November 2, 2017

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

- Total third quarter revenue of $41.9M representing 82% growth over 3Q2016 -
- Increasing 2017 revenue guidance to $160-$185M -

SOUTH PLAINFIELD, N.J., Nov. 2, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2017.

"Our performance this quarter combined with our commercial, financial and R&D advancements should allow us to end 2017 in a strong position," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Our commercial success is driven by our mission of improving the lives of patients with Duchenne."

Third Quarter Financial Highlights:

- Translarna™ net product sales were $32.0 million for the third quarter of 2017, representing 45% growth over $22.0 million reported in the third quarter of 2016.
- EMFLAZA™ net product sales were $9.8 million for the third quarter of 2017.
- Total revenues for the third quarter of 2017 were $41.9 million compared to $23.0 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 $30.0 million for the third quarter of 2017 compared to $31.4 million for the same period in 2016. Non-GAAP R&D expenses were $26.4 million for the third quarter of 2017, excluding $3.6 million in non-cash, stock-based compensation expense, compared to $27.1 million for the same period in 2016, excluding $4.3 million in non-cash, stock-based compensation expense. The decrease in R&D expense for the third quarter of 2017 as compared to the prior year period was primarily due to the completion of our Phase 3 Translarna trials at the end of 2016 partially offset by start-up clinical activities and regulatory spend.
- GAAP SG&A expenses were $31.4 million for the third quarter of 2017 compared to $23.7 million for the same period in 2016. Non-GAAP SG&A expenses were $27.9 million for the third quarter of 2017, excluding $3.5 million in non-cash, stock-based compensation expense, compared to $19.0 million for the same period in 2016, excluding $4.6 million in non-cash, stock-based compensation expense. 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 third quarter of 2017 was $3.4 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 second quarter of 2017 partially offset by reduced interest income from investments.
- Net loss for the third quarter of 2017 was $33.7 million compared to a net loss of $35.2 million for the same period in 2016.
- Cash, cash equivalents, and marketable securities totaled approximately $169.3 million at September 30, 2017 compared to approximately $231.7 million at December 31, 2016.
- Shares issued and outstanding as of September 30, 2017, were 41.5 million, which includes 0.1 million shares of unvested restricted stock awards.

2017 Guidance:

- Translarna net sales guidance for 2017 is anticipated to be between $120 and $140 million. We now anticipate EMFLAZA net sales for 2017 to be between $20 and $25 million, an increase from our prior guidance of $15 to $20 million. This brings 2017 full year revenue guidance between $160 and $185 million, an increase from our prior guidance of $155 million to $180 million, including a $20 million milestone we achieved in mid-October, under our SMA program.
- GAAP R&D and SG&A expense for the full year 2017 are now 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 R&D and SG&A expense are now 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.

- We now expect to end 2017 with over $150 million of cash and cash equivalents, an increase from prior guidance of $120 million.

Key Third Quarter and other Corporate Highlights:

- **Filed Formal Dispute Resolution Request with U.S. FDA to appeal Complete Response Letter for ataluren.** PTC received a Complete Response Letter from the Office of Drug Evaluation I of the U.S. Food and Drug Administration (FDA) for the New Drug Application (NDA) of the investigational medicine ataluren for the treatment of nonsense mutation dystrophinopathies. The letter stated that it is unable to approve the application in its current form. PTC has filed a formal dispute resolution request challenging this decision.

- **EMFLAZA revenue grew to $9.8M in third quarter.** PTC is committed to enabling access to EMFLAZA for all patients in need, regardless of financial situation or insurance status. We demonstrated an increase in EMFLAZA prescriptions over the past quarter. There are currently over 1,500 patients on EMFLAZA and we estimate that there are approximately 9,000 Duchenne patients in the U.S. over the age of five who are eligible to be prescribed EMFLAZA.

- **Continued global expansion of Translarna results in revenue of $32.0M in third quarter.** PTC continues to expand its strong global footprint, with sales generated in over 25 countries. This strong performance reflects continued uptake, sustainable pricing levels, and high (> 90%) compliance to treatment.

- **SMA clinical program advanced into the pivotal portion of the study.** In mid-October, the SUNFISH trial transitioned into the pivotal portion of the study which triggered a $20M milestone to PTC from Roche. Data from the SUNFISH trial was presented at the International Congress of the World Muscle Society. An interim analysis of the five cohorts treated with RG7916 for 28 days demonstrates an exposure-dependent increase in SMN protein.

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)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Revenues:				
Net product revenue	$ 41,780	$ 22,013	$ 116,113	$ 56,328
Collaboration and grant revenue	73	973	249	1,186
Total revenues	41,853	22,986	116,362	57,514
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible asset	1,582	—	2,142	—
Amortization of acquired intangible asset	9,716	—	9,952	—
Research and development (1)	30,024	31,396	88,222	91,622
Selling, general and administrative (2)	31,423	23,654	85,788	72,958
Total operating expenses	72,745	55,050	186,104	164,580
Loss from operations	(30,892)	(32,064)	(69,742)	(107,066)
Interest expense, net	(3,421)	(2,133)	(8,648)	(6,149)
Other income (expense), net	766	(786)	(1,373)	(1,893)
Loss before income tax expense	(33,547)	(34,983)	(79,763)	(115,108)
Income tax expense	(191)	(184)	(507)	(206)
Net loss attributable to common stockholders	$ (33,738)	$ (35,167)	$ (80,270)	$ (115,314)

Weighted-average shares outstanding:				
Basic and diluted (in shares)	41,296,740	34,088,741	38,433,749	34,002,952
Net loss per share—basic and diluted (in dollars per share)	$ (0.82)	$ (1.03)	$ (2.09)	$ (3.39)
(1) Research and development reconciliation				
GAAP research and development	$ 30,024	$ 31,396	$ 88,222	$ 91,622
Less: share-based compensation expense	3,624	4,319	11,986	12,734
Less: one-time restructuring cost	—	5	—	845
Non-GAAP research and development	$ 26,400	$ 27,072	$ 76,236	$ 78,043
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 31,423	$ 23,654	$ 85,788	$ 72,958
Less: share-based compensation expense	3,544	4,640	12,096	13,876
Less: one-time restructuring cost	—	28	—	1,661
Non-GAAP selling, general and administrative	$ 27,879	$ 18,986	$ 73,692	$ 57,421

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

	September 30, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	$ 169,310	$ 231,666
Total assets	**$ 367,720**	**$ 269,345**
Total debt	$ 143,091	$ 98,216
Total deferred revenue	12,701	1,587
Total liabilities	**$ 221,838**	**$ 149,762**
Total stockholders' equity (41,463,121 and 34,169,410 common shares issued and outstanding at September 30, 2017 and December 31, 2016, respectively)	145,882	119,583
Total liabilities and stockholders' equity	**$ 367,720**	**$ 269,345**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Full Year 2017 R&D and SG&A Expense
(In thousands, except per share data)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	250,000	260,000
Less: projected shared-based compensation expense	40,000	40,000
Total projected non-GAAP R&D and SG&A expense	**$ 210,000**	**$ 220,000**

Today's Conference Call and Webcast Reminder

The call 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 2261347. 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.

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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 are forward-looking statements, including the information provided under the heading "2017 Guidance" and statements regarding: the future expectations, plans and prospects for PTC; PTC's plans for further interactions with the FDA regarding the Translarna NDA; the outcome of any formal dispute resolution request filed with the FDA; the size of the DMD patient population eligible for EMFLAZA treatment in the U.S.; expansion of Translarna globally; advancement of PTC's joint collaboration program in SMA; 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 Complete Response letter it received from the FDA in connection with its NDA for Translarna for the treatment of nmDMD either via outcome of any formal dispute resolution request or other interactions with the FDA, 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; 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 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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