PTC Therapeutics Provides Corporate Update and Outlines 5-year Strategic Plan at 2019 J.P. Morgan Healthcare Conference

January 7, 2019

- Multiple products to be launched by 2023 potentially generating ~$1.5 billion revenue annually-
- Plan to submit BLA in 2H 2019 for AADC gene therapy program -
- Risdiplam regulatory filing targeted for 2H 2019 -
- TEGSEDI™ filed in Brazil for expected approval in 2019 -
- Duchenne franchise continuing to expand -

SOUTH PLAINFIELD, N.J., Jan. 7, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided a corporate update, which will be detailed as part of the company's presentation at the 37th Annual J.P. Morgan Healthcare Conference on Tuesday, January 8th at 5:00 p.m. PT. Stuart W. Peltz, Ph.D., PTC's Chief Executive Officer, will highlight the company's growth and diversification focused on bringing clinically differentiated, life-changing treatments to patients affected by rare disorders. Additionally, an overview of the company's strategic vision will be provided as well as preliminary 2018 financial results and 2019 financial guidance. The presentation will be webcast live on the Events and Presentations page under the investors' section of PTC Therapeutics' website at www.ptcbio.com.

Corporate Highlights

Advancing gene therapy portfolio

- PTC plans to submit a BLA with the FDA followed by an MAA in Europe for the AADC deficiency gene therapy program in 2H 2019 with expected launch in 2020. Identification of patients with AADC deficiency has been a priority for the Company, with approximately 100 patients identified to date in the U.S. and Europe. PTC expects to screen about 100,000 patients who are at risk for AADC deficiency before the regulatory approval to maximize patient benefit at time of launch.
- Friedreich's ataxia program is advancing with an expected IND filing and entry into the clinic in 2019.
- PTC is enhancing its internal research and in-house manufacturing capabilities with fully dedicated resources to maximize current and future programs.

Risdiplam regulatory filing progress. The SMA program is a collaboration between PTC, Roche and SMA Foundation.

- Successfully completed enrollment of pivotal portion of FIREFISH trial in Type 1 SMA babies in 2018.
- Regulatory submission targeted for the second half of 2019 based on recent feedback from the FDA & national health authorities in Europe that Part 1 of FIREFISH and SUNFISH may be sufficient to file NDA/MAA.
- The goal of the clinical development program is to support the approval of a broad label so risdiplam can potentially benefit as many SMA patients as possible.
- Short- and long-term cash milestones to PTC expected upon regulatory approval and commercial launches. PTC expects the peak annual royalties/milestones from risdiplam to exceed $200M.

Expanding commercial platform

- TEGSEDI™ application filed with ANVISA- Brazilian regulatory authority. ANVISA granted priority review. PTC expects approval in Brazil by year end 2019. TEGSEDI has the potential to generate peak revenues of approximately $150M in LATAM.
- Duchenne franchise expected to continue to grow over the next 5 years. Translarna ex-U.S. launch in patients 2 to 5 years of age now initiated. Non-ambulatory label expansion is currently under EMA regulatory review. Emflaza® continues to accrue new patients in the U.S. with expected increased market share gained via differentiation and improved market access.
- In recent interactions, the FDA invited PTC to submit a supplementary NDA (sNDA) for Emflaza for patients 2 to 5 years of age on the basis that existing data support its safety and efficacy in this population. PTC recently submitted the sNDA for potential approval in 2019. The previous written request from the FDA that a trial in patients 2 to 5 years of age be performed has been officially withdrawn and the trial will no longer be conducted. PTC now expects to launch Emflaza in this younger population before the end of 2019.

Growing pipeline and R&D capabilities

- PTC's alternative splicing platform has generated another development candidate. PTC258 was selected as a development candidate for Familial dysautonomia (FD), a rare genetic neurological disorder that effects the sensory and autonomic

nervous systems, causing life-threatening medical complications from birth. PTC258 is advancing to IND enabling studies with the goal to enter the clinic in late 2019. This program is in collaboration with MGH and NYU.
- Translarna's dystrophin study was initiated in 4Q 2018 for potential U.S. regulatory submission in early 2020.
- PTC's oncology portfolio continues to advance with the initiation of a study in AML with PTC299 and a DIPG study for PTC596. PTC expects these studies to move to the expanded cohort stage in 2020. PTC596 is also being developed for the treatment of patients with Leiomyosarcoma (LMS) with the first patient in that trial expected to be dosed in Q1 2019.

Preliminary Unaudited 2018 Financial Results

- PTC expects to report Translarna™ (ataluren) net product revenue for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) of approximately $171 million for full year 2018, an increase of 18% over the prior year.
- PTC expects to report EMFLAZA® (deflazacort) net product revenue for the treatment of Duchenne muscular dystrophy (DMD) of approximately $91 million for full year 2018, an increase from $28.8 million in the prior year.
- PTC expects to report year-end cash and cash equivalents of approximately $227 million.

PTC is currently in the process of finalizing its financial results for the 2018 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2018, subject to the completion of PTC's financial closing procedures. In addition, the above information is subject to revision as PTC completes its financial closing procedures for fiscal 2018.

2019 Guidance

- PTC anticipates full year net product revenues to be between $285 and $305 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2019 to be between $395 and $405 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2019 to be between $360 and $370 million, excluding estimated non-cash, stock-based compensation expense of approximately $35 million.

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, the non-GAAP financial measure excludes non-cash, stock-based compensation expense. This non-GAAP financial measure is provided as a complement to financial measures reported in GAAP because management uses this non-GAAP financial measure when assessing and identifying operational trends. In management's opinion, this non-GAAP financial measure is 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. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. A quantitative reconciliation of the non-GAAP financial measure to its closest equivalent GAAP financial measure is included in the table below.

PTC Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP 2019 Projected Full Year R&D and SG&A Expense (In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 395,000	$ 405,000
Less: projected non-cash stock-based compensation expense	35,000	35,000
Total projected non-GAAP R&D and SG&A expense	**$ 360,000**	**$ 370,000**

SMA Milestones/Royalties supporting information

- SMA program Royalties to PTC from Roche based on net sales:

Tier of Calendar Year Worldwide Net Sales in $US million	Percent (%) of Net Sales
0 – 500	8
> 500 – 1,000	11
> 1,000 – 2,000	14
> 2,000	16

- SMA program Sales-threshold-based payments to PTC from Roche:

Event Total Calendar Year Net Sales ($US)	Payment ($US)
> $ 500,000,000	$ 25,000,000
> $ 750,000,000	$ 50,000,000
> $ 1,500,000,000	$ 100,000,000
> $ 2,500,000,000	$ 150,000,000
Total Remaining	$ 325,000,000

- SMA program Milestone-based payments to PTC from Roche:

Event	Payment ($US)
Filing of an NDA in the US	$ 15,000,000
Filing of an NDA in an EU country or with the EMA	$ 15,000,000
Filing of an NDA in Japan	$ 7,500,000
First Commercial Sale in US	$ 20,000,000
First Commercial Sale in the EU	$ 20,000,000
First Commercial Sale in Japan	$ 10,000,000
Total Remaining	$ 87,500,000

About PTC Therapeutics, Inc.
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. PTC's ability to globally commercialize products 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.

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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 headings "Preliminary Unaudited 2018 Financial Results", including with respect to (i) 2018 net product revenue of Translarna for the treatment of nmDMD and EMFLAZA for the treatment of Duchenne muscular dystrophy and (ii) year-end 2018 cash and cash equivalents, and "2019 Guidance", including with respect to (i) 2019 net product revenue guidance and (ii) 2019 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing and potential commercialization of TEGSEDI and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions; PTC's strategy, future operations, future financial position, future revenues, 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 and any other product candidates that PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its new drug application 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; expectations with respect to the potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of TEGSEDI and Waylivra in countries in LATAM and the Caribbean, the commercialization of TEGSEDI and Waylivra, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; the enrollment, conduct, and results 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, including any potential regulatory submissions with regards to Risdiplam; 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 or the acquisition of its gene therapy pipeline, 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, PTC-AADC, TEGSEDI, Waylivra, Risdiplam or any of PTC's other product candidates; 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 Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the periods ended March 31, 2018, June 30, 2018

and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 2018, 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, Emflaza, PTC-AADC, TEGSEDI, Waylivra or Risdiplam.

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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