PTC Therapeutics Provides Corporate Update and Reports Third Quarter 2024 Financial Results

November 7, 2024

– Strong revenue performance, supporting increase in full-year revenue guidance to $750-800 million –

– Regulatory filings for sepiapterin, Translarna™ and AADC gene therapy under review by FDA –

– On track to achieve remaining 2024 clinical and regulatory milestones, including NDA submission for vatiquinone –

WARREN, N.J., Nov. 7, 2024 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ending September 30, 2024.

"I am proud of our team's strong execution," said Matthew Klein, M.D., Chief Executive Officer. "We continue to achieve excellent revenue performance allowing us to raise full-year revenue guidance. In addition, we have submitted three approval applications to FDA so far this year, all of which have been accepted for review, and plan a fourth submission for vatiquinone for Friedreich ataxia in December. We are on our way to accomplishing all of our 2024 objectives and are in a strong financial position to execute on our long-term strategic plans."

Key Corporate Updates:

- Third quarter 2024 total revenue of $197 million
- Third quarter 2024 revenue for the DMD franchise was $124 million
  - Translarna™ (ataluren) net product revenue was $72 million, driven by continued access for patients in existing geographies, continued geographic expansion, high compliance and adherence, and dose adjustments.
  - Emflaza® (deflazacort) net product revenue was $52 million, driven by new patient starts, continued brand loyalty, high compliance and adherence, and dose adjustments.

Key Clinical and Regulatory Milestones:

- PTC submitted an NDA to the FDA for sepiapterin for the treatment of PKU. The file was accepted for review, and the regulatory action date is set for July 29, 2025.
- PTC resubmitted the NDA for Translarna for the treatment of nmDMD; the file was accepted, and a regulatory action date was not provided.
- The BLA for PTC's gene therapy for AADC deficiency remains on schedule for a regulatory action date of November 13, 2024.
- PTC received Fast Track Designation for the PTC518 Huntington's disease program.
- PTC plans to submit an NDA for vatiquinone for the treatment of Friedreich ataxia in December 2024.
- PTC expects to share topline data for the CardinALS trial of utreloxastat for the treatment of ALS in the fourth quarter of 2024.

Third Quarter 2024 Financial Highlights:

- Total revenues were $196.8 million for the third quarter of 2024, compared to $196.6 million for the third quarter of 2023.
- Total revenue includes net product revenue across the commercial portfolio of $135.4 million for the third quarter of 2024, compared to $144.0 million for the third quarter of 2023. Total revenue also includes royalty and manufacturing revenue of $61.4 million in the third quarter of 2024, compared to $52.5 million for the third quarter of 2023.
- Translarna net product revenues were $72.3 million for the third quarter of 2024, compared to $69.0 million for the third quarter of 2023.
- Emflaza net product revenues were $51.9 million for the third quarter of 2024, compared to $67.4 million for the third quarter of 2023.
- Roche reported Evrysdi® 2024 year-to-date sales of approximately CHF 1.2 billion, resulting in royalty revenue of $61.4 million to PTC for the third quarter of 2024, compared to $50.2 million for the third quarter of 2023.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $161.4 million for the third quarter of 2024, compared to $164.2 million for the third quarter of 2023. The decrease in research and development expenses reflects strategic portfolio prioritization, as PTC continues to focus its resources on its differentiated, high-potential R&D programs. Included in third quarter 2024 R&D expense is $50.0 million in regulatory success-based milestones related to the Censa acquisition.
- Non-GAAP R&D expenses were $152.0 million for the third quarter of 2024, excluding $9.4 million in non-cash, stock-based compensation expense, compared to $150.2 million for the third quarter of 2023, excluding $14.0 million in

non-cash, stock-based compensation expense.

- GAAP SG&A expenses were $73.5 million for the third quarter of 2024, compared to $80.9 million for the third quarter of 2023. The decrease in selling, general and administrative expenses reflects lower employee costs as a result of the reduction in workforce in 2023.
- Non-GAAP SG&A expenses were $63.1 million for the third quarter of 2024, excluding $10.3 million in non-cash, stock-based compensation expense, compared to $67.9 million for the third quarter of 2023, excluding $13.0 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a loss of $0.7 million for the third quarter of 2024, compared to a loss of $1.5 million for the third quarter of 2023.
- Tangible asset impairment and losses (gains) on transactions, net, was a loss of $1.8 million for the third quarter of 2024, compared to $0.0 million for the third quarter of 2023. These results were primarily related to fixed asset impairments.
- Net loss was $106.7 million for the third quarter of 2024, compared to net loss of $133.0 million for the third quarter of 2023.
- Cash, cash equivalents, and marketable securities was $1,013.4 million on September 30, 2024, compared to $876.7 million on December 31, 2023.
- Shares issued and outstanding as of September 30, 2024, were 76,952,124.

PTC Updates Full Year 2024 Financial Guidance:

- PTC anticipates total revenues for full year 2024 to be between $750 million and $800 million.
- PTC anticipates GAAP R&D and SG&A expenses for full year 2024 to be between $740 million and $835 million, including expected R&D expense milestone payments of up to $65 million.
- PTC anticipates non-GAAP R&D and SG&A expenses for full year 2024 to be between $660 million 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 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 expenses. As of September 30, 2024, $85 million in milestones have been achieved, of which $65 million has been recorded as R&D operating expenses and $20 million as contingent consideration payable.

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 September 30,		Nine Months Ended September 30,	
	2024	**2023**	**2024**	**2023**
Revenues:				
Net product revenue	$ 135,421	$ 144,038	$ 446,245	$ 506,187
Collaboration revenue	-	-	-	6
Royalty revenue	61,365	50,173	145,702	117,857
Manufacturing revenue	-	2,365	1,661	6,716
Total revenues	196,786	196,576	593,608	630,766
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	10,848	9,493	41,115	36,368
Amortization of acquired intangible asset	3,036	58,649	57,431	145,461
Research and development (1)	161,412	164,212	409,710	545,210
Selling, general and administrative (2)	73,456	80,886	216,228	256,249
Change in the fair value of deferred and contingent consideration	700	1,500	5,700	(125,000)
Intangible asset impairment	-	-	-	217,800

Tangible asset impairment and losses (gains) on transactions, net	1,844	-	3,605	-
Total operating expenses	251,296	314,740	733,789	1,076,088
Loss from operations	(54,510)	(118,164)	(140,181)	(445,322)
Interest expense, net	(41,609)	(28,160)	(125,933)	(84,905)
Other expense, net	(1,872)	(20,266)	(2,306)	(8,832)
Loss before income tax (expense) benefit	(97,991)	(166,590)	(268,420)	(539,059)
Income tax (expense) benefit	(8,663)	33,620	(28,989)	68,247
Net loss attributable to common stockholders	$ (106,654)	$ (132,970)	$ (297,409)	$ (470,812)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	76,925,523	75,377,997	76,716,340	74,618,611
Net loss per share—basic and diluted (in dollars per share)	$ (1.39)	$ (1.76)	$ (3.88)	$ (6.31)
(1) Research and development reconciliation				
GAAP research and development	$ 161,412	$ 164,212	$ 409,710	$ 545,210
Less: share-based compensation expense	9,416	13,986	27,810	44,828
Non-GAAP research and development	$ 151,996	$ 150,226	$ 381,900	$ 500,382
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 73,456	$ 80,886	$ 216,228	$ 256,249
Less: share-based compensation expense	10,339	12,956	29,566	40,300
Non-GAAP selling, general and administrative	**$** 63,117	$ 67,930	$ 186,662	$ 215,949

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

	September 30, 2024	**December 31, 2023**
Cash, cash equivalents and marketable securities	$ 1,013,357	$ 876,739
Total assets	**$ 1,842,236**	**$ 1,895,698**
Total debt	$ 285,106	$ 284,213
Total deferred revenue	-	801
Total liability for sale of future royalties	2,082,051	1,814,097
Total liabilities	**$ 2,896,588**	**$ 2,714,253**
Total stockholders' deficit (76,952,124 and 75,708,889 common shares issued and outstanding at September 30, 2024 and December 31, 2023, respectively)	$ (1,054,352)	$ (818,555)
Total liabilities and stockholders' deficit	**$ 1,842,236**	**$ 1,895,698**

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

	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

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

Acronyms:
AADC: Aromatic l-Amino Acid Decarboxylase
ALS: Amyotrophic Lateral Sclerosis
BLA: Biologics License Application
CHF: Confoederatio Helvetica Francs (Swiss francs)
DMD: Duchenne Muscular Dystrophy
FA: Friedreich Ataxia
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's Disease
NDA: New Drug Application
nmDMD: Nonsense Mutation Duchenne Muscular Dystrophy
PKU: Phenylketonuria
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 Updates Full Year 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 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 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 sepiapterin, including any regulatory submissions and potential approvals, commercialization, 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 PTC's 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; 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, Evrysdi, Tegsedi, Waylivra or sepiapterin.

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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