



## Retrophin Reports Second Quarter 2020 Financial Results

July 30, 2020

*Enrollment ahead of schedule in Phase 3 PROTECT Study of sparsentan in IgAN; topline proteinuria data from both pivotal studies in FSGS and IgAN now anticipated in 2021*

*Net product sales increased eight percent to \$48 million in the second quarter*

*Cash & equivalents of \$457 million following completion of \$109 million common stock offering*

SAN DIEGO, July 30, 2020 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ: RTRX) today reported its second quarter 2020 financial results and provided a corporate update.

- Topline results from the 36-week proteinuria analysis in the Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) anticipated in the first quarter of 2021
- The Phase 3 PROTECT Study of sparsentan in IgA nephropathy (IgAN) is expected to achieve enrollment of the first 280 patients in 2020; topline data from the 36-week proteinuria analysis are now anticipated in the second half of 2021
- Net product sales for the second quarter of 2020 were \$48.4 million, compared to \$44.7 million for the same period in 2019
- Cash, cash equivalents and marketable securities, as of June 30, 2020, totaled \$457.4 million, including net proceeds of \$108.6 million from a common stock offering

"The needs of patients living with rare disease are greater than ever and I am proud of our organization's steadfast dedication to supporting our communities, advancing our development programs and continuing to deliver our approved products during this challenging time," said Eric Dube, Ph.D., chief executive officer of Retrophin. "While significant uncertainties remain as a result of the ongoing COVID-19 pandemic, the continued focus on our key priorities and recent increase in clinical activity puts us on course to achieve topline results from the 36-week proteinuria endpoint in both the pivotal DUPLEX and PROTECT studies of sparsentan during 2021. For the balance of 2020, we will continue to focus on achieving key enrollment milestones while maintaining high quality conduct in these studies. We are also simultaneously preparing for anticipated regulatory submissions, and building upon our existing commercial capabilities for the potential launch of sparsentan, if approved."

### Quarter Ended June 30, 2020

Net product sales for the second quarter of 2020 were \$48.4 million, compared to \$44.7 million for the same period in 2019. For the six months ended June 30, 2020, net product sales were \$96.2 million, compared to \$84.3 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<sup>®</sup>.

Research and development (R&D) expenses for the second quarter of 2020 were \$30.8 million, compared to \$37.9 million for the same period in 2019. For the six months ended June 30, 2020, R&D expenses were \$61.0 million, compared to \$71.4 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 \$28.2 million for the second quarter of 2020, compared to \$35.8 million for the same period in 2019.

Selling, general and administrative (SG&A) expenses for the second quarter of 2020 were \$35.0 million, compared to \$39.0 million for the same period in 2019. For the six months ended June 30, 2020, SG&A expenses were \$68.1 million, compared to \$71.6 million for the same period in 2019. The difference is largely attributable to a decrease in professional fees. On a non-GAAP adjusted basis, SG&A expenses were \$25.8 million for the second quarter of 2020, compared to \$30.4 million for the same period in 2019.

Total other expense, net, for the second quarter of 2020 was \$2.9 million, compared to \$2.1 million for the same period in 2019. The difference is largely attributable to a reduction in interest income.

Net loss for the second quarter of 2020 was \$26.1 million, or \$0.58 per basic share, compared to a net loss of \$38.7 million, or \$0.92 per basic share for the same period in 2019. For the six months ended June 30, 2020, net loss was \$25.3 million, compared to \$79.7 million for the same period in 2019. On a non-GAAP adjusted basis, net loss for the second quarter of 2020 was \$9.9 million, or \$0.22 per basic share, compared to a net loss of \$24.5 million, or \$0.58 per basic share for the same period in 2019.

As of June 30, 2020, the Company had cash, cash equivalents and marketable securities of \$457.4 million. This includes net proceeds of \$108.6 million from a common stock offering completed during the second quarter of 2020.

### 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)  $\leq 1.5$  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. The protocol for the DUPLEX Study also provides for a sample size reassessment to support the confirmatory portion of the study. 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 in conjunction with the outcome of the planned sample size reassessment on this timing.

- 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 380 patients with IgAN, is enrolling ahead of schedule and is expected to achieve enrollment of the first 280 patients in 2020. 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 second half of 2021 and is monitoring the potential impact of the evolving COVID-19 pandemic on this timing.

#### **Conference Call Information**

Retrophin will host a conference call and webcast today, Thursday, July 30, 2020 at 4:30 p.m. ET to discuss company updates as well as second 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 6024625 shortly before 4:30 p.m. ET. The webcast can be accessed at [retrophin.com](http://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, July 30, 2020 to 7:30 p.m. ET, August 6, 2020. The replay number is +1 (855) 859-2056 (U.S.) or +1 (404) 537-3406 (International), confirmation code 6024625.

#### **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<sup>®</sup>, Cholbam<sup>®</sup>, Thiola<sup>®</sup> and Thiola EC<sup>®</sup>.

[Retrophin.com](http://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, the Company's ability to achieve key enrollment milestones in 2020 while maintaining high quality conduct in these studies, 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.

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

	June 30, 2020 (unaudited)	December 31, 2019
<b>Assets</b>		
<b>Current assets:</b>		
Cash and cash equivalents	\$ 237,170	\$ 62,436
Available-for-sale debt securities, at fair value (amortized cost \$218,596, allowance for credit losses of \$0 as of June 30, 2020; amortized cost \$335,206, allowance for credit losses of \$0 as of December 31, 2019)	220,206	336,088
Accounts receivable, net	14,077	18,048
Inventory, net	6,286	6,082
Prepaid expenses and other current assets	7,714	5,015
Tax receivable	20,109	1,395
<b>Total current assets</b>	<b>505,562</b>	<b>429,064</b>
Property and equipment, net	2,930	2,891
Other non-current assets	13,895	14,709
Intangible assets, net	155,371	157,200
Goodwill	936	936
<b>Total assets</b>	<b>\$ 678,694</b>	<b>\$ 604,800</b>
<b>Liabilities and Stockholders' Equity</b>		
<b>Current liabilities:</b>		
Accounts payable	\$ 10,198	\$ 26,614
Accrued expenses	44,379	51,745

Other current liabilities	7,356	8,590
Business combination-related contingent consideration	8,000	8,500
<b>Total current liabilities</b>	<b>69,933</b>	<b>95,449</b>
2025 Convertible debt	210,009	204,861
Other non-current liabilities	19,507	20,894
Business combination-related contingent consideration, less current portion	60,600	62,400
<b>Total liabilities</b>	<b>360,049</b>	<b>383,604</b>

**Stockholders' Equity:**

Preferred stock \$0.0001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2020 and December 31, 2019	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 50,902,874 and 43,088,921 issued and outstanding as of June 30, 2020 and December 31, 2019, respectively	5	4
Additional paid-in capital	758,945	636,910
Accumulated deficit	(441,704	) (416,444
Accumulated other comprehensive income	1,399	726
Total stockholders' equity	318,645	221,196
<b>Total liabilities and stockholders' equity</b>	<b>\$ 678,694</b>	<b>\$ 604,800</b>

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

	<b>Three Months Ended June 30,</b>		<b>Six Months Ended June 30,</b>	
	<b>2020</b>	<b>2019</b>	<b>2020</b>	<b>2019</b>
	<i>(unaudited)</i>			
Net product sales:				
Thiola/Thiola EC	\$ 26,857	\$ 23,778	\$ 52,345	\$ 44,958
Bile acid products	21,573	20,929	43,854	39,319
Total net product sales	48,430	44,707	96,199	84,277
Operating expenses:				
Cost of goods sold	1,494	979	2,864	1,996
Research and development	30,790	37,934	61,038	71,377
Selling, general and administrative	34,971	38,970	68,110	71,639
Change in fair value of contingent consideration	4,286	3,353	2,363	6,522
Write off of L-UDCA IPR&D intangible asset	—	—	—	25,500
Write off of L-UDCA contingent consideration	—	—	—	(18,000
Total operating expenses	71,541	81,236	134,375	159,034
Operating loss	(23,111	) (36,529	) (38,176	) (74,757
Other income (expenses), net:				
Other income (expense), net	426	125	235	(177
Interest income	1,316	2,589	3,291	5,408
Interest expense	(4,634	) (4,817	) (9,521	) (9,682
Total other expense, net	(2,892	) (2,103	) (5,995	) (4,451
Loss before income taxes	(26,003	) (38,632	) (44,171	) (79,208
Income tax (expense) benefit	(65	) (69	) 18,911	(470
Net loss	\$ (26,068	) \$ (38,701	) \$ (25,260	) \$ (79,678
<b>Per share data:</b>				
Basic and diluted net loss per common share	\$ (0.58	) \$ (0.92	) \$ (0.57	) \$ (1.91
Basic and diluted weighted average common shares outstanding	44,763,843	41,957,860	43,943,370	41,685,599

**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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
<b>GAAP operating loss</b>	\$ (23,111 )	\$ (36,529 )	\$ (38,176 )	\$ (74,757 )
R&D operating expense	(30,790 )	(37,934 )	(61,038 )	(71,377 )
Stock compensation	2,332	1,896	4,458	3,566
Amortization & depreciation	289	288	578	574
Subtotal non-GAAP items	2,621	2,184	5,036	4,140
Non-GAAP R&D expense	(28,169 )	(35,750 )	(56,002 )	(67,237 )
SG&A operating expense	(34,971 )	(38,970 )	(68,110 )	(71,639 )
Stock compensation	3,622	3,852	7,406	8,702
Amortization & depreciation	5,542	4,740	10,908	9,355
Subtotal non-GAAP items	9,164	8,592	18,314	18,057
Non-GAAP SG&A expense	(25,807 )	(30,378 )	(49,796 )	(53,582 )
Change in fair value of contingent consideration	4,286	3,353	2,363	6,522
Subtotal non-GAAP items	16,071	14,129	25,713	28,719
<b>Non-GAAP operating loss</b>	<b>\$ (7,040 )</b>	<b>\$ (22,400 )</b>	<b>\$ (12,463 )</b>	<b>\$ (46,038 )</b>
<b>GAAP net loss</b>	<b>\$ (26,068 )</b>	<b>\$ (38,701 )</b>	<b>\$ (25,260 )</b>	<b>\$ (79,678 )</b>
Non-GAAP operating loss adjustments	16,071	14,129	25,713	28,719
Income tax provision (benefit)	65	69	(18,911 )	470
<b>Non-GAAP net loss</b>	<b>\$ (9,932 )</b>	<b>\$ (24,503 )</b>	<b>\$ (18,458 )</b>	<b>\$ (50,489 )</b>
<b>Per share data:</b>				
Basic and diluted net loss per common share	\$ (0.22 )	\$ (0.58 )	\$ (0.42 )	\$ (1.21 )
Basic and diluted weighted average common shares outstanding	44,763,843	41,957,860	43,943,370	41,685,599



Source: Retrophin, Inc.