PTC Therapeutics Reports First Quarter 2018 Financial Results and Provides a Corporate Update

May 9, 2018

SOUTH PLAINFIELD, N.J., May 9, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2018.

"Over the past twenty years it has been our mission to bring clinically differentiated therapies to patients with rare disorders," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "As we look forward, we are executing on that mission and consolidating our position as a leading rare disorder biotech company. We intend to continue to leverage our deep scientific expertise and world class commercial capabilities."

First Quarter 2018 Financial Highlights:

- Total revenues for the first quarter of 2018 were $56.1 million compared to $26.5 million in the same period in 2017. The change in total revenue was a result of Emflaza, which launched in May 2017, and the expanded commercial growth of Translarna.
- Translarna net product revenues were $36.8 million for the first quarter of 2018, representing 39% growth over $26.4 million reported in the first quarter of 2017.
- Emflaza net product revenues were $19.2 million for the first quarter of 2018.
- GAAP R&D expenses were $31.4 million for the first quarter of 2018 compared to $27.4 million for the same period in 2017. Non-GAAP R&D expenses were $27.6 million for the first quarter of 2018, excluding $3.7 million in non-cash, stock-based compensation expense, compared to $22.9 million for the same period in 2017, excluding $4.5 million in non-cash, stock-based compensation expense. The increase in R&D expenses for the first quarter of 2018 as compared to the prior year period was primarily due to increased investment in research programs and advancement of the clinical pipeline.
- GAAP SG&A expenses were $33.0 million for the first quarter of 2018 compared to $25.5 million for the same period in 2017. Non-GAAP SG&A expenses were $29.0 million for the first quarter of 2018, excluding $4.0 million in non-cash, stock-based compensation expense, compared to $20.9 million for the same period in 2017, excluding $4.6 million in non-cash, stock-based compensation expense. The increase in SG&A expenses for the first quarter of 2018 as compared to the prior year period was primarily due to the continued commercial support for the Emflaza launch and continued growth of Translarna marketing activities.
- Net interest expense for the first quarter of 2018 was $3.3 million compared to $2.2 million in the same period in 2017. The increase in net interest expense for the first quarter of 2018 as compared to the prior year period was primarily due to increased interest expense related to the $40 million secured loan facility which we closed during the second quarter of 2017 partially offset by interest income from investments.
- Net loss for the first quarter of 2018 was $19.3 million compared to a net loss of $29.1 million for the same period in 2017.
- Cash, cash equivalents, and marketable securities totaled $178.3 million at March 31, 2018 compared to $191.2 million at December 31, 2017.
- Shares issued and outstanding as of March 31, 2018 were 41.8 million.
- In April 2018, PTC completed a public offering of 4,600,000 shares of common stock, resulting in net offering proceeds of $117.9 million.

2018 Guidance

- Full year 2018 net product revenues to be between $260 and $295 million. PTC anticipates Translarna net product revenue for the full year 2018 to be between $170 and $185 million. PTC projects a 5-year (December 31, 2022) compound annual growth rate of 15% for net product revenues representing continued strong growth year-over-year by increasing penetration in current countries and pursuing opportunities for label expansion. PTC anticipates Emflaza net product revenue for the full year 2018 to be between $90 and $110 million.
- GAAP R&D and SG&A expense for the full year 2018 to be between $280 and $290 million.
- Non-GAAP R&D and SG&A expense for the full year 2018 to be between $250 and $260 million, excluding estimated non-cash, stock-based compensation expense of approximately $30 million.

Key 2018 Corporate Highlights:

- **Analyst Day highlighting PTC's 20-year commitment to developing therapeutics for rare disorders.** PTC's management and research teams provided an in-depth update on the Company's commercial products, Translarna and

Emflaza, its scientific platforms including alternative splicing with Spinal muscular atrophy in pivotal trials and two indications, Huntington's disease and Familial dysautonomia which the company anticipates will enter the clinic in 2020. In addition to these indications, PTC also provided updates on our niche oncology pipeline. There are currently two advanced candidates, PTC596 which is currently in the clinic, and PTC299, which PTC anticipates will re-enter the clinic later this year. In 2018, PTC is expecting to initiate clinical trials in two solid tumor indications for PTC596. PTC299, a DHODH inhibitor, is planned to enter clinical trials in hematological tumors in the third quarter of this year. PTC also has a second DHODH inhibitor fast follower currently in late-stage chemical optimization. A replay of the presentations and panel discussions can be found on the Investors page of the website.

- **Encouraging early data from open-label FIREFISH study in Type 1 SMA babies.** Data recently presented at the American Academy of Neurology 2018 Annual Meeting from Part 1 of the FIREFISH study, the dose finding portion, showed that there have been no drug-related safety findings leading to withdrawal at any dose level of the investigational molecule RG7916. In addition, no babies have required a tracheostomy or permanent ventilation since study initiation and no baby has lost the ability to swallow. The median age of first dose was 6.7 months and the 21 babies in the study received RG7916 for a duration of up to 14.8 months. It was reported in January that two babies had died from causes related to disease progression and the deaths were determined not to be drug related. The primary endpoint of Part 2, the confirmatory part of the FIREFISH study, is the proportion of patients sitting without support after 12 months on RG7916 treatment. Recruitment is ongoing globally for FIREFISH Part 2. The SMA program is a collaboration between PTC, Roche, and the SMA Foundation.
- **Review of the Translarna Pediatric Label Expansion by the Committee for Medicinal Products for Human Use (CHMP) in progress.** The application to expand the label of Translarna for the treatment of nonsense mutation DMD patients who are 2-5 years is currently under review by the CHMP in Europe and a decision is expected mid-year.
- **Dystrophin study for US NDA for ataluren (Translarna) in DMD to begin by the end of 2018.** The Office of New Drugs recommended a possible path forward for the ataluren NDA submission based on the accelerated approval pathway. This would involve a re-submission of an NDA containing the current data on effectiveness of ataluren with new data to be generated on dystrophin production. PTC is working to design such a study and expects to initiate such a study by the end of 2018.
- **Successful closing of public offering.** PTC offered 4,600,000 shares of common stock, successfully raising $117.9 million in the public market. PTC intends to use the net proceeds from this offering to fund its research and development efforts, including clinical trials and studies with respect to its products and product candidates and potential additional indications, including its programs for alternative splicing for the treatment of rare disorders and oncology, commercialization activities for Translarna for the treatment of nmDMD outside of the United States and Emflaza for the treatment of DMD in the United States and for working capital and other general corporate purposes.

Non-GAAP Financial Measures:
In this press release, the financial results and financial guidance of PTC are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, the non-GAAP financial measures exclude stock-based compensation expense. This non-GAAP financial measure is provided as a complement to financial measures reported in GAAP because management uses this non-GAAP financial measure when assessing and identifying operational trends. In management's opinion, this non-GAAP financial measure is useful to investors and other users of PTC's financial statements by providing greater transparency into the historical and projected operating performance of PTC and the company's future outlook. Quantitative reconciliations of non-GAAP financial measures to their closest equivalent GAAP financial measures are included in the tables below.

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 4:30 pm ET and can be access by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 3757748. A live, listen-only webcast of the conference call can be accessed on the Investor Relations section of the PTC website at www.ptcbio.com. A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for two weeks.

About PTC Therapeutics, Inc.
PTC is a science-led, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward Looking Statements:

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including the information provided under the heading "2018 Guidance", including with respect to (i) 2018 net product revenue and net sales guidance for Translarna and Emflaza and (ii) 2018 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; the timing of and likelihood of success of its regulatory path forward in the U.S., including as it relates to any clinical trials and non-clinical studies to generate data on dystrophin production in ataluren, a re-submission of an NDA for ataluren to the FDA, and any further interactions between PTC and the FDA; expansion of Translarna; advancement of PTC's joint collaboration program in SMA; PTC's strategy, future operations, future financial position, future revenues, projected costs; or intended use of proceeds from its public offering of common stock; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for Emflaza and Translarna; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its NDA for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), and PTC's ability to perform additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to realize the anticipated benefits of the acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of Emflaza, as well as other business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of Translarna, Emflaza and PTC's other product candidates; the enrollment, conduct, and results of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna or Emflaza.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

PTC Therapeutics, Inc.

Consolidated Statements of Operations
(In thousands, except per share data)

	Three Months Ended March 31,	
	2018	**2017**
Revenues:		
Net product revenue	$ 55,981	$ 26,442
Collaboration and grant revenue	81	105
Total revenues	56,062	26,547
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible asset	3,045	39
Amortization of acquired intangible asset	5,428	—
Research and development (1)	31,363	27,363
Selling, general and administrative (2)	32,969	25,500
Total operating expenses	72,805	52,902
Loss from operations	(16,743)	(26,355)
Interest expense, net	(3,303)	(2,219)
Other income (expense), net	1,004	(318)

Loss before income tax expense	(19,042)	(28,892)
Income tax expense	(221)	(165)
Net loss attributable to common stockholders	$ (19,263)	$ (29,057)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	41,626,617	34,305,948
Net loss per share—basic and diluted (in dollars per share)	$ (0.46)	$ (0.85)
(1) Research and development expense reconciliation		
GAAP research and development	$ 31,363	$ 27,363
Less: share-based compensation	3,747	4,467
Non-GAAP research and development expense	$ 27,616	$ 22,896
(2) Selling, general and administrative expense reconciliation		
GAAP selling, general and administrative	$ 32,969	$ 25,500
Less: share-based compensation	4,001	4,562
Non-GAAP selling, general and administrative expense	$ 28,968	$ 20,938

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets
(In thousands, except per share data)

	March 31, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 178,270	$ 191,246
Total assets	**$ 379,411**	**$ 391,653**
Total debt	$ 146,878	$ 144,971
Total deferred revenue	9,300	11,891
Total liabilities	**$ 229,103**	**$ 235,216**
Total stockholders' equity (41,809,398 and 41,612,395 common shares issued and outstanding at March 31, 2018 and December 31, 2017, respectively)	150,308	156,437
Total liabilities and stockholders' equity	**$ 379,411**	**$ 391,653**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2018 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 280,000	$ 290,000
Less: projected shared-based compensation expense	30,000	30,000
Total projected non-GAAP R&D and SG&A expense	**$ 250,000**	**$ 260,000**

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