PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 40th Annual J.P. Morgan Healthcare Conference

January 10, 2022

- $536 million unaudited 2021 total revenue representing impressive 41% year-over-year growth -
- $700 - 750 million 2022 total revenue guidance -
- Results are expected in four registration-directed trials this year -
- Three additional registration-directed clinical trials expected to initiate in 2022 -
- Dr. Matthew Klein promoted to Chief Operating Officer and will continue to oversee the Development organization -

SOUTH PLAINFIELD, N.J., Jan. 10, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 40th Annual J.P. Morgan Healthcare Conference today, Monday Jan. 10th at 7:30am EST. Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2021 accomplishments and highlight upcoming potential value-creating milestones. Preliminary 2021 unaudited financial results and 2022 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 2021 Corporate Highlights:

- Unaudited net product revenue of $429 million in 2021 representing 29% year-over-year growth.
- Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $424 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in 2021. Cumulative net product revenue for Translarna exceeds $1 billion and Emflaza exceeds $500 million, since respective launches.
    - Translarna growth was driven by new patients and high compliance in existing geographies and continued geographic expansion.
    - Emflaza growth was due to continued new prescriptions, high compliance, less patient discontinuations and more favorable access.
- Evrysdi® (risdiplam) has shown continued rapid uptake in the United States and is now approved in all major markets including the European Union and Japan. Evrysdi is a product of the Spinal Muscular Atrophy (SMA) collaboration between PTC, the SMA Foundation and Roche.
- Waylivra® (volanesorsen), the only treatment for familial chylomicronemia syndrome was approved by Brazilian Health Regulatory Agency, Agência Nacional de Vigilância Sanitária (ANVISA), and both Waylivra and Tegsedi® (inotersen) received Category 1 classification from Câmara de Regulação do Mercado de Medicamentos - CMED (Drug Market Regulation Chamber) in Brazil. Category 1 classification is given to innovative treatments that provide greater efficacy than the standard of care and allows for pricing in line with international markets.
- PTC successfully advanced its clinical pipeline in 2021:
    - APHENITY, a Phase 3 registration-directed trial of PTC923 in phenylketonuria (PKU) was initiated.
    - Phase 1 healthy volunteer trials of PTC518 and PTC857 were completed.
    - Advanced the oncology platform with the completion of Phase 1b studies of unesbulin in leiomyosarcoma and diffuse intrinsic pontine glioma (DIPG).
    - Completed enrollment for the vatiquinone MOVE-FA registration-directed trial with results expected in the second quarter of 2023.

2022 Potential Key Value-Creating Milestones:

- Results from Study 041 for Translarna are expected mid-year 2022 and if positive could support re-submission of a New Drug Application (NDA) to the Food and Drug Administration (FDA).
- Results for MIT-E, the registration-directed study of vatiquinone in mitochondrial disease associated seizures, are expected in the fourth quarter of 2022.
- Results are expected by year end 2022 for the Phase 3 registration-directed study, APHENITY, for PTC923 in patients with PKU.
- From the splicing platform, the PIVOT-HD Phase 2 study of PTC518 in Huntington's disease patients is planned to initiate in the first quarter of 2022.
- From the Bio-e platform, the registration-directed CardinALS study of PTC857 in amyotrophic lateral sclerosis (ALS)

patients is expected to be initiated in the second quarter of 2022.
- Progress in the gene therapy platform is anticipated in 2022:
  - PTC expects an opinion from the European Medicine Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) for PTC-AADC, the first gene therapy for AADC deficiency, in April 2022.
  - Submission of a Biologics License Application (BLA) to the FDA for PTC-AADC is expected in the second quarter of 2022.

Preliminary Unaudited 2021 Financial Results:

- Total unaudited net revenue for full year 2021 was approximately $536 million.
- Total unaudited net product revenue for full year 2021 was approximately $429 million.
- DMD franchise revenue for year end 2021 included net product revenue for Translarna of approximately $236 million, with $70 million in revenue in the fourth quarter, and Emflaza of approximately $188 million, with $48 million in revenue in the fourth quarter.
- PTC expects to report approximately $107 million in 2021 collaboration and royalty revenue associated with Evrysdi.
- PTC expects to report 2021 year-end cash, cash equivalents and marketable securities of approximately $773 million.

PTC is currently in the process of finalizing its financial results for the 2021 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2021, subject to the completion of PTC's financial closing procedures.

2022 Financial Guidance:

- PTC anticipates total revenues for the full year 2022 to be between $700 and $750 million.
- PTC anticipates net product revenues for the DMD franchise for the full year 2022 to be between $475 and $495 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2022 to be between $915 and $965 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2022 to be between $800 and $850 million, excluding estimated non-cash, stock-based compensation expense of $115 million.

Non-GAAP Financial Measures:

In this press release, the financial results and financial guidance of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP financial measures exclude non-cash, stock-based compensation expense. 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. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. Quantitative reconciliations of the non-GAAP financial measures to their respective closest equivalent GAAP financial measures are included in the table below.

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2022 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 915,000	$ 965,000
Less: projected non-cash, stock-based compensation expense	115,000	115,000
Projected non-GAAP R&D and SG&A expense	**$ 800,000**	**$ 850,000**

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and LinkedIn.

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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 "2022 Financial Guidance", including with respect to (i) 2022 total revenue guidance, (ii) 2022 net product revenue guidance for the DMD franchise and (iii) 2022 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 regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any 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; 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: expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 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 regulatory submissions and commercialization with respect to Evrysdi; PTC's ability to utilize results from 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, to support a marketing approval for Translarna for the treatment of nmDMD in the United States; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA) and other regions, 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 fund, complete and timely submit to the EMA the results of Study 041, 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 clinical trial for emvododstat for COVID-19; 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 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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