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Retrophin Reports Second Quarter 2017 Financial Results

Fosmetpantotenate (RE-024) Phase 3 trial underway in PKAN

Protocol design for pivotal Phase 3 trial of sparsentan in FSGS complete; sparsentan IP estate expanded to 2030 for FSGS

Second quarter revenues increased 16 percent to \$39 million

SAN DIEGO, Aug. 09, 2017 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its second quarter 2017 financial results and provided a corporate update.

- | Dosed the first patient in the Phase 3 FORT (FOsmetpantotenate Replacement Therapy) Study of fosmetpantotenate (RE-024) in pantothenate kinase-associated neurodegeneration (PKAN)
- | Protocol design for Phase 3 trial of sparsentan in focal segmental glomerulosclerosis (FSGS) is complete and preparation for submission of the protocol to the U.S. Food and Drug Administration (FDA) is underway
- | Recent expansion of sparsentan intellectual property estate expected to extend U.S. and European exclusivity for treating glomerulosclerosis, including FSGS, until at least 2030
- | Net product sales for the second quarter of 2017 were \$38.8 million, compared to \$33.3 million for the same period in 2016
- | Cash, cash equivalents and marketable securities, as of June 30, 2017, totaled \$296.0 million

"In recent months, we made great advancements on our strategic priorities to significantly enhance the value of our organization," said Stephen Aselage, chief executive officer of Retrophin. "Underpinned by strong second quarter performance from our base business, we made important progress with both of our late-stage development programs, highlighted by the recent initiation of patient dosing in our Phase 3 FORT study in PKAN and completing the design of our Phase 3 protocol for sparsentan in FSGS. Given that there are no FDA-approved therapies for either of these conditions, we remain focused on the execution of our pivotal trials with the goal of bringing these novel medicines to patients in need."

Quarter Ended June 30, 2017

Net product sales for the second quarter of 2017 were \$38.8 million, compared to \$33.3 million for the same period in 2016. For the six months ended June 30, 2017, net product sales were \$72.4 million, compared to \$62.3 million for the same period in 2016. The increase in net product sales is attributable to growth across the Company's commercial products: Thiola[®], Cholbam[®] and Chenodal[®]. The Company reiterates its full-year 2017 guidance of \$150.0 to \$160.0 million in net product sales.

Research and development (R&D) expenses for the second quarter of 2017 were \$19.5 million, compared to \$17.7 million for the same period in 2016. For the six months ended June 30, 2017, R&D expenses were \$40.3 million, compared to \$32.3 million for the same period in 2016. The increase is largely attributable to non-clinical and clinical efforts related to sparsentan and RE-024. On a non-GAAP adjusted basis, R&D expenses were \$17.0 million for the second quarter of 2017, compared to \$15.0 million for the same period in 2016.

Selling, general and administrative (SG&A) expenses for the second quarter of 2017 were \$28.8 million, compared to \$23.2 million for the same period in 2016. For the six months ended June 30, 2017, SG&A expenses were \$52.0 million, compared to \$42.3 million for the same period in 2016. The difference is largely attributable to an increase in headcount in support of the Company's operational growth, an increase in marketing expenses related to the Company's commercial portfolio, and a non-recurring advancement of approximately \$2.0 million in legal fees. On a non-GAAP adjusted basis, SG&A expenses were \$19.7 million for the second quarter of 2017, compared to \$14.5 million for the same period in 2016.

Total other expense for the second quarter of 2017 was \$1.6 million, compared to \$9.4 million for the same period in 2016. The decrease is largely due to adjustment of the fair value of derivative instruments as a result of changes in the Company's stock price.

Net loss for the second quarter of 2017 was \$13.2 million, or \$0.34 per basic share, compared to \$13.4 million, or \$0.37 per basic share for the same period in 2016. For the six months ended June 30, 2017, net loss was \$24.3 million, compared to

\$2.2 million for the same period in 2016. On a non-GAAP adjusted basis, net income for the second quarter of 2017 was \$1.1 million, or \$0.03 per basic share, compared to \$2.5 million, or \$0.07 per basic share for the same period in 2016.

As of June 30, 2017, the Company had cash, cash equivalents and marketable securities of \$296.0 million.

Program Updates

Fosmetpantotate (RE-024)

- | In July 2017, the Company announced that the first patient had been dosed in the FORT Study, an international, registrational Phase 3 clinical trial assessing the safety and efficacy of fosmetpantotate in approximately 82 patients with PKAN aged 6 to 65 years. The primary endpoint will be the change in score on the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale, from baseline through 24 weeks of treatment. After completing the 24-week treatment period, all patients will be eligible to receive fosmetpantotate as part of an open-label extension. The FORT Study is expected to be registration-enabling in the U.S. and Europe, and is being conducted under a Special Protocol Assessment (SPA) agreement, which indicates concurrence by the FDA that the design of the trial can adequately support a New Drug Application (NDA).
- | Additional study sites participating in the FORT Study are expected to begin patient enrollment over the remainder of 2017.
- | The four PKAN patients receiving RE-024 under physician-initiated treatment outside of the U.S. continue to receive therapy and remain stable.

Sparsentan

- | Following an End of Phase 2 meeting with the FDA in the first quarter of 2017, the Company announced plans to initiate a pivotal Phase 3 clinical trial of sparsentan in FSGS. The study will include an interim analysis of proteinuria to serve as the basis for an NDA filing for Subpart H accelerated approval of sparsentan. The confirmatory endpoint of the study is expected to compare changes from baseline in estimated glomerular filtration rate (eGFR), which is widely regarded as the best overall measure of kidney function.
- | The Company has completed protocol design for its Phase 3 trial of sparsentan in FSGS and is preparing its regulatory submission to gain alignment with the FDA in the second half of 2017, with the pivotal trial expected to initiate thereafter.
- | In the second quarter of 2017, the Company announced the expansion of its intellectual property estate for sparsentan, with newly issued patents in the U.S. and Europe. Notably, these patents covering the use of sparsentan for treating glomerulosclerosis, including FSGS, are expected to extend the Company's exclusivity period for sparsentan until at least 2030.

Conference Call Information

Retrophin will host a conference call and webcast today, Wednesday, August 9, 2017 at 4:30 p.m. ET to discuss development updates and second quarter 2017 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 60017665 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, August 9, 2017 to 7:30 p.m. ET, August 16, 2017. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 60017665.

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, revaluation of acquisition related contingent consideration, stock-based compensation expense, depreciation and amortization expense, change in fair value of derivative instruments; income tax benefit; (ii) the historical non-GAAP SG&A expense measures exclude from GAAP SG&A expenses, as applicable, stock-based compensation expense, and depreciation and amortization 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 fully integrated biopharmaceutical company dedicated to delivering life-changing therapies to people living with rare diseases who have few, if any, treatment options. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including sparsentan for focal segmental glomerulosclerosis (FSGS), a disorder characterized by progressive scarring of the kidney often leading to end-stage renal disease, and RE-024 for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood. Research exploring the potential of early-stage assets in additional rare diseases is also underway. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Thiola[®], Cholbam[®] and Chenodal[®].

Retrophin.com

Forward-Looking Statements

This press release contains "forward-looking statements" as that term is defined in the Private Securities Litigation Reform Act of 1995. Without limiting the foregoing, these statements are often identified by the words "may", "might", "believes", "thinks", "anticipates", "plans", "expects", "intends" or similar expressions. In addition, expressions of our strategies, intentions or plans are also forward-looking statements. Such forward-looking statements are based on current expectations and involve inherent risks and uncertainties, including factors that could delay, divert or change any of them, and could cause actual outcomes and results to differ materially from current expectations. No forward-looking statement can be guaranteed. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are risks and uncertainties associated with the Company's business and finances in general, success of its commercial products as well as risks and uncertainties associated with the Company's preclinical and clinical stage pipeline. Specifically, the Company faces risks associated with market acceptance of its marketed products including efficacy, safety, price, reimbursement and benefit over competing therapies. The risks and uncertainties the Company faces with respect to its preclinical and clinical stage pipeline include risk that the Company's clinical candidates will not be found to be safe or effective and that planned clinical trials will not proceed as planned. Specifically, the Company faces the risk that the planned Phase 3 clinical trial of sparsentan 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 RE-024 will not demonstrate that RE-024 is safe or effective or serve as the basis for an NDA filing as planned; and risk that the Company's product candidates will not be approved for efficacy, safety, regulatory or other reasons, and for each of the programs, risk associated with enrollment of clinical trials for rare diseases and risk that ongoing or planned clinical trials may not succeed or may be delayed for safety, regulatory or other reasons. 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; and risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products. You are cautioned not to place undue reliance on these forward-looking statements as there are important factors that could cause actual results to differ materially from those in forward-looking statements, many of which are beyond our control. The Company undertakes no obligation to publicly update any forward-looking statement, whether as a result of new information, future events, or otherwise. Investors are referred to the full discussion of risks and uncertainties as included in the Company's most recent Form 10-K, Form 10-Q and other filings with the Securities and Exchange Commission.

	June 30, 2017	December 31, 2016
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	\$ 89,265	\$ 41,002
Marketable securities	206,716	214,871
Accounts receivable, net	13,321	18,510
Inventory, net	4,369	2,826
Prepaid expenses and other current assets	2,343	4,831
Prepaid taxes	3,644	3,463
Note receivable, current	—	46,849
Total current assets	<u>319,658</u>	<u>332,352</u>
Property and equipment, net	2,540	2,587
Other assets	3,628	7,364
Intangible assets, net	180,368	182,043
Goodwill	936	936
Total assets	<u>\$ 507,130</u>	<u>\$ 525,282</u>
Liabilities and Stockholders' Equity		
Current liabilities:		
Accounts payable	\$ 9,076	\$ 7,522
Accrued expenses	27,597	33,308
Other current liabilities	1,593	1,842
Guaranteed minimum royalty	2,000	2,000
Business combination-related contingent consideration	16,900	16,150
Derivative financial instruments, warrants	22,460	22,440
Total current liabilities	<u>79,626</u>	<u>83,262</u>
Convertible debt	44,747	44,422
Other non-current liabilities	3,851	4,010
Guaranteed minimum royalty, less current portion	7,624	8,068
Business combination-related contingent consideration, less current portion	74,267	71,328
Deferred income tax liability, net	1,186	6,425
Total liabilities	<u>211,301</u>	<u>217,515</u>
Stockholders' Equity:		
Preferred stock \$0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2017 and December 31, 2016	—	—
Common stock \$0.0001 par value; 100,000,000 shares authorized; 38,290,876 and 37,906,669 issued and outstanding as of June 30, 2017 and December 31, 2016, respectively	4	4
Additional paid-in capital	438,644	421,309
Accumulated deficit	(142,243)	(113,056)
Accumulated other comprehensive loss	(576)	(490)
Total stockholders' equity	<u>295,829</u>	<u>307,767</u>
Total liabilities and stockholders' equity	<u>\$ 507,130</u>	<u>\$ 525,282</u>

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	2016	2017	2016
Net product sales	\$ 38,800	\$ 33,311	\$ 72,420	\$ 62,319
Operating expenses:				
Cost of goods sold	797	1,021	1,506	1,778
Research and development	19,482	17,675	40,342	32,347
Selling, general and administrative	28,835	23,205	51,950	42,330

Change in fair value of contingent consideration	3,284	2,789	6,628	5,485
Total operating expenses	52,398	44,690	100,426	81,940
Operating loss	(13,598)	(11,379)	(28,006)	(19,621)
Other income (expenses), net:				
Other income (expense), net	382	(206)	508	4
Interest expense, net	(658)	(147)	(790)	(309)
Change in fair value of derivative instruments	(1,280)	(9,063)	(20)	5,277
Total other income (expense), net	(1,556)	(9,416)	(302)	4,972
Loss before provision for income taxes	(15,154)	(20,795)	(28,308)	(14,649)
Income tax benefit	1,925	7,392	3,989	12,462
Net loss	\$ (13,229)	\$ (13,403)	\$ (24,319)	\$ (2,187)
Net loss per common share:				
Basic	\$ (0.34)	\$ (0.37)	\$ (0.63)	\$ (0.06)
Diluted	\$ (0.34)	\$ (0.37)	\$ (0.63)	\$ (0.20)
Weighted average common shares outstanding:				
Basic	39,041,145	36,683,429	38,545,982	36,601,807
Diluted	39,041,145	36,683,429	38,545,982	38,063,285

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,	
	2017	2016	2017	2016
GAAP operating loss	\$ (13,598)	\$ (11,379)	\$ (28,006)	\$ (19,621)
R&D operating expense	(19,482)	(17,675)	(40,342)	(32,347)
Stock compensation	2,427	2,641	5,115	5,127
Amortization & depreciation	81	81	162	163
Subtotal non-GAAP items	2,508	2,722	5,277	5,290
Non-GAAP R&D expense	(16,974)	(14,953)	(35,065)	(27,057)
SG&A operating expense	(28,835)	(23,205)	(51,950)	(42,330)
Stock compensation	4,812	4,852	9,217	9,159
Amortization & depreciation	4,356	3,885	8,559	7,695
Subtotal non-GAAP items	9,168	8,737	17,776	16,854
Non-GAAP SG&A expense	(19,667)	(14,468)	(34,174)	(25,476)
Change in valuation of contingent consideration	3,284	2,789	6,628	5,485
Subtotal non-GAAP items	14,960	14,248	29,681	27,629
Non-GAAP operating income	\$ 1,362	\$ 2,869	\$ 1,675	\$ 8,008
GAAP net loss	\$ (13,229)	\$ (13,403)	\$ (24,319)	\$ (2,187)
Non-GAAP operating loss adjustments	14,960	14,248	29,681	27,629
Change in fair value of derivative instruments	1,280	9,063	20	(5,277)
Income tax benefit	(1,925)	(7,392)	(3,989)	(12,462)
Non-GAAP net income	\$ 1,086	\$ 2,516	\$ 1,393	\$ 7,703

Per share data:

Net earnings per common share, basic	\$ 0.03	\$ 0.07	\$ 0.04	\$ 0.21
Weighted average common shares outstanding, basic	<u>39,041,145</u>	<u>36,683,429</u>	<u>38,545,982</u>	<u>36,601,807</u>

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