for the same period in 2017
- Cash, cash equivalents and marketable securities, as of September 30, 2018, totaled $478.8 million

“I am pleased with the continued advancement of our first-in-class fosmetpantotenate and sparsentan programs and look forward to generating our first pivotal top-line data readout from the FORT Study next year. Additionally, the Phase 2 proof-of-concept study of CNSA-001 in PKU is underway and we are looking forward to seeing the results from that study in the first half of 2019,” said Stephen Aselage, chief executive officer of Retrophin. “The third quarter also continued our four-year history of delivering period over period revenue growth; while revenue came in below our internal expectations for the quarter, we expect to continue the period over period revenue growth trend in the fourth quarter. Overall, the progress made in the third quarter sets us up to meet additional strategic milestones and end 2018 well-positioned for the coming year.”

Quarter Ended September 30, 2018

Net product sales for the third quarter of 2018 were $40.7 million, compared to $40.3 million for the same period in 2017. For the nine months ended September 30, 2018, net product sales were $120.5 million, compared to $112.8 million for the same period in 2017.

Research and development (R&D) expenses for the third quarter of 2018 were $32.4 million, compared to $19.6 million for the same period in 2017. For the nine months ended September 30, 2018, R&D expenses were $91.8 million, compared to $58.6 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 $30.6 million for the third quarter of 2018, compared to $17.5 million for the same period in 2017.

Selling, general and administrative (SG&A) expenses for the third quarter of 2018 were $26.1 million, compared to $24.9 million for the same period in 2017. For the nine months ended September 30, 2018, SG&A expenses were $77.7 million, compared to $74.7 million for the same period in 2017. The difference is largely attributable to an increase in commercial expenses. On a non-GAAP adjusted basis, SG&A expenses were $18.3 million for the third quarter of 2018, compared to $15.4 million for the same period in 2017.

Total other expense for the third quarter of 2018 was $18.5 million, compared to $8.4 million for the same period in 2017. The difference is largely attributable to higher interest expense and a loss on early extinguishment of debt related to the repurchase of approximately half of the Company’s outstanding convertible notes due 2019 effected in September 2018.

Net loss for the third quarter of 2018 was $54.5 million, or $1.34 per basic share, compared to $17.8 million, or $0.46 per basic share for the same period in 2017. For the nine months ended September 30, 2018, net loss was $95.2 million, compared to $42.1 million for the same period in 2017. On a non-GAAP adjusted basis, net loss for the third quarter of 2018 was $27.8 million, or $0.68 per basic share, compared to net income of $5.9 million, or $0.15 per basic share for the same period in 2017.

As of September 30, 2018, the Company had cash, cash equivalents and marketable securities of $478.8 million. This includes approximately $227 million raised in September 2018, net of expenses and repurchase of approximately half of the Company’s outstanding convertible notes due 2019.

Program Updates

Fosmetpantotenate

- The Company continues to enroll patients in the Phase 3 FORT Study, an international, pivotal clinical trial assessing the

safety and efficacy of fosmetpantotenate in approximately 82 patients with 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 an NDA. Top-line data are expected in the second half 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 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.

- In October 2018, the Company presented new positive data from the ongoing open-label extension of the Phase 2 DUET study of sparsentan in FSGS at the American Society of Nephrology (ASN) Kidney Week 2018. Key findings from the 84-week analysis suggested patients with FSGS who remained on sparsentan during the open-label period achieved additional progressive reduction of proteinuria, and an increasing proportion of patients achieved FPRE. In addition, treatment with sparsentan in the open-label extension was associated with a stabilization of eGFR out to week 84. Sixty-two patients continue to receive treatment with sparsentan in the ongoing open-label extension of DUET.

- In September 2018, the Company presented the design of its upcoming pivotal Phase 3 PROTECT Study of sparsentan in IgAN at the 15th International Symposium on IgA Nephropathy. 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. Retrophin expects that successful achievement of this endpoint will serve as the basis for Subpart H accelerated approval of sparsentan in the U.S. and 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. Retrophin expects to initiate the PROTECT Study during the fourth quarter of 2018.

CNSA-001

- Patient dosing has commenced in the Phase 2 proof-of-concept study evaluating CNSA-001 in patients with PKU. CNSA-001 is advancing under a joint development and option agreement with Censa Pharmaceuticals. 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 the current standard of care. Top-line data are expected to be available in the first half of 2019.

Thiola

- In the third quarter of 2018, an NDA was submitted to the FDA for approval of the new formulation of Thiola for the treatment of cystinuria. Pending acceptance of the submission and subsequent approval, the Company expects to begin marketing the new formulation in 2019.

Conference Call Information

Retrophin will host a conference call and webcast today, Thursday, November 1, 2018 at 4:30 p.m. ET to discuss company updates as well as third 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 9066529 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, November 1, 2018 to 7:30 p.m. ET, November 8, 2018. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 9066529.

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 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 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 succeed 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 accepted for filling or 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, Form 10-Q and other filings with the Securities and Exchange Commission.

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

	September 30, 2018	December 31, 2017
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 154,820	$ 99,394
Marketable securities	323,993	201,236
Accounts receivable, net	13,450	13,872
Inventory, net	5,129	5,351
Prepaid expenses and other current assets	3,456	3,112
Prepaid taxes	2,141	2,842
Total current assets	502,989	325,807
Property and equipment, net	3,323	3,230
Other assets	7,393	5,556
Investment-equity	15,000	—
Intangible assets, net	186,545	184,817
Goodwill	936	936
Total assets	$ 716,186	$ 520,346
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 9,314	$ 18,938
Accrued expenses	43,348	36,018
Guaranteed minimum royalty	2,000	2,000
Other current liabilities	3,833	3,902
Business combination-related contingent consideration	19,000	9,100
Convertible debt	22,377	—
Derivative financial instruments, warrants	—	15,710
Total current liabilities	99,872	85,668
Convertible debt	192,753	45,077
Other non-current liabilities	4,885	2,472
Guaranteed minimum royalty, less current portion	12,611	13,095
Business combination-related contingent consideration, less current portion	87,000	80,900
Total liabilities	397,121	227,212
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2018 and December 31, 2017	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 41,167,942 and 39,373,745 issued and outstanding as of September 30, 2018 and December 31, 2017, respectively	4	4
Additional paid-in capital	582,706	471,800
Accumulated deficit	(262,562)	(177,655)
Accumulated other comprehensive loss	(1,083)	(1,015)
Total stockholders' equity	319,065	293,134
Total liabilities and stockholders' equity	$ 716,186	$ 520,346

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,	
	2018	2017	2018	2017
Net product sales:				
Bile acid products	$ 18,052	$ 18,839	$ 55,153	$ 52,662
Thiola	22,654	21,501	65,322	60,098
Total net product sales	40,706	40,340	120,475	112,760
Operating expenses:				
Cost of goods sold	1,133	925	3,924	2,431
Research and development	32,448	19,610	91,762	58,592
Selling, general and administrative	26,107	24,852	77,699	74,683
Legal fee settlement	—	—	—	2,000
Change in fair value of contingent consideration	16,601	4,429	22,387	11,057
Restructuring	—	1,132	(242)	2,611
Total operating expenses	76,289	50,948	195,530	151,374
Operating loss	(35,583)	(10,608)	(75,055)	(38,614)
Other income (expenses), net:				
Other income (expense), net	(90)	557	(372)	1,065
Interest income	1,147	595	2,905	2,460
Interest expense	(2,533)	(660)	(4,848)	(3,315)
Loss on early extinguishment of debt	(17,042)	—	(17,042)	—
Change in fair value of derivative instruments	—	(8,901)	—	(8,921)
Total other expense, net	(18,518)	(8,409)	(19,357)	(8,711)
Loss before income taxes	(54,101)	(19,017)	(94,412)	(47,325)
Income tax benefit (expense)	(415)	1,223	(811)	5,212
Net loss	$ (54,516)	$ (17,794)	$ (95,223)	$ (42,113)
Net loss per common share:				
Basic and Diluted	$ (1.34)	$ (0.46)	$ (2.37)	$ (1.10)
Weighted average common shares outstanding:				
Basic and Diluted	40,717,440	38,654,086	40,149,184	38,301,893

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,	
	2018	2017	2018	2017
GAAP operating loss	**$ (35,583)**	**$ (10,608)**	**$ (75,055)**	**$ (38,614)**

R&D operating expense	(32,448)	(19,610)	(91,762)	(58,592)
Stock compensation	1,603	1,998	4,592	7,113
Amortization & depreciation	292	83	684	245
Subtotal non-GAAP items	1,895	2,081	5,276	7,358
Non-GAAP R&D expense	(30,553)	(17,529)	(86,486)	(51,234)
SG&A operating expense	(26,107)	(24,852)	(77,699)	(74,683)
Stock compensation	3,282	4,962	10,328	14,179
Amortization & depreciation	4,506	4,533	13,105	13,092
Subtotal non-GAAP items	7,788	9,495	23,433	27,271
Non-GAAP SG&A expense	(18,319)	(15,357)	(54,266)	(47,412)
Change in valuation of contingent consideration	16,601	4,429	22,387	11,057
Subtotal non-GAAP items	26,284	16,005	51,096	45,686
Non-GAAP operating income (loss)	**$ (9,299)**	**$ 5,397**	**$ (23,959)**	**$ 7,072**
GAAP net loss	**$ (54,516)**	**$ (17,794)**	**$ (95,223)**	**$ (42,113)**
Non-GAAP operating loss adjustments	26,284	16,005	51,096	45,686
Change in fair value of derivative instruments	—	8,901	—	8,921
Income tax benefit (expense)	415	(1,223)	811	(5,212)
Non-GAAP net income (loss)	**$ (27,817)**	**$ 5,889**	**$ (43,316)**	**$ 7,282**
Per share data:				
Net earnings (loss) per common share, basic	$ (0.68)	$ 0.15	$ (1.08)	$ 0.19
Weighted average common shares outstanding, basic	40,717,440	38,654,086	40,149,184	38,301,893

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Source: Retrophin, Inc.