March 16, 2017

PTC Therapeutics Reports Fourth Quarter and Full Year 2016 Financial Results and Provides Corporate Update

- $81.4M in Translarna net sales, a 142% increase vs. 2015 -
- October 24th PDUFA target date for Translarna nmDMD NDA -
- SMA program actively enrolling patients across SUNFISH and FIREFISH trials -
- Translarna 2017 net sales guidance of $105M to $125M -

SOUTH PLAINFIELD, N.J., March 16, 2017 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ending December 31, 2016.

"For nearly 20 years, PTC has been committed to delivering new treatment options to patients living with Duchenne muscular dystrophy globally," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Our strong commercial performance in 2016 coupled with the advancements in our clinical programs brings us closer to that goal. I am proud of what we accomplished in 2016, and we will continue to work diligently to bring Translarna to patients globally, as well as develop treatments for additional rare, genetic disorders."

Fourth Quarter and Full Year 2016 Financial Highlights:

- Translarna net product sales were $25.1 million for the fourth quarter of 2016, representing 98% growth versus $12.7 million in the fourth quarter of 2015. For the full year 2016, Translarna generated $81.4 million in net product sales representing 142% growth compared to $33.7 million in the prior year.
- Total revenues for the fourth quarter of 2016 were $25.2 million versus $12.7 million in the same period of 2015. Total revenues for 2016 were $82.7 million compared to $36.8 million for the same period of 2015. The change in total revenue was primarily due to growing Translarna net product sales, partially offset by lower grant revenue.
- GAAP R&D expenses were $26.0 million for the fourth quarter of 2016 compared to $35.0 million for the fourth quarter of 2015. For the full year 2016, GAAP R&D expenses were $117.6 million compared to $121.8 million in the prior year period. The decrease in R&D expense for the fourth quarter and year ended December 31, 2016, as compared to the prior year periods was primarily due to lower costs associated with research and clinical development activities, partially offset by increased costs related to the manufacture of drug product.
- Non-GAAP R&D expenses were $21.9 million for the fourth quarter of 2016, excluding $4.1 million in non-cash, stock-based compensation expense, compared to $31.4 million for the fourth quarter of 2015, excluding $3.7 million in non-cash, stock-based compensation expense. For the full year 2016, non-GAAP R&D expenses were $100.0 million, excluding $16.8 million in non-cash, stock-based compensation expense and $0.8 million in one-time restructuring expense, compared to $105.7 million for 2015, excluding $16.1 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $24.2 million for the fourth quarter of 2016 compared to $25.9 million for the fourth quarter of 2015. For the full year 2016, GAAP SG&A expenses were $97.1 million compared to $82.1 million in 2015. The increase in SG&A expense for the fourth quarter and year ended December 31, 2016, as compared to the prior year periods, primarily resulted from additional costs associated with commercial activities in support of Translarna across Europe and other regions.
- Non-GAAP SG&A expenses were $19.9 million for the fourth quarter of 2016, excluding $4.3 million in non-cash, stock-based compensation expense, compared to $21.7 million for the fourth quarter of 2015, excluding $4.2 million in non-cash, stock-based compensation expense. Full-year 2016 non-GAAP SG&A expenses were $77.3 million, excluding $18.2 million in non-cash, stock-based compensation expense and $1.6 million in one-time restructuring expense, compared to $64.2 million for 2015, excluding $17.8 million in non-cash, stock-based compensation expense.
- Net interest expense for the fourth quarter of 2016 was $2.1 million compared to net interest expense of $2.5 million in the same period in 2015. The decrease in interest expense is primarily a result of increased interest income related to investments, which partially offset interest expense related to the $150 million convertible debt offering completed during mid-third quarter 2015. The debt was recorded on PTC's balance sheet at a discount, which will be amortized over the life of the bond. For the full year 2016, net interest expense was $8.3 million, compared to $2.4 million for 2015. The increase is primarily due to interest expense accrued in connection with the semi-annual interest payments due on the notes from the convertible debt offering beginning in 2016 for the full year as compared to partial year expense in 2015 partially offset by interest income related to investments.
- Net loss for the fourth quarter of 2016 was $26.8 million compared to a net loss of $50.9 million for the same period in

2015. Net loss for the full year 2016 was $142.1 million compared to $170.4 million for the same period in 2015.
- Cash, cash equivalents, and marketable securities totaled approximately $231.7 million at December 31, 2016 compared to approximately $338.9 million at December 31, 2015.
- Shares issued and outstanding as of December 31, 2016 were 34.3 million, which includes 0.2 million shares of unvested restricted stock.

2017 Guidance:

- For 2017, PTC expects to achieve ex-U.S. Translarna net sales between $105 and $125 million, assuming current exchange rates, representing continued strong growth year-over-year of its sustainable DMD business. This is driven by both increased penetration into the over 25 countries where Translarna is currently available as well as continued geographic expansion into new territories.
- PTC is reviewing its guidance for 2017 operating expenses and ending cash in light of PTC's planned acquisition of Emflaza™ (deflazacort)

Key 2016 Fourth Quarter and other Corporate Highlights:

- **Entry into Asset Purchase Agreement to acquire Emflaza™ (deflazacort).** PTC announced today that it entered into an asset purchase agreement with Marathon Pharmaceuticals, LLC, under which PTC plans to acquire all rights to Emflaza, subject to satisfaction of customary closing conditions. Under the terms of the agreement, PTC will make an upfront payment of $140 million to Marathon, comprising of a combination of cash and stock. Following completion of a transition period, Marathon is entitled to receive payments from PTC based on annual net sales of Emflaza beginning in 2018, which PTC expects will range as a percentage of net sales between the low to mid-20s on a blended average basis. In addition, Marathon has the opportunity to receive a single $50 million sales-based milestone.
- **Successful second year of Translarna sales with 2016 revenues of $81.4M, an increase of 142% over the prior year and achieving the upper-end of guidance.** PTC has expanded on its strong global footprint in Duchenne muscular dystrophy (DMD), with sales now generated in over 25 countries. Market access discussions regarding funding on a country-by-country basis are ongoing. This strong performance reflects rapid uptake, sustainable pricing, and an estimated high (> 90%) compliance to treatment.
- **Filing of New Drug Application for Translarna for the Treatment of Nonsense Mutation Duchenne Muscular Dystrophy Acknowledged.** The FDA has granted standard review and assigned a Prescription Drug User Fee Act (PDUFA) date of October 24, 2017. The PDUFA date is the target date for the FDA to complete its review of the NDA. PTC used the FDA's file over protest regulations to file the NDA, which allowed PTC to have its NDA filed and reviewed following receipt of the FDA's refuse to file determination in February 2016.
- **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. SUNFISH and FIREFISH are both two part studies in childhood onset (Type 2/3) and infant onset (Type 1) SMA patients, respectively. Both studies are enrolling the initial dose escalation part of the study which will then transition to the pivotal part of the study evaluating efficacy. Commencement of the pivotal portion of either study will trigger a single $20 million milestone payment to PTC from Roche. RG7916 was recently granted orphan-drug designation by the FDA.
- **Announced results from Phase 3 clinical trial of Translarna (ataluren) in nonsense mutation cystic fibrosis patients.** ACT CF did not achieve its primary or secondary endpoints. Ataluren was generally well tolerated and ACT CF confirmed a favorable safety profile for ataluren, which has now been used by more than 1,000 patients across multiple indications. PTC plans to discontinue current clinical development of ataluren in cystic fibrosis and close ongoing extension studies. The company has withdrawn its application for marketing authorization in cystic fibrosis in Europe.
- **Advanced clinical pipeline in rare disorders and oncology.** We continue to pursue our Phase 2 proof-of-concept studies of Translarna in additional rare disease indications, including aniridia, MPS I, and Dravet/CDKL5. Clinical development of PTC596, PTC's cancer stem cell investigational new drug, is expected to progress in 2017. Additionally, PTC's genetic disorders research organization is actively advancing lead optimization programs from its splicing platform focused on Huntington's disease and Familial Dysautonomia.

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 results 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 the Company's strategic plans. These results 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 measures are useful to investors and other users of our financial statements by providing greater transparency into the operating performance at PTC and the company's future outlook.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2016	**2015**	**2016**	**2015**
Revenues:				
Net product revenue	$ 25,119	$ 12,694	$ 81,447	$ 33,696
Collaboration and grant revenue	72	40	1,258	3,070
Total revenues	25,191	12,734	82,705	36,766
Operating expenses:				
Research and development (1)	26,011	35,048	117,633	121,816
Selling, general and administrative (2)	24,172	25,887	97,130	82,080
Total operating expenses	50,183	60,935	214,763	203,896
Loss from operations	(24,992)	(48,201)	(132,058)	(167,130)
Interest expense, net	(2,127)	(2,537)	(8,276)	(2,367)
Other income (expense), net	686	42	(1,207)	(465)
Loss before income tax expense	(26,433)	(50,696)	(141,541)	(169,962)
Income tax expense	(363)	(252)	(569)	(485)
Net loss attributable to common stockholders	$ (26,796)	$ (50,948)	$ (142,110)	$ (170,447)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	34,168,249	33,915,316	34,044,584	33,626,248
Net loss per share—basic and diluted (in dollars per share)	$ (0.78)	$ (1.50)	$ (4.17)	$ (5.07)
(1) Research and development expense reconciliation				
GAAP research and development	$ 26,011	$ 35,048	$ 117,633	$ 121,816
Less: share-based compensation	4,078	3,686	16,812	16,138
Less: one-time restructuring cost	(5)	—	840	—
Non-GAAP research and development expense	$ 21,938	$ 31,362	$ 99,981	$ 105,678
(2) Selling, general and administrative expense reconciliation				
GAAP selling, general and administrative	$ 24,172	$ 25,887	$ 97,130	$ 82,080
Less: share-based compensation	4,321	4,163	18,197	17,841
Less: one-time restructuring cost	(17)	—	1,644	—
Non-GAAP selling, general and administrative expense	$ 19,868	$ 21,724	$ 77,289	$ 64,239

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

	December 31, 2016	December 31, 2015
Cash, cash equivalents and marketable securities	$ 231,666	$ 338,925
Total assets	**$ 269,345**	**$ 365,281**
Total debt	$ 98,216	$ 91,848
Total deferred revenue	1,587	139
Total liabilities	**$ 149,762**	**$ 139,280**
Total stockholders' equity (34,169,410 and 33,916,559 common shares issued and outstanding at December 31, 2016 and December 31, 2015, respectively)	119,583	226,001
Total liabilities and stockholders' equity	**$ 269,345**	**$ 365,281**

Today's Conference Call and Webcast Reminder

PTC will host a call today at 8:30 am ET, which can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) and providing the passcode 61526711. 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. An accompanying slide presentation will be posted at 8:15 AM 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' 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, 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 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; the closure of extension studies for Translarna for the treatment of nonsense mutation cystic fibrosis; the clinical utility and potential advantages of Translarna (ataluren); advancement of PTC's studies of Translarna for the treatment of other indications; progression of clinical development of PTC596; PTC's expectations with respect to the closing of its planned acquisition of all rights to Emflaza™ (deflazacort), or the "planned acquisition"; PTC's expectations with respect to contingent payments to Marathon based on annual net sales; 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 "look forward", "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 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 and PTC's other product candidates; the outcome of pricing and reimbursement negotiations in those territories in which PTC may be authorized to sell Translarna for the treatment of nmDMD; 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 RG7916; PTC's scientific approach and general development progress; satisfaction of the conditions to closing the planned acquisition in the anticipated timeframe or at all; PTC's ability to realize the anticipated benefits of the planned acquisition, including the possibility that the expected benefits from the planned acquisition will not be realized or will not be realized within the expected time period; negative effects of the announcement of the planned acquisition on the market price of PTC's common stock; the risk of significant transaction costs, unknown liabilities, and litigation and/or regulatory actions

related to the planned acquisition; 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 or 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 Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization for Translarna for the treatment of nmDMD in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

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.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/ptc-therapeutics-reports-fourth-quarter-and-full-year-2016-financial-results-and-provides-corporate-update-300424706.html

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