



NEWS RELEASE

Retrophin Reports First Quarter 2020 Financial Results

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Enrollment continues in the pivotal DUPLEX and PROTECT studies of sparsentan

DUPLEX Study of sparsentan in FSGS enrolled first 190 patients required for interim proteinuria analysis

Net product sales increased to \$47.8 million

SAN DIEGO, May 11, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its first quarter 2020 financial results and provided a corporate update.

- In March 2020, the Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) achieved enrollment of the first 190 patients required to support the 36-week proteinuria analysis; data from this 36-week endpoint, if positive, is expected to serve as the basis for accelerated approval submissions in the U.S. and Europe
- The pivotal PROTECT Study of sparsentan in IgA nephropathy (IgAN) continues enrollment toward 280 patients required to enable the 36-week proteinuria analysis for accelerated approval submissions
- Suzanne L. Bruhn, Ph.D., joined the Board of Directors; Dr. Bruhn brings more than 20 years of successful biopharmaceutical development and commercialization experience to the Retrophin Board
- Net product sales for the first quarter of 2020 were \$47.8 million, compared to \$39.6 million for the same period in 2019
- Cash, cash equivalents and marketable securities, as of March 31, 2020, totaled \$356.5 million

"In the first quarter of 2020 we delivered strong results, and leading into the COVID-19 pandemic, generated

positive momentum across all aspects of our business. We achieved the critical milestone of enrolling the first 190 patients in our pivotal DUPLEX Study of sparsentan in FSGS, and our commercial organization further demonstrated its ability to deliver therapies to patients in the rare nephrology and hepatology communities,” said Eric Dube, Ph.D., chief executive officer of Retrophin. “As we navigate the evolving global pandemic, we recognize that these are difficult times for everyone, especially for those families living with rare disease. Our mission has never been more important, and while there are significant uncertainties and challenges to overcome, our goals remain unchanged. I am confident in the operational and financial strength of our organization as well as the resilience of our team members, which I believe will allow us to continue delivering on our near-and long-term priorities and supporting our employees, patients and global communities during this time.”

First Quarter 2020 Financial Results

Net product sales for the first quarter of 2020 were \$47.8 million, compared to \$39.6 million for the same period in 2019. The increase in net product sales is attributable to growth across the Company’s commercial products including the launch of THIOLA EC[®]. The Company estimates that approximately \$1.5 million to \$2.0 million of net product sales in the first quarter of 2020 resulted from increased demand and higher patient compliance rates directly related to the COVID-19 pandemic.

Research and development (R&D) expenses for the first quarter of 2020 were \$30.2 million, compared to \$33.4 million for the same period in 2019. The difference is largely attributable to the discontinuation of the fosmetpantotenate development program during the fourth quarter of 2019. On a non-GAAP adjusted basis, R&D expenses were \$27.8 million for the first quarter of 2020, compared to \$31.5 million for the same period in 2019.

Selling, general and administrative (SG&A) expenses for the first quarter of 2020 were \$33.1 million, compared to \$32.7 million for the same period in 2019. 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 \$24.0 million for the first quarter of 2020, compared to \$23.2 million for the same period in 2019.

Total other expense, net, for the first quarter of 2020 was \$3.1 million, compared to \$2.3 million for the same period in 2019. The difference is largely attributable to a reduction in interest income.

The Company recorded an income tax benefit of \$19.0 million in the first quarter of 2020. This benefit resulted from the CARES Act legislation, which provides for net operating losses to be carried back to preceding taxable years to generate a refund of previously paid income taxes.

Net income for the first quarter of 2020 was \$0.8 million, or \$0.02 per basic share, compared to a net loss of \$41.0 million, or \$0.99 per basic share for the same period in 2019. On a non-GAAP adjusted basis, net loss for the first

quarter of 2020 was \$8.5 million, or \$0.20 per basic share, compared to a net loss of \$26.0 million, or \$0.63 per basic share for the same period in 2019.

As of March 31, 2020, the Company had cash, cash equivalents and marketable securities of \$356.5 million.

COVID-19 Update

- The Company has taken steps in line with guidance from federal, state and local authorities to help protect the health and safety of its team members, healthcare providers and other stakeholders. In particular, the Company has implemented a work from home policy for those able to perform their jobs from home and is effectively supporting the needs of patients and physicians through virtual interactions.
- Retrophin recognizes that the COVID-19 pandemic has created an increased burden on rare disease patients, caregivers and the advocacy organizations that assist them. In response to these heightened needs, Retrophin has created an emergency assistance fund to provide COVID-19 grant support for patient communities as they strive together to navigate these challenging times.
- To date, the Company has maintained continuity in its supply chain and has not had disruption in the supply of its approved or investigational therapies. Assuming the current state of the COVID-19 pandemic, the Company does not anticipate a disruption of supply. The Company's patient support services and direct to patient pharmacy provider remain staffed and fully functional and are continuing to provide uninterrupted access and support to patients prescribed the Company's approved products.
- Retrophin remains committed to its ongoing development plans and clinical programs, and is working closely with clinical sites, investigators, service providers and the patient communities to monitor and best manage the potential impact of the evolving COVID-19 pandemic. For its ongoing clinical studies, the Company is working in alignment with recent COVID-19 related U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) guidance and prioritizing patient safety, continuous supply of investigational medicine, preserving trial conduct and documentation. The Company believes that the previously communicated timelines of its ongoing clinical trials could be impacted as it experiences a slowing in patient enrollment and site initiations due to the COVID-19 impact on the health system.
- The Company is continuing to assess the evolving impacts of the COVID-19 pandemic and will provide additional information at the appropriate time.

Program Updates

Sparsentan

- In March 2020, The Company achieved enrollment of the first 190 patients 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. At this time, the Company continues to believe that top-line efficacy data from the 36-week proteinuria endpoint analysis in the first quarter of 2021 are achievable, but it is continuing to monitor the impact of the evolving COVID-19 pandemic.
- 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 IgAN, continues to enroll. In May 2020, the Company, in conjunction with ongoing FDA dialogue, adopted a measurement of the rate of change in eGFR over 110-weeks following the initiation of randomized treatment, as the confirmatory endpoint of the study and increased the total sample size from 280 patients to 380 patients. The primary efficacy endpoint evaluating the change in proteinuria (urine protein-to-creatinine ratio) from baseline after 36 weeks of treatment of approximately 280 patients remains unchanged. Successful achievement of the proteinuria 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 the aforementioned rate of change in eGFR following the initiation of randomized treatment, over 58-week and 110-week periods, as well as the rate of change in eGFR over 52-week and 104-week periods following the first six weeks of randomized treatment in approximately 380 patients. At this time, the Company continues to believe that top-line efficacy data from the 36-week proteinuria endpoint analysis in the first half of 2022 are achievable, but it is continuing to monitor the impact of the evolving COVID-19 pandemic.

Conference Call Information

Retrophin will host a conference call and webcast today, Monday, May 11, 2020 at 4:30 p.m. ET to discuss company updates as well as first quarter 2020 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 6658444 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 11, 2020 to 7:30 p.m. ET, May 18, 2020. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 6658444.

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 include, but are not limited to, references to the Company's current expectations around timelines for top-line data from the proteinuria endpoints in the DUPLEX and PROTECT Studies, plans for regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe, references to the Company's ability to deliver on its near and long term priorities, statements regarding expected benefits from its operational and financial strength and the expected impacts on the Company's business from the COVID-19 pandemic, including expectations regarding continued supply of its commercial and investigational products, and continued enrollment and conduct of its clinical trials during the pandemic. 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 DUPLEX Study will not demonstrate that sparsentan is safe or effective or serve as a basis for accelerated approval of sparsentan for FSGS as planned; risk that the PROTECT Study will not demonstrate that sparsentan is safe or effective or serve as the basis for accelerated approval of sparsentan for IgAN 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. The Company faces additional risks associated with the potential impacts the COVID-19 pandemic may have on its business, including, but not limited to (i) the Company's ability to continue its ongoing development activities and clinical trials, (ii) the timing of such clinical trials and the release of data from those trials, (iii) the Company's and its suppliers' ability to successfully manufacture its commercial products and product candidates, and (iv) the market for and sales of its commercial products. You are cautioned not to place undue reliance on the 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)

	March 31, 2020 (unaudited)	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	\$ 67,019	\$ 62,436
Available-for-sale debt securities, at fair value (amortized cost \$290,977, allowance for credit losses of \$0 as of March 31, 2020; amortized cost \$335,206, allowance for credit losses of \$0 as of December 31, 2019)	289,441	336,088
Accounts receivable, net	18,085	18,048
Inventory, net	6,593	6,082
Prepaid expenses and other current assets	5,309	5,015
Tax receivable	20,183	1,395
Total current assets	406,630	429,064
Property and equipment, net	2,617	2,891
Other non-current assets	6,803	14,709
Intangible assets, net	156,077	157,200
Goodwill	936	936
Total assets	\$ 573,063	\$ 604,800
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 9,966	\$ 26,614
Accrued expenses	41,590	51,745
Other current liabilities	8,435	—
Business combination-related contingent consideration	8,100	8,590
2019 Convertible debt	—	8,500
Total current liabilities	68,091	95,449
2025 Convertible debt	207,412	204,861
Other non-current liabilities	13,312	20,894
Business combination-related contingent consideration, less current portion	58,500	62,400

Total liabilities	347,315	383,604
Stockholders' Equity:		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of March 31, 2020 and December 31, 2019	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 43,153,215 and 43,088,921 issued and outstanding as of March 31, 2020 and December 31, 2019, respectively	4	4
Additional paid-in capital	642,880	636,910
Accumulated deficit	(415,636)	(416,444)
Accumulated other comprehensive income (loss)	(1,500)	726
Total stockholders' equity	225,748	221,196
Total liabilities and stockholders' equity	\$ 573,063	\$ 604,800

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

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

	Three Months Ended March 31,	
	2020	2019
Net product sales:		
Thiola	\$ 25,488	\$ 21,180
Bile acid products	22,281	18,390
Total net product sales	47,769	39,570
Operating expenses:		
Cost of goods sold	1,370	1,017
Research and development	30,249	33,443
Selling, general and administrative	33,139	32,669
Change in fair value of contingent consideration	(1,922)	3,169
Write off of L-UDCA IPR&D intangible asset	—	25,500
Write off of L-UDCA contingent consideration	—	(18,000)
Total operating expenses	62,836	77,798
Operating loss	(15,067)	(38,228)
Other income (expenses), net:		
Other expense, net	(190)	(302)
Interest income	1,976	2,819
Interest expense	(4,887)	(4,865)
Total other expense, net	(3,101)	(2,348)
Loss before income taxes	(18,168)	(40,576)
Income tax benefit (expense)	18,976	(401)
Net income (loss)	\$ 808	\$ (40,977)
Per share data:		
Net income (loss) per common share, basic	\$ 0.02	\$ (0.99)
Net income (loss) per common share, diluted	\$ 0.02	\$ (0.99)
Weighted average common shares outstanding, basic	43,122,897	41,410,314
Weighted average common shares outstanding, diluted	43,592,499	41,410,314

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,		
	2020	2019	
GAAP operating loss	\$ (15,067) \$ (38,228)
R&D operating expense	(30,249) (33,443)
Stock compensation	2,126	1,670	
Amortization & depreciation	289	286	
Subtotal non-GAAP items	2,415	1,956	
Non-GAAP R&D expense	(27,834) (31,487)
SG&A operating expense	(33,139) (32,669)
Stock compensation	3,784	4,850	
Amortization & depreciation	5,366	4,615	
Subtotal non-GAAP items	9,150	9,465	
Non-GAAP SG&A expense	(23,989) (23,204)
Change in fair value of contingent consideration	(1,922) 3,169	
Subtotal non-GAAP items	9,643	14,590	
Non-GAAP operating loss	\$ (5,424) \$ (23,638)
GAAP net income (loss)	\$ 808	\$ (40,977)
Non-GAAP operating loss adjustments	9,643	14,590	
Income tax (benefit) provision	(18,976) 401	
Non-GAAP net loss	\$ (8,525) \$ (25,986)
Per share data:			
Net loss per common share, basic	\$ (0.20) \$ (0.63)
Weighted average common shares outstanding, basic	43,122,897	41,410,314	

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

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Source: Retrophin, Inc.