# Retrophin Reports Second Quarter 2018 Financial Results and Announces CEO Transition Plan

July 26, 2018

*Patients continue to enroll in Phase 3 FORT Study of fosmetpantotenate in PKAN and Phase 3 DUPLEX Study of sparsentan in FSGS*

*Phase 2 proof-of-concept study of CNSA-001 in patients with PKU set to initiate; top-line results expected in early 2019*

*Retrophin Chief Executive Officer Stephen Aselage announces his decision to retire; Board of Directors initiates search for successor*

SAN DIEGO, July 26, 2018 (GLOBE NEWSWIRE) -- Retrophin, Inc. (NASDAQ:RTRX) today reported its second quarter 2018 financial results and provided a corporate update.

- In June 2018, the Company announced the initiation of enrollment for pediatric patients with pantothenate kinase-associated neurodegeneration (PKAN) in the Phase 3 FORT study of fosmetpantotenate following a scheduled clinical safety review by the independent Data Monitoring Committee (DMC); top-line data remains on-track for second half of 2019
- Patient enrollment in the Phase 3 DUPLEX Study of sparsentan in focal segmental glomerulosclerosis (FSGS) continues; top-line data from the interim efficacy analysis are expected in the second half of 2020
- Start-up activities continue in anticipation of initiating a pivotal study of sparsentan in IgA nephropathy (IgAN) during the fourth quarter of 2018
- Phase 2 proof-of-concept study evaluating CNSA-001 in phenylketonuria (PKU) is expected to commence patient dosing in the coming weeks; top-line data are expected in early 2019
- Net product sales for the second quarter of 2018 were $41.3 million, compared to $38.8 million for the same period in 2017
- Cash, cash equivalents and marketable securities, as of June 30, 2018, totaled $255.7 million

“Our sustained clinical and operational execution has further propelled us toward our goal of having three pivotal Phase 3 studies underway by the end of 2018,” said Stephen Aselage, chief executive officer of Retrophin. “In addition, with a Phase 2 proof-of-concept study of CNSA-001 in PKU nearing initiation, we believe we are entering a significant period of growth for Retrophin as our pipeline advances toward major inflection points. This progress underscores our commitment to delivering life-changing therapies to people living with rare diseases who have few or no approved treatment options.”

**Quarter Ended June 30, 2018**

Net product sales for the second quarter of 2018 were $41.3 million, compared to $38.8 million for the same period in 2017. For the six months ended June 30, 2018, net product sales were $79.8 million, compared to $72.4 million for the same period in 2017. The increase in net product sales is attributable to growth across the Company’s commercial products: Chenodal ®, Cholbam® and Thiola®. The Company continues to expect full year 2018 net product sales to be in the range of $170.0 to $180.0 million.

Research and development (R&D) expenses for the second quarter of 2018 were $34.5 million, compared to $19.5 million for the same period in 2017. For the six months ended June 30, 2018, R&D expenses were $59.1 million, compared to $40.3 million for the same period in 2017. The difference is largely attributable to support of non-clinical and clinical efforts related to fosmetpantotenate and sparsentan, as well as development funding to support the advancement of CNSA-001. On a non-GAAP adjusted basis, R&D expenses were $32.6 million for the second quarter of 2018, compared to $17.0 million for the same period in 2017.

Selling, general and administrative (SG&A) expenses for the second quarter of 2018 were $25.1 million, compared to $28.8 million for the same period in 2017. For the six months ended June 30, 2018, SG&A expenses were $51.6 million, compared to $52.0 million for the same period in 2017. The difference is largely attributable to a decrease in legal fees. On a non-GAAP adjusted basis, SG&A expenses were $16.9 million for the second quarter of 2018, compared to $19.7 million for the same period in 2017.

Total other expense for the second quarter of 2018 was $0.6 million, compared to $1.6 million for the same period in 2017. The difference is largely attributable to a change in accounting guidelines in which adjustments to the fair value of derivative instruments no longer impact the net income or loss of the Company.

Net loss for the second quarter of 2018 was $22.3 million, or $0.56 per basic share, compared to $13.2 million, or $0.34 per basic share for the same period in 2017. For the six months ended June 30, 2018, net loss was $40.7 million, compared to $24.3 million for the same period in 2017. On a non-GAAP adjusted basis, net loss for the second quarter of 2018 was $9.9 million, or $0.25 per basic share, compared to net income of $1.1 million, or $0.03 per basic share for the same period in 2017.

As of June 30, 2018, the Company had cash, cash equivalents and marketable securities of $255.7 million.

**Program Updates**

*Fosmetpantotenate*

- The Company continues to enroll patients in the Phase 3 FORT Study, an international, registrational clinical trial assessing the safety and efficacy of fosmetpantotenate in approximately 82 patients with PKAN aged 6 to 65 years. The primary endpoint in the study is the change from baseline in the Pantothenate Kinase-Associated Neurodegeneration Activities of Daily Living (PKAN-ADL) scale through 24 weeks of treatment. After completing the 24-week treatment period, all patients will be eligible to receive fosmetpantotenate 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 U.S. Food and Drug Administration (FDA) that the design of the trial can adequately support the filing of a New Drug Application (NDA). Top-line data are expected in the second half of 2019.

- In June 2018, the Company announced that the independent DMC for the FORT Study completed its scheduled clinical safety review required to open enrollment for pediatric patients. Upon review of the available safety and tolerability data of fosmetpantotenate in adult patients with PKAN in the study, the DMC recommended that the pivotal trial continue as planned, and supported initiation of enrollment in pediatric patients aged 6 to 17 years.

- Four PKAN patients receiving fosmetpantotenate for more than three years under physician-initiated treatment outside of the U.S. continue to receive therapy and remain stable.

*Sparsentan*

- The Company continues to enroll patients in the pivotal Phase 3 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 aged 8 to 75 years. 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 modified partial remission of proteinuria [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 primary endpoint of the study is the change in slope of estimated glomerular filtration rate (eGFR) after 108 weeks of treatment, successful achievement of the 36-week interim efficacy endpoint is expected to serve as the basis for Subpart H accelerated approval in the U.S. and Conditional Marketing Authorization (CMA) consideration in Europe. Top-line data from the 36-week interim endpoint efficacy analysis are expected in the second half of 2020.

- In April 2018, the Company received feedback from both the FDA and European Medicines Agency (EMA) indicating a single Phase 3 trial of sparsentan in IgAN could support registration in the U.S. and Europe. Protocol finalization is underway and study start-up activities continue in anticipation of initiating a pivotal study in IgAN during the fourth quarter of 2018.

- In June 2018, the Company announced that the United States Patent and Trademark Office (USPTO) issued a new patent that expands the Company's current intellectual property by providing coverage for the use of sparsentan in the treatment of IgAN. The new patent also broadens the existing coverage in FSGS to include all doses of sparsentan between 200 and 800 mg/day. The patent has a stated expiration date of March 30, 2030.

*CNSA-001*

- In the second quarter of 2018, CNSA-001, an orally bioavailable proprietary form of sepiapterin under a joint development and option agreement with Censa Pharmaceuticals, completed its Phase 1 study, and the program is advancing to a Phase 2 proof-of-concept study in patients with PKU. The Phase 2 study will be a randomized, double crossover, open-label, active-controlled study of multiple doses of CNSA-001 compared to the maximum recommended dose of the current standard of care. Top-line data are expected to be available in early 2019.

*Thiola*

- In the second half of 2018, the Company expects an NDA to be filed for the new formulation of Thiola for the treatment of cystinuria. Pending approval, the Company expects to begin marketing the new formulation in 2019.

**Chief Executive Officer Transition**

Retrophin also announced today that Stephen Aselage plans to retire as the Company's Chief Executive Officer. The Board of Directors has initiated a global search for his successor that will include both internal and external candidates, and Mr. Aselage will remain in his role as CEO until his replacement is appointed.

Gary Lyons, Chairman of the Retrophin Board of Directors, stated, "Steve is an exceptional leader who has proven instrumental in transforming Retrophin into the innovative biopharmaceutical company it is today. The Board is grateful for Steve's dedication to the company and its mission, and we are committed to building upon his success."

Mr. Aselage commented, "It is a privilege to lead the talented and dedicated team at Retrophin. The company is in a position of strength and has

become a leader in the rare disease community, putting people with rare diseases at the center of everything we do. Given the collective progress our organization has made over the past several years, I am confident that now is the appropriate time to begin transitioning the company to a new leader who can build upon our success and guide Retrophin for the years ahead."

**Conference Call Information**

Retrophin will host a conference call and webcast today, Thursday, July 26, 2018 at 4:30 p.m. ET to discuss company updates as well as second quarter 2018 financial results. To participate in the conference call, dial +1-855-219-9219 (U.S.) or +1-315-625-6891 (International), confirmation code 8268598 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, July 26, 2018 to 7:30 p.m. ET, August 2, 2018. The replay number is +1-855-859-2056 (U.S.) or +1-404-537-3406 (International), confirmation code 8268598.

**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, change in fair value of derivative instruments 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 diseases. The Company's approach centers on its pipeline featuring late-stage assets targeting rare diseases with significant unmet medical needs, including fosmetpantotenate for pantothenate kinase-associated neurodegeneration (PKAN), a life-threatening neurological disorder that typically begins in early childhood, and sparsentan for focal segmental glomerulosclerosis (FSGS) and IgA nephropathy (IgAN), disorders characterized by progressive scarring of the kidney often leading to end-stage renal disease. Research in additional rare diseases is also underway, including a joint development arrangement evaluating the potential of CNSA-001 in phenylketonuria (PKU), a rare genetic metabolic condition that can lead to neurological and behavioral impairment. Retrophin's R&D efforts are supported by revenues from the Company's commercial products Chenodal ®, Cholbam® and Thiola®.

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 current or future 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 planned Phase 3 clinical trial of sparsentan in IgAN will not proceed as planned or will not demonstrate that sparsentan is safe or effective or serve as the basis for an NDA filing as planned; risk that the Phase 3 clinical trial of fosmetpantotenate will not demonstrate that fosmetpantotenate is safe or effective or serve as the basis for an NDA filing as planned; and for each of its development programs and for its partner's CNSA-001 program, 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 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 its option to acquire Censa Pharmaceuticals and the CNSA-001 program; 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.

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

| | June 30, 2018 | December 31, 2017 |
|---|---|---|
| **Assets** | (unaudited) | |
| **Current assets:** | | |
| Cash and cash equivalents | $ 89,305 | $ 99,394 |
| Marketable securities | 166,381 | 201,236 |
| Accounts receivable, net | 12,324 | 13,872 |
| Inventory, net | 5,388 | 5,351 |
| Prepaid expenses and other current assets | 2,906 | 3,112 |
| Prepaid taxes | 2,613 | 2,842 |
| **Total current assets** | 278,917 | 325,807 |
| Property and equipment, net | 3,455 | 3,230 |
| Other assets | 5,351 | 5,556 |
| Investment-equity | 15,000 | — |
| Intangible assets, net | 187,485 | 184,817 |
| Goodwill | 936 | 936 |
| **Total assets** | $ 491,144 | $ 520,346 |
| **Liabilities and Stockholders' Equity** | | |
| **Current liabilities:** | | |
| Accounts payable | $ 7,847 | $ 18,938 |
| Accrued expenses | 35,270 | 36,018 |
| Guaranteed minimum royalty | 2,000 | 2,000 |
| Other current liabilities | 3,479 | 3,902 |
| Business combination-related contingent consideration | 9,500 | 9,100 |
| Convertible Debt | 45,401 | — |
| Derivative financial instruments, warrants | — | 15,710 |
| **Total current liabilities** | 103,497 | 85,668 |
| Convertible debt | — | 45,077 |
| Other non-current liabilities | 4,880 | 2,472 |
| Guaranteed minimum royalty, less current portion | 12,778 | 13,095 |
| Business combination-related contingent consideration, less current portion | 82,000 | 80,900 |
| **Total liabilities** | 203,155 | 227,212 |
| **Stockholders' Equity:** | | |
| Preferred stock $0.001 par value; 20,000,000 shares authorized; 0 issued and outstanding as of June 30, 2018 and December 31, 2017 | — | — |
| Common stock $0.0001 par value; 100,000,000 shares authorized; 40,370,521 and 39,373,745 issued and outstanding as of June 30, 2018 and December 31, 2017, respectively | 4 | 4 |
| Additional paid-in capital | 497,183 | 471,800 |
| Accumulated deficit | (208,046) | (177,655) |
| Accumulated other comprehensive loss | (1,152) | (1,015) |
| **Total stockholders' equity** | 287,989 | 293,134 |
| **Total liabilities and stockholders' equity** | $ 491,144 | $ 520,346 |

**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, | |
|---|---|---|---|---|
| | **2018** | **2017** | **2018** | **2017** |
| Net product sales: | | | | |
| Bile acid products | $ 18,594 | $ 18,087 | $ 37,102 | $ 33,823 |
| Thiola | 22,743 | 20,713 | 42,667 | 38,597 |
| Total net product sales | 41,337 | 38,800 | 79,769 | 72,420 |
| Operating expenses: | | | | |
| Cost of goods sold | 1,178 | 797 | 2,791 | 1,506 |
| Research and development | 34,460 | 19,482 | 59,096 | 40,342 |
| Selling, general and administrative | 25,100 | 28,835 | 51,568 | 51,950 |
| Change in fair value of contingent consideration | 2,159 | 3,284 | 5,786 | 6,628 |
| Total operating expenses | 62,897 | 52,398 | 119,241 | 100,426 |
| Operating loss | (21,560) | (13,598) | (39,472) | (28,006) |
| Other income (expenses), net: | | | | |
| Other income (loss), net | (403) | 382 | (282) | 508 |
| Interest expense, net | (199) | (658) | (557) | (790) |
| Change in fair value of derivative instruments | — | (1,280) | — | (20) |
| Total other expense, net | (602) | (1,556) | (839) | (302) |
| Loss before provision for income taxes | (22,162) | (15,154) | (40,311) | (28,308) |
| Income tax benefit (expense) | (167) | 1,925 | (396) | 3,989 |
| Net loss | $ (22,329) | $ (13,229) | $ (40,707) | $ (24,319) |
| Net loss per common share: | | | | |
| Basic | $ (0.56) | $ (0.34) | $ (1.03) | $ (0.63) |
| Diluted | $ (0.56) | $ (0.34) | $ (1.03) | $ (0.63) |
| Weighted average common shares outstanding: | | | | |
| Basic | 40,061,045 | 39,041,145 | 39,641,334 | 38,545,982 |
| Diluted | 40,061,045 | 39,041,145 | 39,641,334 | 38,545,982 |

**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, | |
|---|---|---|---|---|
| | **2018** | **2017** | **2018** | **2017** |

| | | | | |
|---|---|---|---|---|
| **GAAP operating loss** | **$ (21,560)** | **$ (13,598)** | **$ (39,472)** | **$ (28,006)** |
| R&D operating expense | (34,460) | (19,482) | (59,096) | (40,342) |
| Stock compensation | 1,582 | 2,427 | 2,989 | 5,115 |
| Amortization & depreciation | 289 | 81 | 392 | 162 |
| Subtotal non-GAAP items | 1,871 | 2,508 | 3,381 | 5,277 |
| Non-GAAP R&D expense | (32,589) | (16,974) | (55,715) | (35,065) |
| SG&A operating expense | (25,100) | (28,835) | (51,568) | (51,950) |
| Stock compensation | 3,844 | 4,812 | 7,046 | 9,217 |
| Amortization & depreciation | 4,354 | 4,356 | 8,599 | 8,559 |
| Subtotal non-GAAP items | 8,198 | 9,168 | 15,645 | 17,776 |
| Non-GAAP SG&A expense | (16,902) | (19,667) | (35,923) | (34,174) |
| Change in valuation of contingent consideration | 2,159 | 3,284 | 5,786 | 6,628 |
| Subtotal non-GAAP items | 12,228 | 14,960 | 24,812 | 29,681 |
| **Non-GAAP operating income (loss)** | **$ (9,332)** | **$ 1,362** | **$ (14,660)** | **$ 1,675** |
| **GAAP net loss** | **$ (22,329)** | **$ (13,229)** | **$ (40,707)** | **$ (24,319)** |
| Non-GAAP operating loss adjustments | 12,228 | 14,960 | 24,812 | 29,681 |
| Change in fair value of derivative instruments | — | 1,280 | — | 20 |
| Income tax benefit (expense) | 167 | (1,925) | 396 | (3,989) |
| **Non-GAAP net income (loss)** | **$ (9,934)** | **$ 1,086** | **$ (15,499)** | **$ 1,393** |
| **Per share data:** | | | | |
| Net earnings (loss) per common share, basic | $ (0.25) | $ 0.03 | $ (0.39) | $ 0.04 |
| Weighted average common shares outstanding, basic | 40,061,045 | 39,041,145 | 39,641,334 | 38,545,982 |

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Source: Retrophin, Inc.