PTC Therapeutics Provides Update on R&D Pipeline and Commercial Progress at 39th Annual J.P. Morgan Healthcare Conference

January 11, 2021

- ~$333 million preliminary unaudited 2020 total net product revenue representing 14% year-over-year growth -
- Huntington splicing program healthy volunteer data expected 1H 2021 -
- Multiple late-stage clinical programs advancing with six registration-directed trials -
- 2021 Duchenne franchise revenue guidance of $355-$375 million -

SOUTH PLAINFIELD, N.J., Jan. 11, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its R&D pipeline and commercial progress at the 39th Annual J.P. Morgan Healthcare Conference today, Monday, Jan. 11 at 7:30 a.m. EST. Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2020 accomplishments and highlight upcoming potential value-creating milestones. Preliminary 2020 unaudited financial results and 2021 financial guidance will also be provided. The presentation will be webcast live on the Events and Presentations page of the Investors section of PTC Therapeutics website at www.ptcbio.com.

Key 2020 Corporate Highlights:

- The Duchenne muscular dystrophy (DMD) franchise, consisting of Translarna™ (ataluren) and Emflaza® (deflazacort), continue to demonstrate year-over-year growth, with 2020 preliminary unaudited revenue of approximately $331 million.
  - Translarna growth is primarily driven by geographical expansion and label modifications allowing broader access.
  - Emflaza experienced strong 38% year-over-year revenue growth in 2020 due to increased patient prescriptions and high compliance.
- In August, Evrysdi™ (risdiplam) was approved by the FDA for patients with Spinal Muscular Atrophy (SMA). A strong global launch is underway with increasing U.S. patient uptake across all disease subtypes in both treatment naïve and switch patients. The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) opinion for Evrysdi is expected in 1H 2021, and priority review of the Japanese New Drug Application (NDA) is ongoing. Evrysdi is the first at-home, orally administered SMA treatment and was developed from PTC's proprietary splicing platform. Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation and Roche.
- PTC initiated three clinical trials from its novel Bio-e platform, including two registration-directed trials with vatiquinone (PTC743) in Mitochondrial epilepsy (MIT-E) and Friedreich ataxia (MOVE-FA) that are actively enrolling.

2021 Potential Value-Creating Milestones:

- Top-line results from the Translarna dystrophin study are expected in 1Q 2021. With positive results, PTC plans to submit an NDA to the FDA.
- PTC is preparing to launch its first potential gene therapy, PTC-AADC, for the highly morbid genetic pediatric disorder aromatic L-amino acid decarboxylase deficiency (AADC-d). Launch efforts include expanding genetic testing programs and the identification and preparation of expert pediatric neurosurgical centers.
- PTC-AADC expected regulatory milestones include a CHMP opinion for a potential approval and the submission of a Biologics License Application (BLA) to the FDA in 1H 2021.
- Results from PTC's Huntington disease program Phase 1 study of PTC518 in healthy volunteers are expected in 1H 2021. PTC518 was discovered from PTC's validated splicing platform.
- A registration-directed clinical trial of PTC299 for COVID-19 (FITE19) continues to enroll patients. PTC anticipates completing the study in 1H 2021.
- A registration-directed study, APHENITY, for PTC923 in patients with phenylketonuria (PKU) is expected to initiate in mid-2021.
- Results by year end are expected from ongoing clinical trials evaluating PTC596 in Leiomyosarcoma and Diffuse Intrinsic Pontine Glioma (DIPG).
- Gene therapy manufacturing for clinical trials will begin at PTC's state-of-the-art biologics production facility in Hopewell, N.J.

Preliminary Unaudited 2020 Financial Results:

- Total unaudited net product revenue for full year 2020 was approximately $333 million.

- DMD franchise revenue for year end 2020 included net product revenue for Translarna of approximately $192 million with $69 million in revenue in the fourth quarter and approximately $139 million year end 2020 revenue for Emflaza with $37 million in revenue in fourth quarter.
- PTC expects to report $42.5 million in 2020 revenue associated with Evrysdi milestones. PTC will report fourth quarter royalty revenue for Evrysdi on the company's next earnings call.
- PTC expects to report 2020 year-end cash and cash equivalents of approximately $1.1 billion.

PTC is currently in the process of finalizing its financial results for the 2020 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2020, subject to the completion of PTC's financial closing procedures.

2021 Financial Guidance:

- PTC anticipates full year net product revenues for the DMD franchise to be between $355 and $375 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2021 to be between $825 and $855 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2021 to be between $725 and $755 million, excluding estimated non-cash, stock-based compensation expense of approximately $100 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 Projected Full Year 2021 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 825,000	$ 855,000
Less: projected non-cash, stock-based compensation expense	100,000	100,000
Projected non-GAAP R&D and SG&A expense	**$ 725,000**	**$ 755,000**

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 "2021 Guidance", including with respect to (i) 2021 net product revenue guidance and (ii) 2021 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; 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 commercialization of its products and product candidates; the timing with respect to orders for PTC's products; 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 manufacturing 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 ongoing 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 respect to Evrysdi; 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™; the enrollment, conduct and results of PTC's PTC299 clinical trial for COVID-19; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 PTC's products and 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 manufacturing facility; 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, Evrysdi, Tegsedi, Waylivra or PTC-AADC.

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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