PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 41st Annual J.P. Morgan Healthcare Conference

January 9, 2023

- ~$710 million unaudited 2022 total revenue, representing an impressive over 30% year-over-year growth –

- $940 million - $1.0 billion 2023 total revenue guidance –

- Results from three registration-directed clinical trials expected in 2023 –

- One additional registration-directed clinical trial expected to initiate in 2023 –

SOUTH PLAINFIELD, N.J., Jan. 9, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 41st Annual J.P. Morgan Healthcare Conference today, Monday January 9, at 10:30am EST/7:30am PST. Matthew Klein, M.D., Chief Operating Officer of PTC Therapeutics, will provide an update on 2022 accomplishments and highlight upcoming 2023 potential value-creating milestones. Preliminary 2022 unaudited financial results and 2023 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 2022 Corporate Highlights:

- Upstaza™, the first direct-administered gene therapy into the brain, was approved in the EU and UK for AADC deficiency patients.
- Unaudited net product revenue of $535 million in 2022 representing 25% year-over-year growth.
  - Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $289 million for Translarna™ (ataluren) and $218 million for Emflaza® (deflazacort) in 2022.
    - Translarna growth was driven by new patients in existing geographies and continued geographic expansion.
    - Emflaza growth was due to continued new prescriptions, high compliance, fewer patient discontinuations and more favorable access.
  - Upstaza™ unaudited net product revenue was $13 million driven by patients being treated through early access programs and commercial access.
- Evrysdi® (risdiplam) is now approved in more than 90 countries. It has established market leadership in all major markets and is on track to become the global market leader in treatment of spinal muscular atrophy (SMA). Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation and Roche.
- PTC successfully advanced its clinical pipeline in 2022:
  - CardinALS, a registration-directed Phase 2 clinical trial of PTC857 in amyotrophic lateral sclerosis, was initiated.
  - PIVOT-HD, a Phase 2 clinical trial of PTC518 in Huntington's disease, was initiated.
  - SunriseLMS, a registration-directed Phase 2 trial of unesbulin in leiomyosarcoma, was initiated.
  - The placebo-controlled portion of Study 041, a Phase 3 clinical trial of Translarna for nmDMD, was completed. Study 041 results supported submission of a Type II variation for conversion to standard marketing authorization in the EU.
  - FITE-19, a Phase 2/3 clinical trial of emvododstat for COVID-19, was completed.

2023 Potential Key Value-Creating Milestones:

- Results from the placebo-controlled portion of APHENITY, the Phase 3 registration-directed clinical trial of sepiapterin in patients with PKU, are expected in the first quarter of 2023.
- Results from MIT-E, the Phase 2/3 registration-directed clinical trial of vatiquinone in mitochondrial disease associated seizures, are expected in the first quarter of 2023.
- Results from MOVE-FA, the Phase 3 registration-directed clinical trial of vatiquinone in Friedreich ataxia, are expected in the second quarter of 2023.
- Results from the 12-week portion of PIVOT-HD, the Phase 2 study of PTC518 in Huntington's disease, are expected in the second quarter of 2023.
- Submission of a Biologics License Application (BLA) to the FDA for Upstaza is expected in the first half of 2023.
- A Phase 2/3 clinical trial of unesbulin in diffuse intrinsic pontine glioblastoma is expected to initiate in the fourth quarter of 2023.

Preliminary Unaudited 2022 Financial Results:

- Total unaudited net revenue for full year 2022 was approximately $710 million.
- Total unaudited net product revenue for full year 2022 was approximately $535 million.
- DMD franchise unaudited revenue for full year 2022 was approximately $507 million, including net product revenue for Translarna of approximately $289 million and for Emflaza of approximately $218 million.
- PTC expects to report approximately $175 million in 2022 collaboration and royalty revenue associated with Evrysdi.

PTC is currently in the process of finalizing its financial results for the 2022 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2022, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on sell side analyst consensus estimates.

2023 Financial Guidance:

- PTC anticipates total revenues for the full year 2023 to be between $940 million and $1.0 billion.
- PTC anticipates net product revenues for the DMD franchise for the full year 2023 to be between $545 and $565 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2023 to be between $1.01 and $1.06 billion.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2023 to be between $890 and $940 million, excluding estimated non-cash, stock-based compensation expense of $120 million. PTC anticipates up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions.

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 2023 R&D and SG&A Expense (In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 1,010,000	$ 1,060,000
Less: projected non-cash, stock-based compensation expense	120,000	120,000
Projected non-GAAP R&D and SG&A expense	$ 890,000	$ 940,000

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 "2023 Financial Guidance", including with respect to (i) 2023 total revenue guidance, (ii) 2023 net product revenue guidance for the DMD franchise,(iii) 2023 GAAP and non-GAAP R&D and SG&A expense guidance and (iv) 2023 acquisition related one-time 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions, commercialization 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, 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; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, 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 complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; 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 and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the 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 its lease agreements, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; 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, Upstaza, Evrysdi, Tegsedi or Waylivra.

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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