March 6, 2018

PTC Therapeutics Reports Fourth Quarter and Full Year 2017 Financial Results and Provides Corporate Update

- 2017 total revenues of $194M, a 135% increase vs. 2016 -
- Full year 2018 net product revenue guidance of $260M to $295M -
- Conference call at 4:30 p.m. ET -

SOUTH PLAINFIELD, N.J., March 6, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ended December 31, 2017.

"Our mission for the past 20 years has been to develop and bring treatments to patients with rare genetic disorders," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are thrilled with the progress we made in 2017. We now have two approved commercial products for patients living with Duchenne muscular dystrophy and have built a global commercial platform. We are also excited with the progress in our splicing platform including the rapidly advancing SMA program. Our commercial success and our developing pipeline positions PTC as a fast-growing, global rare disorder company."

Fourth Quarter and Full Year 2017 Financial Highlights:

- Total revenues for the fourth quarter of 2017 were $78.0 million compared to $25.2 million in the fourth quarter of 2016. For the full year 2017, total revenues were $194.4 million compared to $82.7 million in 2016. The change in total revenue was a result of the expanded commercial launch of Translarna™, the successful U.S. Emflaza™ launch and a $20 million milestone payment achieved from Roche for the initiation of the pivotal portion of the SMA clinical trial.
- Translarna net product sales were $41.0 million for the fourth quarter of 2017, representing 63% growth over $25.1 million reported in the fourth quarter of 2016. For the full year 2017, Translarna generated $145.2 million in net product sales, representing 78% growth compared to $81.4 million in 2016.
- Emflaza net product sales were $17.0 million for the fourth quarter of 2017 and $28.8 million for the full year 2017.
- GAAP R&D expenses were $29.2 million for the fourth quarter of 2017 compared to $26.0 million for the same period in 2016. For the full year 2017, GAAP R&D expenses were $117.5 million compared to $117.6 million in 2016. The increase in GAAP R&D expense for the fourth quarter of 2017 as compared to the prior year period was due to increased clinical activities and regulatory spend.
- Non-GAAP R&D expenses were $25.7 million for the fourth quarter of 2017, excluding $3.5 million in non-cash, stock-based compensation expense, compared to $21.9 million for the same period in 2016, excluding $4.1 million in non-cash, stock-based compensation expense. For the full year 2017, non-GAAP R&D expenses were $102.0 million, excluding $15.5 million in non-cash, stock-based compensation expense, compared to $100.0 million for 2016, excluding $16.8 million in non-cash, stock-based compensation expense and $0.8 million in one-time restructuring expense.
- GAAP SG&A expenses were $35.5 million for the fourth quarter of 2017 compared to $24.2 million for the same period in 2016. For the full year 2017, GAAP SG&A expenses were $121.3 million compared to $97.1 million in 2016. The increase in SG&A expenses for the fourth quarter and year ended December 31, 2017, as compared to the prior year periods, was primarily due to the continued commercial support for the Emflaza launch.
- Non-GAAP SG&A expenses were $32.5 million for the fourth quarter of 2017, excluding $3.0 million in non-cash, stock-based compensation expense, compared to $19.9 million for the same period in 2016, excluding $4.3 million in non-cash, stock-based compensation expense. For the full year 2017, non-GAAP SG&A expenses were $106.2 million, excluding $15.1 million in non-cash, stock-based compensation expense, compared to $77.3 million for 2016, excluding $18.2 million in non-cash, stock-based compensation expense and $1.6 million in one-time restructuring expense.
- Net interest expense for the fourth quarter of 2017 was $3.4 million compared to net interest expense of $2.1 million in the same period in 2016. For the full year 2017, net interest expense was $12.1 million compared to net interest expense of $8.3 million in 2016. The increase in net interest expense for the fourth quarter and year ended December 31, 2017, as compared to the prior year periods, 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 interest income from investments.
- Net income for the fourth quarter of 2017 was $1.3 million compared to a net loss of $26.8 million for the same period in 2016. Net loss for the full year 2017 was $79.0 million, compared to $142.1 million for the same period in 2016.
- Cash, cash equivalents, and marketable securities totaled approximately $191.2 million at December 31, 2017

compared to $231.7 million at December 31, 2016.
- Shares issued and outstanding as of December 31, 2017, were 41.6 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 Fourth Quarter and Full Year 2017 Corporate Highlights:

- **Strong commercial execution has led to robust year over year revenue growth.** In 2017, our Duchenne muscular dystrophy franchise generated $174 million dollars.
  - Translarna reported revenue of approximately $145 million dollars, a 78% increase over the prior year. The demand for Translarna continues to increase in established regions such as Western Europe, LATAM, and most recently in the Middle East and Central and Eastern Europe. PTC plans for continued growth for Translarna by increasing penetration in current countries and pursuing opportunities for label expansion. In January, we guided to a 5-year, 15% CAGR through year end of 2022.
  - Since launching in May, Emflaza reported revenue of approximately $29 million in 2017. The reception of Emflaza by both patients and healthcare providers has been very strong. We are focused on enabling all eligible patients to have access to this therapy. In line with PTC's mission, PTC has established programs with the goal of ensuring that all eligible patients will have access to Emflaza regardless of financial or insurance status.
- **Data demonstrating that Emflaza is a differentiated product over prednisone were published in top-tier peer-reviewed journal and in recently published Duchenne treatment guidelines.** These data indicate that Emflaza delays the loss of major milestones by 2 to 3 years compared to prednisone. Based on the understanding of both our own internally generated results and independently published data, we believe that Emflaza should be the standard of care for all Duchenne muscular dystrophy patients.
- **Regulatory update for Translarna in the US**. The Office of New Drugs of the U.S. Food and Drug Administration has reiterated the FDA's prior position and denied PTC's appeal of the Complete Response Letter in relation to the New Drug Application (NDA) for ataluren. In its response, 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. We intend to follow the FDA's recommendation and will collect such dystrophin data using newer technologies via procedures and methods that will be mutually agreeable to us and the FDA. The response also stated that Study 041, which is currently enrolling, could serve as the confirmatory post-approval trial required in connection with the accelerated approval framework.
- **SMA program advancing with two registration-directed trials.** The SUNFISH trial in the spinal muscular atrophy (SMA) program transitioned to the pivotal portion which triggered a $20M milestone payment from Roche in the fourth quarter of 2017. A dose has been selected in the FIREFISH trial and it is anticipated to transition to the pivotal stage in the coming weeks. A recent presentation at the International Scientific Congress on SMA in Krakow reviewed the ongoing, dose-finding Part 1 of FIREFISH in the Type 1 SMA infants highlighting survival data, as well as safety and interim clinical data. No patients had discontinued due to adverse events. Early interim clinical data reported no patient lost the ability to swallow and no patient has required tracheostomy or reached permanent ventilation. The program also includes an additional study, JEWELFISH, for patients who have previously received splicing therapies. The SMA program is a joint collaboration with Roche and the SMA Foundation.
- **PTC to provide details of its developing R&D pipeline at its Analyst Day, April 17th.** PTC will outline its plans for the continued sustainable growth of its internally developed programs.

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, these 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 financial measures 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 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.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	**2016**	**2017**	**2016**
Revenues:				
Net product revenue	$ 57,953	$ 25,119	$ 174,066	$ 81,447
Collaboration and grant revenue	20,077	72	20,326	1,258
Total revenues	78,030	25,191	194,392	82,705
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible asset	2,434	—	4,577	—
Amortization of acquired intangible asset	5,428	—	15,380	—
Research and development (1)	29,234	26,011	117,456	117,633
Selling, general and administrative (2)	35,482	24,172	121,271	97,130
Total operating expenses	72,578	50,183	258,684	214,763
Income (loss) from operations	5,452	(24,992)	(64,292)	(132,058)
Interest expense, net	(3,446)	(2,127)	(12,094)	(8,276)
Other income (expense), net	93	686	(1,279)	(1,207)
Income (loss) before income tax expense	2,099	(26,433)	(77,665)	(141,541)
Income tax expense	(829)	(363)	(1,335)	(569)
Net income (loss) attributable to common stockholders	$ 1,270	$ (26,796)	$ (79,000)	$ (142,110)
Weighted-average shares outstanding:				
Basic (in shares)	41,344,897	34,168,249	39,183,073	34,044,584
Diluted (in shares)	41,965,276	34,168,249	39,183,073	34,044,584
Net income (loss) per share:				
Basic (in dollars per share)	$ 0.03	$ (0.78)	$ (2.02)	$ (4.17)
Diluted (in dollars per share)	$ 0.03	$ (0.78)	$ (2.02)	$ (4.17)
(1) Research and development reconciliation				
GAAP research and development	$ 29,234	$ 26,011	$ 117,456	$ 117,633
Less: share-based compensation expense	3,470	4,078	15,456	16,812
Less: one-time restructuring cost	—	(5)	—	840
Non-GAAP research and development	$ 25,764	$ 21,938	$ 102,000	$ 99,981
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 35,483	$ 24,172	$ 121,271	$ 97,130
Less: share-based compensation expense	3,007	4,321	15,103	18,197
Less: one-time restructuring cost	—	(17)	—	1,644
Non-GAAP selling, general and administrative	$ 32,476	$ 19,868	$ 106,168	$ 77,289

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

	December 31, 2017	December 31, 2016
Cash, cash equivalents and marketable securities	$ 191,246	$ 231,666
Total assets	**$ 391,653**	**$ 269,345**
Total debt	$ 144,971	$ 98,216
Total deferred revenue	11,891	1,587
Total liabilities	**$ 235,216**	**$ 149,762**
Total stockholders' equity (41,612,395 and 34,169,410 common shares issued and outstanding at December 31, 2017 and December 31, 2016, respectively)	156,437	119,583

Total liabilities and stockholders' equity	**$ 391,653**	**$ 269,345**

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 R&D and SG&A shared-based compensation expense	30,000	30,000
Total projected non-GAAP R&D and SG&A expense	**$ 250,000**	**$ 260,000**

Upcoming Events:
PTC will participate in the following upcoming investor conferences:

- Cowen and Company 38th Annual Health Care Conference, on Wednesday, March 14th at 8:00 a.m. ET
- Barclays Global Healthcare Conference 2018, on Thursday, March 15th, at 9:00 a.m. ET

PTC will host an Analyst Day on April 17th from noon - 5 p.m. ET to provide an update on the company's growing pipeline.

Today's Conference Call and Webcast Reminder
Today's conference call will take place at 4:30 p.m. 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 429-97-93. 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 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. For more information, 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, 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 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: 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 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 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 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.

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