May 8, 2017

PTC Therapeutics Reports First Quarter 2017 Financial Results and Provides Corporate Update

- Translarna™ first quarter net sales of $26.4M representing 40% year-over-year growth -
- Full-year 2017 Translarna net sales guidance increased to $115 to $130M -
- EMFLAZATM for the treatment of Duchenne muscular dystrophy planned to launch in the coming weeks -

SOUTH PLAINFIELD, N.J., May 8, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2017.

"We are thrilled to bring EMFLAZA to Duchenne muscular dystrophy patients in the United States," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "The acquisition of EMFLAZA supports our commitment to providing new treatment options for Duchenne patients. We plan to launch EMFLAZA in the coming weeks and are focused on providing access to all eligible patients. We are also pleased with Translarna's continued sales growth outside the U.S. with another strong quarter. With Translarna and EMFLAZA, we now have two of the three approved Duchenne drugs globally."

First Quarter Financial Highlights:

- Translarna net product sales were $26.4 million for the first quarter of 2017, representing 40% growth over $18.9 million reported in the first quarter of 2016.
- Total revenues for the first quarter of 2017 were $26.5 million compared to $18.9 million in the same period of 2016. The change in total revenue was a result of the expanded commercial launch of Translarna.
- GAAP R&D expenses were $27.4 million for the first quarter of 2017 compared to $31.4 million for the same period in 2016. Non-GAAP R&D expenses were $22.9 million for the first quarter of 2017, excluding $4.5 million in non-cash, stock-based compensation expense, compared to $26.4 million for the same period in 2016, excluding $4.3 million in non-cash, stock-based compensation expense and one-time restructuring costs of $0.7 million. The decrease in R&D expense for the first quarter of 2017 as compared to the prior year period was primarily due to the completion of our ACT CF study at the end of 2016 and the ongoing reduction of clinical trial expenses.
- GAAP SG&A expenses were $25.5 million for the first quarter of 2017 compared to $25.9 million for the same period in 2016. Non-GAAP SG&A expenses were $20.9 million for the first quarter of 2017, excluding $4.6 million in non-cash, stock-based compensation expense, compared to $20.2 million for the same period in 2016, excluding $4.6 million in non-cash, stock-based compensation expense and one-time restructuring costs of $1.2 million. SG&A expenses were relatively flat for the first quarter of 2017 as compared to the same period in 2016.
- Net interest expense for the first quarter of 2017 was $2.2 million compared to net interest expense of $2.0 million in the same period in 2016. The increase in net interest expense is primarily a result of reduced interest income from investments.
- Net loss for the first quarter of 2017 was $29.1 million compared to a net loss of $41.2 million for the same period in 2016.
- Cash, cash equivalents, and marketable securities totaled approximately $202.6 million at March 31, 2017 compared to approximately $231.7 million at December 31, 2016.
- Shares issued and outstanding as of March 31, 2017 were 34.6 million, which includes 0.3 million shares of unvested restricted stock.

2017 Guidance:

- Translarna net sales for 2017 are now anticipated to be between $115 and $130 million, an increase from prior guidance of $105 to $125 million. This guidance assumes the current exchange rates and the continued commercial expansion for Translarna in nmDMD outside of the U.S. Taking into account the time to achieve reimbursement and other launch-related factors, PTC anticipates EMFLAZA net sales for 2017 to be between $5 and $10 million. PTC also anticipates a potential $20 million milestone payment in 2017 related to the SMA program for total 2017 revenues between $120 and $160 million.
- GAAP operating expenses for full year 2017 are anticipated to be between $250 to $260 million. Excluding estimated non-cash, stock-based compensation expense of approximately $40 million, full year 2017 non-GAAP operating

expenses are anticipated to be between $210 and $220 million. These operating expenses are expected to be primarily in support of the commercial availability of Translarna outside of the U.S. and in support of the pending commercial launch of EMFLAZA in the U.S., as well as the continued research and clinical development of other product pipeline candidates.

- PTC recently closed on a $60 million senior secured term loan facility with MidCap Financial, of which $40 million was drawn at close. As a result of the $75 million in cash utilized in the EMFLAZA acquisition, partially offset by the $40 million draw-down of term-loan financing from MidCap Financial, PTC now expects to end 2017 with cash and cash equivalents of approximately $100 million.

Key First Quarter and other Corporate Highlights:

- **Commercial launch of EMFLAZA for the treatment of Duchenne muscular dystrophy.** PTC is preparing for the commercial launch of EMFLAZA in the U.S., which is expected to begin in the coming weeks. PTC has established programs with the goal of ensuring that eligible patients will have access to EMFLAZA regardless of financial or insurance status. Over 900 patients, about 10% of the 9,000 eligible Duchenne patients in the U.S., have already been prescribed EMFLAZA and submitted START forms.
- **Review of the Translarna NDA for nonsense mutation Duchenne muscular dystrophy by the FDA in progress.** The FDA acknowledged PTC's filing over protest of the New Drug Application (NDA) for Translarna for the treatment of nmDMD and assigned the company a Prescription Drug User Fee Act (PDUFA) target date of October 24, 2017.
- **Two SMA clinical trials on track to advance into pivotal studies in 2017.** The spinal muscular atrophy (SMA) program, a joint collaboration with Roche and the SMA Foundation, is expected to advance into two pivotal studies in 2017. The SUNFISH study in Type 2/3 patients and the FIREFISH study in Type 1 patients are both enrolling the initial dose escalation part of the respective studies. Initiation of the pivotal second part of both studies is expected in the second half of 2017 and initiation of the first pivotal study will trigger a single $20 million milestone payment to PTC from Roche. Data from the first part of the studies is expected to be presented at scientific meetings later this year and early next year.

Non-GAAP Financial Measures:
In this press release, PTC's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial measures exclude stock-based compensation expense and one-time restructuring expenses relating to the reorganization of operations intended to improve efficiency and better align costs and employment structure with PTC's strategic plans. These non-GAAP financial measures are provided as a complement to results reported in GAAP because management uses these non-GAAP financial measures when assessing and identifying operational trends. In management's opinion, these non-GAAP financial measures are useful to investors and other users of PTC's financial statements by providing greater transparency into the operating performance at PTC and the company's future outlook. Quantitative reconciliations of GAAP financial measures are included in the tables below.

PTC Therapeutics, Inc.
Consolidated Statements of Operations
(In thousands, except per share data)

	Three Months Ended March 31,	
	2017	**2016**
Revenues:		
Net product revenue	$ 26,442	$ 18,878
Collaboration and grant revenue	105	17
Total revenues	26,547	18,895
Operating expenses:		
Cost of product sales	39	—
Research and development (1)	27,363	31,399
Selling, general and administrative (2)	25,500	25,938
Total operating expenses	52,902	57,337
Loss from operations	(26,355)	(38,442)
Interest expense, net	(2,219)	(1,956)
Other expense, net	(318)	(721)
Loss before income tax expense	(28,892)	(41,119)
Income tax expense	(165)	(114)

Net loss attributable to common stockholders	$ (29,057)	$ (41,233)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	34,305,948	33,919,169
Net loss per share—basic and diluted (in dollars per share)	$ (0.85)	$ (1.22)
(1) Research and development expense reconciliation		
GAAP research and development	$ 27,363	$ 31,399
Less: share-based compensation	4,467	4,328
Less: one-time restructuring cost	—	716
Non-GAAP research and development expense	$ 22,896	$ 26,355
(2) Selling, general and administrative expense reconciliation		
GAAP selling, general and administrative	$ 25,500	$ 25,938
Less: share-based compensation	4,562	4,587
Less: one-time restructuring cost	—	1,187
Non-GAAP selling, general and administrative expense	$ 20,938	$ 20,164

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

	March 31, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	$ 202,577	$ 231,666
Total assets	**$ 248,645**	**$ 269,345**
Total debt	$ 99,895	$ 98,216
Total deferred revenue	2,194	1,587
Total liabilities	**$ 147,818**	**$ 149,762**
Total stockholders' equity (34,316,836 and 34,169,410 common shares issued and outstanding at March 31, 2017 and December 31, 2016, respectively)	100,827	119,583
Total liabilities and stockholders' equity	**$ 248,645**	**$ 269,345**

Today's Conference Call and Webcast Reminder

Today's conference call will take place at 8:30 AM (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 11527010. 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 global biopharmaceutical company focused on the discovery, development, and commercialization of novel medicines using our expertise in RNA biology. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. PTC has discovered all of its compounds currently under development using its proprietary technologies. Since its founding nearly 20 years ago, PTC's mission has focused on developing treatments to fundamentally change the lives of patients living with rare genetic disorders. The company was founded in 1998 and is headquartered in South Plainfield, New Jersey. For more information on the company, please visit our website www.ptcbio.com.

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About MidCap Financial

MidCap Financial is a middle market-focused, specialty finance firm that provides senior debt solutions to businesses across all industries. Its debt solutions include general and healthcare asset-based working capital loans, leveraged loans to private equity-backed companies, life sciences loans to VC-backed and public companies, commercial real estate loans, and lender finance loans.

Additional information about MidCap Financial can be found at www.midcapfinancial.com

Forward Looking Statements:

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release are forward-looking statements, including the information provided under the heading "2017 Guidance" and statements regarding: the future expectations, plans and prospects for PTC; timing of the pending commercial launch in the U.S. of EMFLAZA for the treatment of DMD; the size of the DMD patient population eligible for EMFLAZA treatment in the U.S.; the PDUFA date for the NDA; advancement of PTC's joint collaboration program in SMA, including whether and when Sunfish or Firefish may transition into the pivotal part of the applicable study and whether and when a milestone payment to PTC from Roche may be triggered; PTC's strategy, future operations, future financial position, future revenues or projected costs; 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: PTC's preparations for a commercial launch of EMFLAZA in the U.S., including its ability to finalize in a timely manner regulatory, distribution and commercial matters that must be concluded prior to a launch by PTC; 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 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 resolve the matters set forth in the Refuse to File letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD, including whether PTC's filing of the NDA over protest with the FDA will result in a timely or successful review of the NDA, and whether PTC will be required to perform additional clinical and non-clinical trials or analyses at significant cost, which, if successful, could potentially support the approval of the NDA filed over protest or a new NDA submission; 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; the eligible patient base and commercial potential of Translarna, EMFLAZA and PTC's other product candidates; the enrollment and conduct of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development of 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 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.

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