PTC Therapeutics Provides Update on Commercial Performance and R&D Pipeline at 43rd Annual J.P. Morgan Healthcare Conference

January 13, 2025

– Unaudited 2024 total revenue of approximately $814 million, exceeding guidance –

– Four approval applications submitted to FDA in 2024 –

– Global launch preparations underway for sepiapterin for PKU, a $1 billion market opportunity; CHMP opinion expected Q2 2025, U.S. approval decision in July 2025 –

– License and collaboration agreement with Novartis for PTC518 program closed –

– PIVOT-HD data readout for PTC518 expected Q2 2025 –

WARREN, N.J., Jan. 13, 2025 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided an update on the Company's progress and its outlook for 2025. Matthew B. Klein, M.D., Chief Executive Officer of PTC, will discuss these updates at the 43rd Annual J.P. Morgan Healthcare Conference today at 11:15 a.m. PST / 2:15 p.m. EST. The presentation will be webcast live on the Events and Presentations page under the Investors section of PTC Therapeutics' website at https://ir.ptcbio.com/events-presentations and will be archived for 30 days following the presentation.

"I am very proud of our team's performance across every part of the company in 2024," Dr. Klein said. "We met every one of our planned clinical and regulatory milestones on schedule. Commercially, we outperformed guidance. And we begin 2025 with a strong cash balance, ready to build on our successes. PTC, now stronger and more innovative than ever, will continue to work to bring transformative therapies to the patient communities we serve in 2025 and beyond."

Key 2024 Corporate Highlights:

- Unaudited net product revenue of approximately $814 million, exceeding guidance.
  - Strong performance was driven by in-line products, including the DMD franchise with unaudited net product revenue of approximately $340 million for Translarna ™ (ataluren) and approximately $207 million for Emflaza® (deflazacort) in 2024.
- Cash balance of approximately $1.1 billion as of December 31, 2024, with an additional $1.0 billion in upfront proceeds from PTC518 collaboration agreement with Novartis following closing.
- PTC submitted four regulatory approval applications to FDA:
  - Kebilidi ™ (eladocagene exuparvovec-tneq) gene therapy for AADC deficiency, approved in November 2024.
  - Sepiapterin for children and adults with PKU, accepted with an FDA action date of July 29, 2025.
- Vatiquinone for children and adults with FA, with filing acceptance decision anticipated Q1 2025.
- Translarna for nmDMD, accepted with no action date provided.
- PTC signed a global license and collaboration agreement with Novartis for the research, development and commercialization of PTC518 for HD, which has now closed. Key aspects of the transaction include the following:
  - PTC to receive $1.0 billion in upfront proceeds following closing.
  - PTC is eligible to receive up to $1.9 billion in development, regulatory and sales milestones.
  - PTC to receive 40% profit share on U.S. sales, and double-digit tiered royalties on ex-U.S. sales.
  - Novartis will assume global development, manufacturing and commercial responsibilities following the completion of the placebo-controlled portion of PIVOT-HD, which is expected in 1H 2025.
- PTC received $150 million from the sale of the Rare Pediatric Disease PRV it received with FDA approval of Kebilidi.
- In December 2024, PTC had a Type C meeting with FDA to discuss the potential for HTT lowering to serve as a surrogate endpoint to support accelerated approval for PTC518 for HD. The Agency was aligned with the scientific rationale for HTT lowering as a potential surrogate endpoint and asked that PTC provide additional clinical data, such as those being collected in PIVOT-HD, to show associations between HTT lowering and changes in clinical outcome measures.
- **Potential Key Clinical and Regulatory Events:**
- CHMP opinion on sepiapterin MAA expected in Q2 2025.
- FDA approval decision on sepiapterin NDA expected July 29, 2025.
- Results from the PIVOT-HD Phase 2 study of PTC518 expected in Q2 2025.
- NDA acceptance of vatiquinone expected in Q1 2025, with potential regulatory approval in 2H 2025.

Unaudited 2024 Financial Results:

- Total unaudited net revenue for full-year 2024 was approximately $814 million.
- Total unaudited net product revenue for full-year 2024 was approximately $601 million.
- DMD franchise unaudited revenue for full-year 2024 was approximately $547 million, including unaudited net product revenue for Translarna of approximately $340 million and for Emflaza of approximately $207 million.
- PTC expects to report approximately $211 million of full-year 2024 royalty revenue associated with Evrysdi®.

PTC is finalizing its financial results for the 2024 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2024, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on internal estimates.

2025 Financial Guidance:

- PTC anticipates total revenues for the full-year 2025 to be between $600 million and $800 million, including in-line products, potential new product launches and royalty revenue from Evrysdi.
- PTC anticipates GAAP R&D and SG&A expense for the full-year 2025 to be between $805 million and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full-year 2025 to be between $730 million and $760 million, excluding estimated non-cash, stock-based compensation expense of $75 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 2025 R&D and SG&A expense
(In millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 805	$ 835
Less: projected non-cash, stock-based compensation expense	75	75
Projected non-GAAP R&D and SG&A expense	$ 730	$ 760

Acronyms:

AADC: Aromatic l-Amino Acid Decarboxylase
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
FA: Friedreich's ataxia
FDA: Food and Drug Administration
HD: Huntington's Disease
MAA: Marketing Authorization Application
NDA: New Drug Application
nmDMD: nonsense mutation Duchenne Muscular Dystrophy
PKU: Phenylketonuria
PRV: Priority Review Voucher
R&D: Research and Development
SG&A: Selling, General and Administrative

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living 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. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and X.

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Forward-Looking Statement
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 "2025 Financial Guidance", including with respect to (i) 2025 total revenue guidance and (ii) 2025 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, commercialization and other matters with respect to 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: 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; 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 Commission adopts the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) for the conditional marketing authorization for Translarna in the EEA, or PTC's ability to identify other potential mechanisms by which it may provide Translarna to nmDMD patients in the EEA; PTC's ability to use the clinical data from its international drug registry study and real-world evidence concerning Translarna's benefits to support a continued marketing authorization for Translarna for the treatment of nmDMD in the EEA; PTC's ability to use 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, and from its international drug registry study to support a marketing approval for Translarna for the treatment of nmDMD in the United States; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from its trials in Translarna; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation including its right to receive any upfront payment, development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; expectations with respect to Kebilidi and Upstaza, including commercialization, manufacturing capabilities, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to sepiapterin, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; 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 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; 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, Kebilidi, Upstaza, Evrysdi, Tegsedi, Waylivra, sepiapterin or vatiqunone.

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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