August 8, 2017

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

- Translarna™ second quarter sales of $45.8 M representing 197% growth over 2Q2016 -
- Over 1,200 patients on EMFLAZA™ 12 weeks into launch -
- ~400% dose-dependent increase in full length SMN2 mRNA shown in SUNFISH trial interim analysis -
- 2017 revenue guidance increased to $155-180 M -

SOUTH PLAINFIELD, N.J., Aug. 8, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2017.

"Since our founding nearly 20 years ago, it has been our mission to provide treatments to patients living with rare diseases who have limited treatment options," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "I am proud to have introduced Translarna, the first therapy for nonsense mutation Duchenne muscular dystrophy patients and we remain dedicated to working with patients and their families to bring Translarna to the U.S. We are also pleased with the successful early launch of EMFLAZA. Consistent with our mission, we are working to ensure broad access to EMFLAZA to all eligible Duchenne patients regardless of financial or insurance status."

Second Quarter Financial Highlights:

- Translarna net product sales were $45.8 million for the second quarter of 2017, representing 197% growth over $15.4 million reported in the second quarter of 2016.
- EMFLAZA net product sales were $2.1 million for the second quarter of 2017.
- Total revenues for the second quarter of 2017 were $48.0 million compared to $15.6 million in the same period of 2016. The change in total revenue was a result of the expanded commercial launch of Translarna and the successful U.S. EMFLAZA launch.
- GAAP R&D expenses were $30.8 million for the second quarter of 2017 compared to $28.8 million for the same period in 2016. Non-GAAP R&D expenses were $26.9 million for the second quarter of 2017, excluding $3.9 million in non-cash, stock-based compensation expense, compared to $24.6 million for the same period in 2016, excluding $4.1 million in non-cash, stock-based compensation expense and one-time restructuring costs of $0.1 million. The increase in R&D expense for the second quarter of 2017 as compared to the prior year period was primarily due to start-up of clinical activities and regulatory spend, partially offset by the decreased costs due to the completion of our CF program at the end of 2016.
- GAAP SG&A expenses were $28.9 million for the second quarter of 2017 compared to $23.4 million for the same period in 2016. Non-GAAP SG&A expenses were $24.9 million for the second quarter of 2017, excluding $4.0 million in non-cash, stock-based compensation expense, compared to $18.3 million for the same period in 2016, excluding $4.6 million in non-cash, stock-based compensation expense and one-time restructuring costs of $0.4 million. The increase in SG&A expenses primarily related to the expansion of the U.S. commercial sales team in support of the launch of EMFLAZA.
- Net interest expense for the second quarter of 2017 was $3.0 million compared to net interest expense of $2.1 million in the same period in 2016. The increase in net interest expense is primarily a result of increased interest expense related to the $40 million secured loan facility which we closed during the quarter partially offset by reduced interest income from investments.
- Net loss for the second quarter of 2017 was $17.5 million compared to a net loss of $38.9 million for the same period in 2016.
- Cash, cash equivalents, and marketable securities totaled approximately $181.1 million at June 30, 2017 compared to approximately $231.7 million at December 31, 2016.
- Shares issued and outstanding as of June 30, 2017, were 41.3 million, which includes 0.1 million shares of unvested restricted stock awards.

2017 Guidance:

- Translarna net sales for 2017 are now anticipated to be between $120 and $140 million, an increase from prior guidance of $115 to $130 million. PTC anticipates EMFLAZA net sales for 2017 to be between $15 and $20 million.

PTC also anticipates a $20 million milestone payment in 2017 related to the SMA program for total 2017 revenues between $155 and $180 million.

- GAAP operating expenses for the 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 million and $220 million. These expenses will be primarily in support of the commercial availability of Translarna globally, the commercial launch of EMFLAZA in the U.S. and the continued research and clinical development of other product pipeline candidates.
- PTC expects to end 2017 with over $120 million of cash and cash equivalents.

Key Second Quarter and Other Corporate Highlights:

- **EMFLAZA™ for the treatment of Duchenne muscular dystrophy successfully launched in the U.S. with establishment of EMFLAZACares Program.** PTC has successfully launched EMFLAZA in the U.S. with over 1,200 patients receiving therapy only 12 weeks into the launch. We estimate that there are 9,000 Duchenne patients in the U.S. over the age of five. EMFLAZACares is a program designed to enable all eligible patients to have access to EMFLAZA regardless of financial or insurance status. Based on progress to date, we are raising our 2017 guidance to $15-20M from $5-10M.

- **Translarna™ revenue of $45.8 M in second quarter, which represents a 197% growth over 2Q2016.** PTC continues to expand on its strong global footprint in Duchenne muscular dystrophy, with sales generated in over 25 countries. Market access discussions regarding funding on a country-by-country basis are ongoing. This strong performance reflects continued uptake, sustainable pricing levels, and high (> 90%) compliance to treatment.

- **NDA for Translarna under FDA review with PDUFA date of October 24, 2017.** The FDA has assigned the New Drug Application (NDA) for ataluren (Translarna™) a Prescription Drug User Fee Act (PDUFA) date of October 24, 2017. The company is preparing for the Advisory Committee Meeting, which is tentatively scheduled for September 28, 2017.

- **SMA clinical program on track to advance to a pivotal phase in the second half of 2017.** The spinal muscular atrophy (SMA) program, a joint collaboration with Roche and the SMA Foundation, is expected to advance into pivotal studies in the second half of 2017. Commencement of the pivotal portion of either study will trigger a $20 million milestone payment to PTC from Roche. Preliminary data from the first cohort of the SUNFISH trial was presented at the CureSMA Conference and demonstrated a dose dependent increase up to 400% in SMN2 transcript. In addition, no toxicities requiring patients' withdrawal had been observed in the clinic to date.

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, 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)
Unaudited

	Three Months Ended June 30,		Six Months Ended June 30,	
	2017	**2016**	**2017**	**2016**
Revenues:				
Net product revenue	$ 47,891	$ 15,437	$ 74,334	$ 34,314
Collaboration and grant revenue	71	196	176	214
Total revenues	47,962	15,633	74,510	34,528
Operating expenses:				
Cost of product sales	758	—	797	—
Research and development (1)	30,835	28,827	58,198	60,226

Selling, general and administrative (2)	28,866	23,366	54,365	49,304
Total operating expenses	60,459	52,193	113,360	109,530
Loss from operations	(12,497)	(36,560)	(38,850)	(75,002)
Interest expense, net	(3,008)	(2,060)	(5,227)	(4,016)
Other expense, net	(1,820)	(387)	(2,139)	(1,107)
Loss before income tax expense	(17,325)	(39,007)	(46,216)	(80,125)
Income tax (expense) benefit	(150)	93	(316)	(22)
Net loss attributable to common stockholders	$ (17,475)	$ (38,914)	$ (46,532)	$ (80,147)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	39,621,738	34,000,333	36,978,528	33,959,751
Net loss per share—basic and diluted (in dollars per share)	$ (0.44)	$ (1.14)	$ (1.26)	$ (2.36)
(1) Research and development reconciliation				
GAAP research and development	$ 30,835	$ 28,827	$ 58,198	$ 60,226
Less: share-based compensation expense	3,895	4,087	8,362	8,415
Less: one-time restructuring cost	—	118	—	834
Non-GAAP research and development	$ 26,940	$ 24,622	$ 49,836	$ 50,977
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 28,866	$ 23,366	$ 54,365	$ 49,304
Less: share-based compensation expense	3,990	4,649	8,552	9,236
Less: one-time restructuring cost	—	430	—	1,617
Non-GAAP selling, general and administrative	$ 24,876	$ 18,287	$ 45,813	$ 38,451

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

	June 30, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	$ 181,069	$ 231,666
Total assets	**$ 383,078**	**$ 269,345**
Total debt	$ 141,242	$ 98,216
Total deferred revenue	6,430	1,587
Total liabilities	**$ 213,347**	**$ 149,762**
Total stockholders' equity (41,304,008 and 34,169,410 common shares issued and outstanding at June 30, 2017 and December 31, 2016, respectively)	169,731	119,583
Total liabilities and stockholders' equity	**$ 383,078**	**$ 269,345**

Upcoming Events:
PTC management will present a company update at the upcoming Citi 12th Annual Biotech Conference on Thursday, September 7th. The presentation will be webcast live on the Events and Presentations page under the Investor Relations section of PTC Therapeutics website at www.ptcbio.com and will be archived for 2 weeks following the presentation. It is recommended that users connect to PTC's website several minutes prior to the start of the webcast to ensure a timely connection. PTC's current Investor Presentation is available at the same website location.

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 4:30 PM ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 58898313. 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
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.

For More Information:

Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.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; the size of the DMD patient population eligible for EMFLAZA treatment in the U.S.; the PDUFA date and FDA advisory committee meeting date for the Translarna 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 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; the recommendation the advisory committee provides to the FDA for Translarna for the treatment of nmDMD; 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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