PTC Therapeutics Provides Corporate Update and Highlights Pipeline Progress at 2020 J.P. Morgan Healthcare Conference

January 13, 2020

- PTC-AADC MAA submitted; BLA now expected to be submitted in 2Q 2020 -
- 3 clinical trials from PTC's new redox platform to begin in 2020 -
- ~ $306 million preliminary unaudited 2019 total revenue -
- 2020 Duchenne franchise revenue guidance of $320-340 million -

SOUTH PLAINFIELD, N.J., Jan. 13, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today is providing a corporate update that will be presented at the 38th Annual J.P. Morgan Healthcare Conference on Wednesday, January 15th at 8:30 a.m. PST. Stuart W. Peltz, Ph.D., PTC's Chief Executive Officer, will provide an update on the 2019 accomplishments and highlight value creating events in the upcoming year. Additionally, the Company is providing preliminary 2019 financial results and 2020 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:

Gene Therapy Platform Update:

- PTC has submitted a Marketing Authorization Application (MAA) for the potential approval of a gene therapy treatment, PTC-AADC, for AADC deficiency with the European Medicines Agency (EMA). PTC expects the Committee for Medicinal Products for Human Use (CHMP) opinion in 2H 2020.
- In a recent interaction with the U.S. Food and Drug Administration (FDA) there was a request for additional information concerning the use of the commercial delivery system for PTC-AADC in young patients. Patients have been identified who are immediately available for treatment with the commercial delivery system. We anticipate that this will cause a short delay of the Biologics License Application (BLA) and should allow for submission to the FDA in 2Q 2020.
- PTC has identified 200 AADC patients to date and continues to anticipate that over 300 AADC patients will be identified by launch.
- PTC-FA gene therapy for Friedreich ataxia is progressing and is anticipated to enter the clinic in 3Q 2020.
- In order to control and accelerate its gene therapy platform, PTC secured a 15-year lease on ~185,000 sq. ft. of space, which includes a state-of-the-art biologics production facility with supporting research and operations buildings in NJ. PTC expects manufacturing to begin at this facility in 2020.

Risdiplam Regulatory & Clinical Updates:

- The risdiplam Prescription Drug User Fee Act (PDUFA) date for a decision by the FDA is May 24, 2020. Risdiplam is expected to be indicated for spinal muscular atrophy (SMA) type 1, 2 & 3 patients, if approved.
- In November 2019, PTC announced that the pivotal part of the SUNFISH trial in SMA type 2/3 patients was successful, meeting its primary endpoint. Data from the pivotal part of SUNFISH will be presented at the SMA Europe Conference from February 5 – 7, 2020.
- Topline results from the pivotal portion of the open label FIREFISH study in type 1 SMA patients are anticipated in 1Q 2020.
- The SMA program has studied the broadest cohort of SMA patients in clinical trials to date. This program is a collaboration between PTC, Roche and the SMA Foundation.

Expanding commercial platform:

- The Duchenne muscular dystrophy (DMD) franchise, consisting of Translarna™ and Emflaza®, continues to grow with 2019 unaudited revenue of approximately $291 million.

- Translarna™ continues to grow with further penetration into existing territories, geographic expansion into new territories, increased awareness and earlier diagnosis. PTC plans to re-submit the Translarna New Drug Application (NDA) to the FDA with the data from the dystrophin study in mid-year 2020.
- Growth in Emflaza® revenue was based on a recent label expansion in the 2-5-year-old patient population, and reduced payer restrictions resulting from multiple publications showing benefit of Emflaza relative to prednisone. One such recent publication, which included real-world data from Cincinnati Children's hospital, specifically highlighted the benefit of switching from prednisone to Emflaza.

- Tegsedi® (inotersen) received approval from the Brazilian health regulatory authority (ANVISA) for the treatment of stage 1 or 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR), to delay disease progression and improve quality of life. The commercial launch for Tegsedi is ongoing with patient and physician services to support the launch, including medical education and diagnosis efforts.

Growing pipeline and R&D capabilities:

- In 2020, PTC plans to initiate three trials in its newly acquired redox platform with two unique compounds that regulate inflammation and oxidative stress.
  - These include potential registrational trials in mitochondrial epilepsy and Friedreich ataxia with PTC743. Additionally, a Phase 1 trial with PTC857 is also planned targeting GBA Parkinson's disease.
- In late 2020, PTC expects to file an investigational new drug (IND) application for its development candidate in Huntington disease. PTC has several additional programs from the splicing platform in development with undisclosed targets.

Preliminary Unaudited 2019 Financial Results

- Total unaudited revenue for 2019 was approximately $306 million. This includes expected net product revenue for the Duchenne muscular dystrophy franchise of approximately $291 million for the full year 2019 and a $15 million milestone payment from Roche for the risdiplam NDA acceptance.
- PTC expects to report net product revenue for Translarna of approximately $190 million for full year 2019. As a reminder, the ANVISA approval in Brazil should lead to expanded market access in 2020 and beyond but led to a price discount that impacted 2019 net sales.
- Additionally, PTC expects to report net product revenue for Emflaza of approximately $101 million for the full year 2019.
- PTC expects to report 2019 year-end cash and cash equivalents of approximately $686 million.

PTC is currently in the process of finalizing its financial results for the 2019 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2019, 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 2019.

2020 Guidance

- PTC anticipates full year net product revenues for the DMD franchise to be between $320 and $340 million. New product launches, including Tegsedi, Waylivra, PTC-AADC and risdiplam, could contribute revenue in 2020. Remaining milestones and royalties from Roche on the SMA program are outlined in the table below.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2020 to be between $610 and $640 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2020 to be between $545 and $575 million, excluding estimated non-cash, stock-based compensation expense of approximately $65 million.
- The increase in R&D and SG&A expense are based in part on highly leverageable and scalable investments towards the $1.5 billion projected revenue in 2023, including gene therapy manufacturing, an increase in the number of programs advancing into the clinic and launches.

SMA Milestones/Royalties supporting information

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

Event	Payment ($US)
Filing of a MAA 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	$72,500,000

- 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

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 Projected Full Year 2020 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 610,000	$ 640,000
Less: projected non-cash, stock-based compensation expense	65,000	65,000
Projected non-GAAP R&D and SG&A expense	**$ 545,000**	**$ 575,000**

About PTC Therapeutics, Inc.
PTC is a science-driven, 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 heading "2020 Guidance", including with respect to (i) 2020 net product revenue guidance and (ii) 2020 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 and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza and related 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 PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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 and potential commercialization with regards to risdiplam; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary 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 commercialization of Tegsedi and Waylivra significant 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 lease agreement for its leased biologics facility; 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 and 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, 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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