PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 42nd Annual J.P. Morgan Healthcare Conference

January 8, 2024

– Unaudited 2023 total revenue of $946 million, representing 35% year-over-year growth –

– Regulatory filings in the EU and US for sepiapterin in PKU, a potential $1 billion global commercial opportunity, remain on track for 2024 –

– Multiple study readouts planned for 2024, including 12-month interim data from the PIVOT-HD study of PTC518 in HD patients –

SOUTH PLAINFIELD, N.J., Jan. 8, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 42nd Annual J.P. Morgan Healthcare Conference today, Monday, January 8, at 10:30am EST/7:30am PST. Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2023 accomplishments and highlight potential 2024 value-creating milestones. Preliminary 2023 unaudited financial results and 2024 financial guidance will also be provided. The presentation is being webcast live on the Events and Presentations page of the Investors section of PTC Therapeutics website at www.ptcbio.com.

"2023 was a transformational year for PTC as we made necessary and important changes to become a leaner, more focused and financially stronger company positioned for future success," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "We look forward to a successful 2024, with anticipated key global regulatory submissions for our PKU program and multiple data readouts, including from our PTC518 HD program."

Key 2023 Corporate Highlights:

- Unaudited net product revenue of $661 million in 2023 representing 23% year-over-year growth.
  - Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $355 million for Translarna™ (ataluren) and $255 million for Emflaza® (deflazacort) in 2023.
    - Translarna growth was driven by new patients in existing geographies and continued geographic expansion.
    - Emflaza growth was due to continued new prescriptions, high compliance, and more favorable access.
- PTC announced positive readouts from key programs in its clinical pipeline in 2023:
  - Highly statistically significant and clinically meaningful results in the Phase 3 registration-directed APHENITY clinical trial of sepiapterin in adult and pediatric patients with PKU.
  - Achievement of all key objectives of the readout of 12-week interim data in the PIVOT-HD study of PTC518 in HD.
- PTC announced strategic portfolio prioritizations, which resulted in reductions in both operating expenses and headcount of approximately 25% and 30%, respectively.
- In October, PTC finalized a royalty agreement, in which Royalty Pharma acquired additional royalties of Evrysdi for $1.0 billion upfront. The agreement included options for PTC to sell the remainder of its royalties of Evrysdi for up to $500 million or for Royalty Pharma to acquire half of such retained royalties for up to $250 million at a later date, less royalties received by PTC. PTC maintains all economics associated with up to $250 million in the remaining commercial sales milestones associated with Evrysdi global net sales. The proceeds from the financing were used to retire all outstanding debt obligations with Blackstone Life Sciences.

2024 Potential Key Clinical and Regulatory Events:

- Submission of a MAA to the EMA for sepiapterin for the treatment of PKU is expected in the first quarter.
- Submission of an NDA to the FDA for sepiapterin for the treatment of PKU is expected no later than the third quarter.
- Results from 12-month interim data from the PIVOT-HD trial of PTC518 in HD patients are expected in the second quarter.
- Submission of a BLA to the FDA for Upstaza for the treatment of AADC deficiency is expected in the first quarter.
- Topline results from the CardinALS trial of utreloxastat in ALS are expected in the fourth quarter.
- PTC expects the CHMP opinion from the re-examination procedure of the negative opinion on the Translarna conditional marketing authorization renewal in late January 2024, with ratification of that opinion by the European Commission 67 days later.
- FDA meeting for Translarna to align on the specific contents of a potential NDA resubmission is scheduled for the first quarter.
- FDA meeting for vatiquinone to discuss how the MOVE-FA data along with additional clinical and preclinical data could support an NDA submission in FA is scheduled for the first quarter.
- Scientific advice feedback from the EMA on a potential submission of vatiquinone for conditional marketing authorization for Friedreich ataxia is expected in the first quarter.

Unaudited 2023 Financial Results:

- Total unaudited net revenue for full-year 2023 was approximately $946 million.
- Total unaudited net product revenue for full-year 2023 was approximately $661 million.
- DMD franchise unaudited revenue for full-year 2023 was approximately $610 million, including unaudited net product revenue for Translarna of approximately $355 million and for Emflaza of approximately $255 million.
- PTC expects to report approximately $278 million for full-year 2023 collaboration and royalty revenue associated with Evrysdi.

PTC is currently in the process of finalizing its financial results for the 2023 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2023, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on internal estimates.

2024 Financial Guidance:

- PTC anticipates total revenues for the full-year 2024 to be between $600 million and $850 million.
- PTC anticipates GAAP R&D and SG&A expense for the full-year 2024 to be between $740 and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2024 to be between $660 and $755 million, including expected R&D expense milestone payments of up to $65 million and excluding estimated non-cash, stock-based compensation expense of $80 million.
- PTC anticipates up to $90 million of payments for the full year 2024 upon achievement of potential regulatory success-based milestones from previous acquisitions, of which up to $65 million will be recorded as R&D operating expense.

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 2024 R&D and SG&A Expense
(In millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 740	$ 835
Less: projected non-cash, stock-based compensation expense	80	80
Projected non-GAAP R&D and SG&A expense	**$ 660**	**$ 755**

PTC Therapeutics, Inc.
Reconciliation of GAAP Milestone Payments Full Year 2024
(in millions)

Projected GAAP R&D Expense Related Milestone Payments	$ 65
Projected GAAP Contingent Consideration Payable Related Milestone Payments	25
Total Projected GAAP Milestone Payments	**$ 90**

Acronyms:
AADC: Aromatic l-Amino Acid Decarboxylase
ALS: Amyotrophic Lateral Sclerosis
BLA: Biologics License Application
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EMA: European Medicines Agency
FDA: Food and Drug Administration
MAA: Marketing Authorization Application
NDA: New Drug Application
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy

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.

For More Information:

Investors
Kylie O'Keefe
+1 (908) 300-0691
kokeefe@ptcbio.com

Media
Jeanine Clemente
+1 (908) 912-9406
jclemente@ptcbio.com

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 "2024 Financial Guidance", including with respect to (i) 2024 total revenue guidance, (ii) 2024 GAAP and non-GAAP R&D and SG&A expense guidance and (iii) 2024 acquisition related milestone payment 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; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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 Medicines Agency (EMA) determines in the re-examination process that the benefit-risk balance of Translarna authorization supports renewal of such authorization; 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, which was a specific obligation to continued marketing authorization in the EEA, to support a renewal of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA; PTC's ability to utilize results from Study 041 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 our trials in Translarna; expectations with respect to Upstaza, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities, 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; expectations with respect to the commercialization of Tegsedi and Waylivra; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and early research programs in gene therapy and reductions in workforce, which may be in different periods and may be materially higher than estimated; the savings that may result from the discontinuation of PTC's strategic pipeline prioritization and reductions in workforce, which may be materially less than expected; 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; the potential financial impact and benefits of PTC's leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of its lease agreements, including 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, 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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