PTC Therapeutics Provides Corporate Update and Reports Fourth Quarter and Full Year 2024 Financial Results

February 27, 2025

– Full year 2024 revenue of $807 million, exceeding guidance –

– All 2024 clinical and regulatory milestones were achieved on schedule, including four NDA submissions, all of which were accepted for filing –

– License and collaboration agreement with Novartis for PTC518 Huntington's disease program closed in January 2025 –

– Cash of over $2.0 billion as of January 2025 –

WARREN, N.J., Feb. 27, 2025 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the fourth quarter ending December 31, 2024.

"Our strong fourth quarter rounds out a year of significant accomplishment across every part of our company," said Matthew B. Klein, M.D., Chief Executive Officer. "In 2024, our commercial team delivered another outstanding performance, we achieved all clinical and regulatory milestones on schedule and we solidified our balance sheet. We now have over $2 billion in cash to support our planned commercial and R&D activities in 2025 and beyond. With the many accomplishments of 2024 and our team's demonstrated ability to execute across every part of the business, we have built a strong foundation for continued success."

Key Corporate Updates:

- Fourth quarter 2024 total revenue of $213 million
- Fourth quarter 2024 revenue for the DMD franchise of $144 million, including net product revenue for Translarna ™ of $94 million and for Emflaza® of $50 million
- Sold Rare Disease PRV received with FDA approval of Kebilidi ™ for $150 million
- License and collaboration agreement signed with Novartis for PTC518 Huntington's disease program, closed in January 2025

Key Clinical and Regulatory Milestones:

- PTC submitted four regulatory approval applications to FDA in 2024, all of which have been accepted for review:
  - Kebilidi (eladocagene exuparvovec-tneq) gene therapy for the treatment of AADC deficiency in the U.S., approved in November 2024
  - Sepiapterin for children and adults with PKU, with a target regulatory action date of July 29, 2025
  - Vatiquinone for children and adults with Friedreich's ataxia, granted priority review with a target regulatory action date of August 19, 2025
  - Translarna for nmDMD
- PTC submitted several additional marketing authorization applications outside the U.S. for sepiapterin in 2024, with CHMP opinion on sepiapterin MAA expected in Q2 2025 and a regulatory decision in Japan expected in Q4 2025.
- Type C meeting with FDA held in December 2024 to discuss HTT lowering as potential 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.
- 12-month results from the PIVOT-HD Phase 2 study of PTC518 expected in Q2 2025.

Fourth Quarter and Full Year 2024 Financial Highlights:

- Total revenues were $213.2 million for the fourth quarter of 2024, compared to $307.1 million for the fourth quarter of 2023. Total revenue was $806.8 million for full year 2024, compared to $937.8 million for full year 2023.
- Total revenue includes net product revenue across the commercial portfolio of $154.7 million for the fourth quarter of 2024 and $601.0 million for full year 2024, compared to $155.1 million for the fourth quarter of 2023 and $661.2 million for full year 2023. Total revenue also includes royalty, collaboration, and manufacturing revenue of $58.5 million in the fourth quarter of 2024 and $205.8 million for full year 2024, compared to $152.0 million for the fourth quarter of 2023 and $276.6 million for full year 2023.
- Translarna net product revenues were $93.7 million for the fourth quarter of 2024, compared to $75.2 million for the fourth quarter of 2023. Translarna net product revenues were $339.9 million for full year 2024, compared to $355.8 million for full year 2023.
- Emflaza net product revenues were $50.5 million for the fourth quarter of 2024, compared to $67.4 million for the fourth quarter of 2023. Emflaza net product revenues were $207.2 million for full year 2024, compared to $255.1 million for full year 2023. These results were driven by the expiration of Emflaza's orphan drug exclusivity in February 2024.

- Roche reported Evrysdi® full year 2024 sales of approximately 1,631 CHF million, resulting in royalty revenue of $203.9 million to PTC for full year 2024, as compared to $168.9 million for full year 2023. Also in the fourth quarter of 2023, PTC recorded a sales milestone of $100.0 million for the achievement of $1.5 billion in worldwide annual net sales from Evrysdi. This sales milestone was recorded as collaboration revenue.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $124.8 million for the fourth quarter of 2024, compared to $121.4 million for the fourth quarter of 2023. GAAP R&D expenses were $534.5 million for full year 2024, compared to $666.6 million for full year 2023. The slight increase in R&D expense for the fourth quarter of 2024 reflects additional costs due to manufacturing, regulatory filings, and inspections primarily related to Kebilidi and sepiapterin. The decrease in R&D expense for full year 2024 relates to decreases in program spend related to our strategic portfolio prioritization as we continue to focus our resources on our differentiated, high potential research and development programs. R&D expense also included a total of $65.0 million regulatory success-based milestones paid to the former Censa securityholders for the year ended December 31, 2024, as compared to a $30.0 million success-based development milestone payable to the former Censa securityholders for the year ended December 31, 2023.
- Non-GAAP R&D expenses were $116.0 million for the fourth quarter of 2024, excluding $8.8 million in non-cash, stock-based compensation expense, compared to $113.2 million for the fourth quarter of 2023, excluding $8.1 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $497.9 million for full year 2024, excluding $36.6 million in non-cash, stock-based compensation expense, compared to $613.6 million for full year 2023, excluding $52.9 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $84.7 million for the fourth quarter of 2024, compared to $76.3 million for the fourth quarter of 2023. GAAP SG&A expenses were $300.9 million for full year 2024, compared to $332.5 million for full year 2023. The increase in SG&A expense for the fourth quarter of 2024 reflects our continued investment to support commercial activities, including expanding our commercial portfolio. The decrease in SG&A expense for full year 2024 was primarily due to lower employee costs as a result of the reduction in the workforce in 2023.
- Non-GAAP SG&A expenses were $76.3 million for the fourth quarter of 2024, excluding $8.4 million in non-cash, stock-based compensation expense, compared to $67.9 million for the fourth quarter of 2023, excluding $8.4 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $262.9 million for full year 2024, excluding $38.0 million in non-cash, stock-based compensation expense, compared to $283.8 million for full year 2023, excluding $48.7 million in non-cash, stock-based compensation expense.
- The change in the fair value of deferred and contingent consideration was a gain of $10.2 million for the fourth quarter of 2024, compared to a gain of $2.7 million for the fourth quarter of 2023. Change in the fair value of deferred and contingent consideration was a gain of $4.5 million for full year 2024, compared to a gain of $127.7 million for full year 2023. The full year 2024 change is primarily related to the Company's strategic portfolio prioritization and decision to discontinue its preclinical and early research programs in its gene therapy platform in May 2023, which included programs for FA and Angelman syndrome.
- The intangible asset impairment was $159.5 million for the fourth quarter and full year 2024, compared to $0.0 million for fourth quarter and $217.8 million for the full year 2023, which represented a non-cash charge. For the fourth quarter and full year 2024, we impaired $159.5 million related to a decrease in projected cash flows due to refinements in current market assumptions and the timing of patient treatments for AADC. For the full year 2023, we fully impaired the FA and Angelman syndrome intangible assets and recorded impairment expense of $217.8 million.
- Net loss was $65.9 million for the fourth quarter of 2024, compared to net loss of $155.8 million for the fourth quarter of 2023. Net loss was $363.3 million for full year 2024, compared to net loss of $626.6 million for full year 2023.
- Cash, cash equivalents, and marketable securities was $1,139.7 million on December 31, 2024, compared to $876.7 million on December 31, 2023.
- Shares issued and outstanding as of December 31, 2024, were 77,704,188.

PTC Full Year 2025 Financial Guidance:

- PTC anticipates total revenues for 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 expenses for full year 2025 to be between $805 million and $835 million.
- PTC anticipates non-GAAP R&D and SG&A expenses for 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 of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP R&D and SG&A expense 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.
Consolidated Statements of Operations

(In thousands, except share and per share data)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2024	2023	2024	2023
Revenues:				
Net product revenue	154,706	$ 155,062	$ 600,951	$ 661,249
Collaboration revenue	304	100,024	304	100,030
Royalty revenue	58,162	50,999	203,864	168,856
Manufacturing revenue	-	971	1,661	7,687
Total revenues	213,172	307,056	806,780	937,822
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	16,283	29,118	57,398	65,486
Amortization of acquired intangible asset	3,307	77,174	60,738	222,635
Research and development (1)	124,770	121,353	534,480	666,563
Selling, general and administrative (2)	84,683	76,291	300,911	332,540
Change in the fair value of contingent consideration	(10,175)	(2,700)	(4,475)	(127,700)
Intangible asset impairment	159,548	-	159,548	217,800
Tangible asset impairment and losses (gains) on transactions, net	(2,855)	-	750	-
Total operating expenses	375,561	301,236	1,109,350	1,377,324
(Loss) income from operations	(162,389)	5,820	(302,570)	(439,502)
Interest expense, net	(41,060)	(44,274)	(166,993)	(129,180)
Other income, net	8,850	18,961	6,544	10,130
Gain on Sale of priority review voucher	99,900	-	99,900	-
Loss on extinguishment of debt	-	(137,558)	-	(137,558)
Loss before income tax benefit (expense)	(94,699)	(157,051)	(363,119)	(696,110)
Income tax benefit (expense)	28,813	1,259	(176)	69,506
Net loss attributable to common stockholders	$ (65,886)	$ (155,792)	$ (363,295)	$ (626,604)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	77,201,783	75,490,569	76,845,055	74,838,392
Net loss per share—basic and diluted (in dollars per share)	$ (0.85)	$ (2.06)	$ (4.73)	$ (8.37)
(1) Research and development reconciliation				
GAAP research and development	$ 124,770	$ 121,353	$ 534,480	$ 666,563
Less: share-based compensation expense	8,818	8,113	36,629	52,941
Non-GAAP research and development	$ 115,952	$ 113,240	$ 497,851	$ 613,622
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 84,683	$ 76,291	$ 300,911	$ 332,540
Less: share-based compensation expense	8,420	8,395	37,986	48,695
Non-GAAP selling, general and administrative	$ 76,263	$ 67,896	$ 262,925	$ 283,845

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets
(in thousands, except share data)

	December 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 1,139,696	$ 876,739
Total Assets	**$ 1,705,024**	**$ 1,895,698**
Total debt	$ 285,412	$ 284,213
Total deferred revenue	5,505	801
Total liability for sale of future royalties	2,081,776	1,814,097
Total liabilities	**$ 2,803,095**	**$ 2,714,253**
Total stockholders' deficit (77,704,188 and 75,708,889 common shares issued and outstanding at December 31, 2024 and December 31, 2023, respectively)	$ (1,098,071)	$ (818,555)
Total liabilities and stockholders' deficit	**$ 1,705,024**	**$ 1,895,698**

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
CHF: Confoederatio Helvetica Francs (Swiss francs)
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
FA: Friedreich's Ataxia
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's Disease
HTT: Huntingtin
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

Today's Conference Call and Webcast Reminder:
To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, X, 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 "PTC Full Year 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 development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; expectations with respect to Upstaza/Kebilidi, 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 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, Upstaza, Kebilidi, Evrysdi, Tegsedi, Waylivra, sepiapterin or vatiquinone.

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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