Retrophin Reports Fourth Quarter and Full Year 2019 Financial Results

February 24, 2020

Pivotal DUPLEX and PROTECT studies of sparsentan enrolling towards topline readouts to support potential NDA and CMA filings

Full year 2019 net product sales of $175 million

SAN DIEGO, Feb. 24, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its fourth quarter and full year 2019 financial results and provided a corporate update.

- The Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) nears enrollment of 190 patients to support the 36-week proteinuria analysis; topline data on-track for first half of 2021
- The Phase 3 PROTECT Study remains in position to reach enrollment of 280 patients with IgA nephropathy (IgAN) by early 2021 and report topline data in the first half of 2022
- Net product sales for the fourth quarter of 2019 were $46.7 million, compared to $43.8 million for the same period in 2018
- Net product sales for the full year 2019 were $175.3 million, compared to $164.2 million for the full year 2018
- Cash, cash equivalents and marketable securities, as of December 31, 2019, totaled $398.5 million

“In 2019 we strengthened our focus through disciplined decisions to move beyond the clinical programs that did not meet our expectations, and we successfully executed on the key strategies to advance our pivotal studies of sparsentan in FSGS and IgA nephropathy,” said Eric Dube, Ph.D., chief executive officer of Retrophin. “With its potential to become the first medicine approved for FSGS and IgA nephropathy, we are seeing growing enthusiasm for sparsentan among the nephrology community and anticipation for the topline readouts from the 36-week proteinuria assessments in DUPLEX and PROTECT. In 2020 we will continue to focus on completing enrollment and maintaining high quality trial conduct in these two pivotal studies, while simultaneously preparing for regulatory submissions for the programs. Further, we will continue to focus on building upon our existing commercial capabilities in order to support the continued organic growth of our approved products, and ultimately maximizing sparsentan’s potential for patients.”

Fourth Quarter and Full Year 2019 Financial Results

Net product sales for the fourth quarter of 2019 were $46.7 million, compared to $43.8 million for the same period in 2018. For the full year 2019, net product sales were $175.3 million, compared to $164.2 million for the same period in 2018. The increase in net product sales is attributable to growth across the Company’s commercial products including the launch of THIOLA EC ®. In 2020, the Company anticipates mid-single-digit percentage growth in net product sales compared to 2019.

Research and development (R&D) expenses for the fourth quarter of 2019 were $36.4 million, compared to $32.0 million for the same period in 2018. For the full year 2019, R&D expenses were $141.0 million, compared to $123.8 million for the same period in 2018. The difference is largely attributable to increased support of clinical and product development efforts. On a non-GAAP adjusted basis, R&D expenses were $34.5 million for the fourth quarter of 2019, compared to $30.1 million for the same period in 2018. For the full year 2019, non-GAAP adjusted R&D expenses were $132.9 million, compared to $116.6 million in 2018.

Selling, general and administrative (SG&A) expenses for the fourth quarter of 2019 were $27.5 million, compared to $26.0 million for the same period in 2018. For the full year 2019, SG&A expenses were $129.0 million, compared to $103.7 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 professional fees. On a non-GAAP adjusted basis, SG&A expenses were $19.6 million for the fourth quarter of 2019, compared to $18.1 million for the same period in 2018. For the full year 2019, non-GAAP adjusted SG&A expenses were $95.5 million, compared to $72.4 million in 2018.

Total other expense, net, for the fourth quarter of 2019 was $2.1 million, compared to $2.5 million for the same period in 2018. For the full year 2019, total other expense, net, was $9.1 million, compared to $21.8 million for the same period in 2018. The difference is largely attributable to a loss on early extinguishment of debt related to the repurchase of outstanding convertible notes due 2019 effected in September 2018.

Net loss for the fourth quarter of 2019 was $30.3 million, or $0.70 per basic share, compared to $7.5 million, or $0.18 per basic share for the same period in 2018. For the full year 2019, net loss was $146.4 million, or $3.46 per basic share, compared to $102.7 million, or $2.54 per basic share for the same period in 2018. On a non-GAAP adjusted basis, net loss for the fourth quarter of 2019 was $11.2 million, or $0.26 per basic share, compared to a net loss of $8.5 million, or $0.21 per basic share for the same period in 2018. For the full year 2019, non-GAAP adjusted net loss was $89.9 million, or $2.12 per basic share, compared to a non-GAAP adjusted net income of $51.8 million, or $1.28 per basic share for the same period in 2018.

As of December 31, 2019, the Company had cash, cash equivalents and marketable securities of $398.5 million.

Program Updates

Sparsentan

- The Company continues patient enrollment 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 with FSGS. 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 a New Drug Application (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 first half of 2021.

- 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. 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.

Chenodal®

- In January 2020, the Company randomized the first patients in the RESTORE Study, a Phase 3 clinical trial to evaluate the effects of Chenodal® (chenodeoxycholic acid) in approximately 12 patients with cerebrotendinous xanthomatosis (CTX). While Chenodal is not labeled or marketed for CTX, it is currently considered the standard of care. The pivotal study is anticipated to ultimately support an NDA submission for marketing authorization of Chenodal for CTX in the United States.

Conference Call Information

Retrophin will host a conference call and webcast today, Monday, February 24, 2020 at 4:30 p.m. ET to discuss company updates as well as fourth quarter and full year 2019 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 3436545 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 24, 2020 to 7:30 p.m. ET, March 2, 2020. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 3436545.

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 sparsentan, a product candidate in late-stage development for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), rare disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including partnerships with leaders in patient advocacy and government research to identify potential therapeutics for NGLY1 deficiency and Alagille syndrome, conditions with no approved treatment options. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal ®, Cholbam®, Thiola® and Thiola EC®.

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 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 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; and for each of its development programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing 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 regulatory interactions; and risks and uncertainties relating to competitive products, including potential generic competition with certain of the Company's 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.

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

	December 31, 2019	December 31, 2018
Assets		
Current assets:		
Cash and cash equivalents	$ 62,436	$ 102,873
Marketable securities	336,088	368,668
Accounts receivable, net	18,048	12,662
Inventory, net	6,082	5,619
Prepaid expenses and other current assets	5,015	4,140
Prepaid taxes	1,395	1,716
Total current assets	429,064	495,678
Property and equipment, net	2,891	3,146
Other assets	14,709	7,709
Investment-equity	—	15,000
Intangible assets, net	157,200	186,691
Goodwill	936	936
Total assets	$ 604,800	$ 709,160
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	$ 26,614	$ 6,954
Accrued expenses	51,745	49,695
Other current liabilities	8,590	6,165
Business combination-related contingent consideration	8,500	19,350
Convertible debt	—	22,457
Total current liabilities	95,449	104,621
Convertible debt	204,861	195,091

Other noncurrent liabilities	20,894	17,545
Business combination-related contingent consideration, less current portion	62,400	73,650
Total liabilities	383,604	390,907
Stockholders' Equity:		
Preferred stock $0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of December 31, 2019 and 2018, respectively	—	—
Common stock $0.0001 par value; 100,000,000 shares authorized; 43,088,921 and 41,389,524 issued and outstanding as of December 31, 2019 and 2018, respectively	4	4
Additional paid-in capital	636,910	589,795
Accumulated deficit	(416,444)	(270,017)
Accumulated other comprehensive income (loss)	726	(1,529)
Total stockholders' equity	221,196	318,253
Total liabilities and stockholders' equity	$ 604,800	$ 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)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2019	**2018**	**2019**	**2018**
	(unaudited)			
Net product sales:				
Thiola/Thiola EC	$ 26,246	$ 23,855	$ 95,638	$ 89,176
Bile acid products	20,442	19,916	79,700	75,070
Total net product sales	46,688	43,771	175,338	164,246
Operating expenses:				
Cost of goods sold	1,725	1,603	5,234	5,527
Research and development	36,366	31,995	140,963	123,757
Selling, general and administrative	27,533	25,955	128,951	103,654
Change in fair value of contingent consideration	9,231	(10,797)	15,051	11,590
Restructuring	—	—	—	(242)
Impairment of L-UDCA IPR&D intangible asset	—	—	25,500	—
Write off of L-UDCA contingent consideration	—	—	(18,000)	—
Impairment of long-term investment	—	—	15,000	—
Total operating expenses	74,855	48,756	312,699	244,286
Operating loss	(28,167)	(4,985)	(137,361)	(80,040)
Other Income (expense), net:				
Other income (expense), net	359	(102)	(314)	(474)
Interest income	2,180	2,697	10,055	5,499
Interest expense	(4,599)	(5,065)	(18,828)	(9,810)
Loss on extinguishment of debt	—	—	—	(17,042)
Total other expense, net	(2,060)	(2,470)	(9,087)	(21,827)

Loss before benefit (provision) for income taxes	(30,227)	(7,455)	(146,448)	(101,867)
Income tax benefit (provision)	(32)	—	21	(811)
Net loss	$ (30,259)	$ (7,455)	$ (146,427)	$ (102,678)
Net earnings (loss) per common share, basic	$ (0.70)	$ (0.18)	$ (3.46)	$ (2.54)
Net earnings (loss) per common share, diluted	$ (0.70)	$ (0.18)	$ (3.46)	$ (2.54)
Weighted average common shares outstanding, basic	43,023,479	41,275,872	42,339,961	40,433,171
Weighted average common shares outstanding, diluted	43,023,479	41,275,872	42,339,961	40,433,171

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,	
	2019	2018	2019	2018
GAAP operating loss	**$ (28,167)**	**$ (4,985)**	**$ (137,361)**	**$ (80,040)**
R&D operating expense	(36,366)	(31,995)	(140,963)	(123,757)
Stock compensation	1,609	1,632	6,910	6,224
Amortization & depreciation	292	292	1,159	976
Subtotal non-GAAP items	1,901	1,924	8,069	7,200
Non-GAAP R&D expense	(34,465)	(30,071)	(132,894)	(116,557)
SG&A operating expense	(27,533)	(25,955)	(128,951)	(103,654)
Stock compensation	2,888	3,222	14,195	13,550
Amortization & depreciation	4,998	4,587	19,249	17,692
Subtotal non-GAAP items	7,886	7,809	33,444	31,242
Non-GAAP SG&A expense	(19,647)	(18,146)	(95,507)	(72,412)
Change in fair value of contingent consideration	9,231	(10,797)	15,051	11,590
Subtotal non-GAAP items	19,018	(1,064)	56,564	50,032
Non-GAAP operating loss	**$ (9,149)**	**$ (6,049)**	**$ (80,797)**	**$ (30,008)**
GAAP net loss	**$ (30,259)**	**$ (7,455)**	**$ (146,427)**	**$ (102,678)**
Non-GAAP operating loss adjustments	19,018	(1,064)	56,564	50,032
Income tax benefit (provision)	32	—	(21)	811
Non-GAAP net loss	**$ (11,209)**	**$ (8,519)**	**$ (89,884)**	**$ (51,835)**
Per share data:				
Net earnings per common share, basic	$ (0.26)	$ (0.21)	$ (2.12)	$ (1.28)

Weighted average common shares outstanding, basic	43,023,479	41,275,872	42,339,961	40,433,171

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

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