



Retrophin Reports Third Quarter 2020 Financial Results

November 5, 2020

Pivotal DUPLEX Study of sparsentan in FSGS nears completion of enrollment; topline proteinuria data expected 1Q21

Pivotal PROTECT Study achieved enrollment of 280th patient with IgAN to enable topline proteinuria data in 3Q21

Expanding pipeline of potential first-in-class therapies with agreement to acquire OT-58 for the treatment of classical homocystinuria

Net product sales increased 15 percent to \$51 million in the third quarter

SAN DIEGO, Nov. 05, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its third quarter 2020 financial results and provided a corporate update.

- Patient enrollment nears completion in the Phase 3 DUPLEX Study evaluating sparsentan in focal segmental glomerulosclerosis (FSGS); topline results from the 36-week interim analysis are expected in the first quarter of 2021
- The Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) achieved enrollment of the 280th patient ahead of schedule; topline data from the 36-week proteinuria analysis are now anticipated in the third quarter of 2021
- Announced agreement to acquire OT-58, an investigational human enzyme replacement therapy with disease modifying potential in Phase 1/2 development for the treatment of classical homocystinuria
- Net product sales for the third quarter of 2020 were \$51.1 million, compared to \$44.4 million for the same period in 2019
- Cash, cash equivalents and marketable securities, as of September 30, 2020, totaled \$456.3 million

"We reported another quarter of strong execution across our clinical, commercial and business development disciplines and are currently on pace to meet or exceed the key objectives that we set at the beginning of the year," said Eric Dube, Ph.D., chief executive officer of Retrophin. "The DUPLEX Study of sparsentan in FSGS is nearing completion of enrollment and the PROTECT Study in IgA nephropathy achieved enrollment of the 280th patient during the quarter, positioning us for topline readouts from the proteinuria endpoints in both pivotal studies next year. In addition, our commercial organization delivered 15 percent growth which reflects our organization's continued focus on meeting the needs of the rare disease community and puts us on track to exceed our guidance for the year."

Dr. Dube continued, "We also recently reached an agreement to acquire OT-58, a Phase 1/2 investigational human enzyme replacement therapy with disease modifying potential for the treatment of classical homocystinuria (HCU). This program will expand our pipeline of potential first-in-class programs, and provide an opportunity for us to leverage our late-stage development and commercialization capabilities with the goal of ultimately delivering a new treatment option for people living with HCU."

Quarter Ended September 30, 2020

Net product sales for the third quarter of 2020 were \$51.1 million, compared to \$44.4 million for the same period in 2019. For the nine months ended September 30, 2020, net product sales were \$147.3 million, compared to \$128.7 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[®]. For the full year 2020, the Company anticipates net product sales will exceed the previous guidance of mid-single-digit percentage growth compared to 2019.

Research and development (R&D) expenses for the third quarter of 2020 were \$32.3 million, compared to \$33.2 million for the same period in 2019. For the nine months ended September 30, 2020, R&D expenses were \$93.4 million, compared to \$104.6 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 \$29.5 million for the third quarter of 2020, compared to \$31.2 million for the same period in 2019.

Selling, general and administrative (SG&A) expenses for the third quarter of 2020 were \$32.0 million, compared to \$29.8 million for the same period in 2019. For the nine months ended September 30, 2020, SG&A expenses were \$100.1 million, compared to \$101.4 million for the same period in 2019. The difference is largely attributable to an increase in professional fees. On a non-GAAP adjusted basis, SG&A expenses were \$22.9 million for the third quarter of 2020, compared to \$22.3 million for the same period in 2019.

Total other expense, net, for the third quarter of 2020 was \$3.1 million, compared to \$2.6 million for the same period in 2019. The difference is largely attributable to a reduction in interest income.

Net loss for the third quarter of 2020 was \$22.5 million, or \$0.44 per basic share, compared to a net loss of \$36.5 million, or \$0.85 per basic share for the same period in 2019. For the nine months ended September 30, 2020, net loss was \$47.8 million, compared to \$116.2 million for the same period in 2019. On a non-GAAP adjusted basis, net loss for the third quarter of 2020 was \$5.6 million, or \$0.11 per basic share, compared to a net loss of \$28.2 million, or \$0.66 per basic share for the same period in 2019.

As of September 30, 2020, the Company had cash, cash equivalents and marketable securities of \$456.3 million. This includes proceeds of approximately \$6.0 million from the \$19.0 million income tax benefit that resulted from the CARES Act legislation and was recorded in the first quarter of 2020.

Program Updates

- The 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 continues to advance towards complete enrollment. In March 2020, the pivotal DUPLEX Study achieved enrollment of the first 190 patients. The DUPLEX Study protocol provides for an unblinded analysis of at least 190 patients to be performed after 36 weeks of treatment to evaluate the interim efficacy endpoint – the proportion of patients achieving a FSGS partial remission of proteinuria endpoint (FPRE), which is defined as urine protein-to-creatinine ratio (Up/C) ≤ 1.5 g/g and a >40 percent reduction in Up/C from baseline, at Week 36. While the confirmatory endpoint of the study is the change in slope of estimated glomerular filtration rate (eGFR) from baseline 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 anticipate reporting topline efficacy data from the 36-week proteinuria endpoint analysis in the first quarter of 2021 and is monitoring the potential impact of the evolving COVID-19 pandemic on this timing.
- In September 2020, the Company achieved enrollment of the first 280 patients in the pivotal PROTECT Study, a global, randomized, multicenter, double-blind, parallel-arm, active-controlled Phase 3 clinical trial evaluating the safety and efficacy of sparsentan in approximately 380 patients with IgAN. The PROTECT Study protocol provides for an unblinded analysis of at least 280 patients to be performed after 36 weeks of treatment to evaluate the primary efficacy endpoint – the change in proteinuria (urine protein-to-creatinine ratio) at Week 36 from baseline. 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 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 anticipates reporting topline efficacy data from the 36-week proteinuria endpoint analysis in the third quarter of 2021 and is monitoring the potential impact of the evolving COVID-19 pandemic on this timing.

Agreement to Acquire Orphan Technologies and OT-58

- In October 2020, Retrophin entered into a definitive agreement to acquire Orphan Technologies Limited, a privately held, clinical-stage biopharmaceutical company focused on the development of product candidate OT-58 for the treatment of classical homocystinuria (HCU). OT-58 is a novel investigational human enzyme replacement therapy being evaluated in Phase 1/2 development for the treatment of classical HCU, a rare metabolic disorder characterized by elevated levels of plasma homocysteine that can lead to life-threatening thrombotic events such as stroke and heart attacks, ophthalmologic and skeletal complications, as well as developmental delay. In preclinical studies, OT-58 has demonstrated an ability to reduce total homocysteine levels and improve clinical parameters. Specifically, dosing of OT-58 in mouse models corrected metabolite levels, including up to 90% reduction in homocysteine levels in plasma and tissues, and appeared to prolong survival, prevent osteoporosis and rescue ocular structure. OT-58 is currently advancing in a Phase 1/2 dose escalation study to assess its safety, tolerability, pharmacokinetics, pharmacodynamics and clinical effects in patients with classical HCU. OT-58 has been granted Rare Pediatric Disease and Fast Track designations by the US Food and Drug Administration (FDA), as well as Orphan Drug designation in the US and Europe.

Under the terms of the agreement, Retrophin will make an upfront payment of \$90 million in cash upon closing of the transaction. Orphan Technologies shareholders will also be eligible to receive up to \$427 million in additional cash payments contingent upon the achievement of key milestones in the development and commercialization of OT-58. Retrophin will also pay a tiered mid-single digit royalty on future net sales of OT-58 in the US and Europe, and potentially make a milestone payment in the event a pediatric rare disease voucher is granted. The transaction is expected to close in the fourth quarter of 2020.

Conference Call Information

Retrophin will host a conference call and webcast today, Thursday, November 5, 2020 at 4:30 p.m. ET to discuss company updates as well as third 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 7362327 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 5, 2020 to 7:30 p.m. ET, November 12, 2020. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 7362327.

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[®].

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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, the Company's ability to meet or exceed the key objectives that it set at the beginning of the year; expectations regarding enrollment projections in the DUPLEX study, revenue guidance for the year and the closing of its planned acquisition of Orphan Technologies; the potential impact upon and benefits to the Company from the proposed acquisition; the potential for OT-58 to ultimately become a new treatment option for HCU; plans for regulatory submissions for sparsentan under the Subpart H accelerated approval pathway in the U.S. and CMA consideration in Europe, and the expected impacts on the Company's business from the COVID-19 pandemic, including expectations regarding 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)

	September 30, 2020	December 31, 2019
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	\$ 200,481	\$ 62,436
Available-for-sale debt securities, at fair value (amortized cost \$254,655, allowance for credit losses of \$0 as of September 30, 2020; amortized cost \$335,206, allowance for credit losses of \$0 as of December 31, 2019)	255,786	336,088
Accounts receivable, net	15,025	18,048
Inventory, net	7,259	6,082
Prepaid expenses and other current assets	5,516	5,015
Tax receivable	13,615	1,395
Total current assets	497,682	429,064
Property and equipment, net	5,696	2,891
Other non-current assets	33,689	14,709
Intangible assets, net	154,217	157,200
Goodwill	936	936
Total assets	\$ 692,220	\$ 604,800
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 9,431	\$ 26,614
Accrued expenses	50,320	51,745
Other current liabilities	6,775	8,590
Business combination-related contingent consideration	8,900	8,500
Total current liabilities	75,426	95,449
Convertible debt	212,651	204,861
Other non-current liabilities	40,330	20,894
Business combination-related contingent consideration, less current portion	62,400	62,400
Total liabilities	390,807	383,604
Stockholders' Equity:		
Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of September 30, 2020 and December 31, 2019	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 51,023,187 and 43,088,921 issued and outstanding as of September 30, 2020 and December 31, 2019, respectively	5	4
Additional paid-in capital	765,307	636,910
Accumulated deficit	(464,253)	(416,444)
Accumulated other comprehensive income	354	726
Total stockholders' equity	301,413	221,196
Total liabilities and stockholders' equity	\$ 692,220	\$ 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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
	<i>(unaudited)</i>			
Net product sales:				
Thiola	\$ 28,227	\$ 24,435	\$ 80,572	\$ 69,393
Bile acid products	22,912	19,938	66,766	59,258
Total net product sales	51,139	44,373	147,338	128,651
Operating expenses:				
Cost of goods sold	1,189	1,513	4,054	3,509
Research and development	32,349	33,220	93,387	104,597
Selling, general and administrative	31,951	29,779	100,061	101,418
Change in fair value of contingent consideration	5,085	(702)	7,448	5,820
Write off 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	—	15,000
Total operating expenses	70,574	78,810	204,950	237,844
Operating loss	(19,435)	(34,437)	(57,612)	(109,193)
Other income (expenses), net:				
Other income (expense), net	553	(496)	788	(673)
Interest income	1,123	2,467	4,414	7,875
Interest expense	(4,767)	(4,547)	(14,287)	(14,230)
Total other expense, net	(3,091)	(2,576)	(9,085)	(7,028)
Loss before income taxes	(22,526)	(37,013)	(66,697)	(116,221)
Income tax (expense) benefit	(23)	523	18,888	53
Net loss	\$ (22,549)	\$ (36,490)	\$ (47,809)	\$ (116,168)
Per share data:				
Basic and diluted net loss per common share	\$ (0.44)	\$ (0.85)	\$ (1.03)	\$ (2.76)
Basic and diluted weighted average common shares outstanding	50,929,575	42,943,828	46,289,103	42,109,618

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 September 30,		Nine Months Ended September 30,	
	2020	2019	2020	2019
GAAP operating loss	\$ (19,435)	\$ (34,437)	\$ (57,612)	\$ (109,193)
R&D operating expense	(32,349)	(33,220)	(93,387)	(104,597)
Stock compensation	2,510	1,735	6,968	5,301
Amortization & depreciation	292	293	870	867
Subtotal non-GAAP items	2,802	2,028	7,838	6,168
Non-GAAP R&D expense	(29,547)	(31,192)	(85,549)	(98,429)
SG&A operating expense	(31,951)	(29,779)	(100,061)	(101,418)
Stock compensation	2,888	2,605	10,294	11,307
Amortization & depreciation	6,168	4,896	17,076	14,251
Subtotal non-GAAP items	9,056	7,501	27,370	25,558
Non-GAAP SG&A expense	(22,895)	(22,278)	(72,691)	(75,860)
Change in fair value of contingent consideration	5,085	(702)	7,448	5,820
Subtotal non-GAAP items	16,943	8,827	42,656	37,546
Non-GAAP operating loss	\$ (2,492)	\$ (25,610)	\$ (14,956)	\$ (71,647)
GAAP net loss	\$ (22,549)	\$ (36,490)	\$ (47,809)	\$ (116,168)
Non-GAAP operating loss adjustments	16,943	8,827	42,656	37,546
Income tax provision (benefit)	23	(523)	(18,888)	(53)
Non-GAAP net loss	\$ (5,583)	\$ (28,186)	\$ (24,041)	\$ (78,675)
Per share data:				
Basic and diluted net loss per common share	\$ (0.11)	\$ (0.66)	\$ (0.52)	\$ (1.87)
Basic and diluted weighted average common shares outstanding	50,929,575	42,943,828	46,289,103	42,109,618

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

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Source: Retrophin, Inc.