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

August 8, 2024

– Continued strong revenue performance –

– NDAs for sepiapterin and Translarna™ submitted to FDA –

– Positive interim data readout from PTC518 PIVOT-HD study –

– On track to achieve remaining 2024 clinical and regulatory milestones –

WARREN, N.J., Aug. 8, 2024 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ending June 30, 2024.

"I am proud of our team's continued outstanding execution as we have accomplished all our objectives so far this year," said Matthew Klein, M.D., Chief Executive Officer. "We are in a strong cash position, submitted three drug approval applications to the FDA and remain on schedule to achieve the many milestones we have set for the remainder of 2024."

Key Corporate Updates:

- Second quarter 2024 total revenue of $187 million
- Second quarter 2024 revenue for the DMD franchise was $118 million
    - Translarna™ (ataluren) net product revenue was $70 million, driven by continued access for patients in existing geographies and continued geographic expansion.
    - Emflaza® (deflazacort) net product revenue was $47 million, driven by new patient starts and continued brand loyalty.

Key Clinical and Regulatory Milestones:

- PTC submitted an NDA to the FDA for sepiapterin for the treatment of PKU in July 2024. Further regulatory submissions are planned for Japan and Brazil later in 2024.
- PTC resubmitted the NDA for Translarna for the treatment of nmDMD in July 2024.
- PTC achieved all objectives in the interim readout of the Phase 2 PIVOT-HD study of PTC518 in Huntington's disease patients, which were disclosed in the second quarter.
- PTC plans to submit an NDA for vatiquinone for the treatment of Friedreich ataxia in late 2024.
- PTC expects to share topline data for the CardinALS trial of utreloxastat for the treatment of ALS in the fourth quarter of 2024.
- The BLA for PTC's gene therapy for AADC deficiency was accepted by FDA with priority review; the target regulatory action date is November 13, 2024.

Second-Quarter 2024 Financial Highlights:

- Total revenues were $186.7 million for the second quarter of 2024, compared to $213.8 million for the second quarter of 2023.
- Total revenues include net product revenue across the commercial portfolio of $133.2 million for the second quarter of 2024, compared to $174.6 million for the second quarter of 2023. Total revenues also include royalty and manufacturing revenue of $53.5 million for the second quarter of 2024, compared to $39.2 million for the second quarter of 2023.
- Translarna net product revenues were $70.4 million for the second quarter of 2024, compared to $96.5 million for the second quarter of 2023. These results were due to the timing of bulk government orders.
- Emflaza net product revenues were $47.3 million for the second quarter of 2024, compared to $65.7 million for the second quarter of 2023. These results were driven by the expiration of Emflaza's orphan drug exclusivity in February 2024.
- Roche reported Evrysdi 2024 year-to-date sales of approximately CHF 838 million, resulting in royalty revenue of $53.2 million to PTC for the second quarter of 2024, as compared to $36.9 million for the second quarter of 2023.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $132.2 million for the second quarter of 2024, compared to $185.9 million for the second quarter of 2023. The decrease in quarterly research and development expenses reflects strategic portfolio prioritization as PTC continues to focus its resources on its differentiated, high-potential programs. Included in second quarter 2024 R&D expense is a $15.0 million regulatory

success-based milestone related to the Censa acquisition.

- Non-GAAP R&D expenses were $122.7 million for the second quarter of 2024, excluding $9.4 million in non-cash, stock-based compensation expense, compared to $170.3 million for the second quarter of 2023, excluding $15.5 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $69.5 million for the second quarter of 2024, compared to $88.4 million for the second quarter of 2023. The decrease in quarterly selling, general and administrative expenses reflects lower employee costs due to the reduction in workforce in 2023.
- Non-GAAP SG&A expenses were $59.7 million for the second quarter of 2024, excluding $9.8 million in non-cash, stock-based compensation expense, compared to $74.6 million for the second quarter of 2023, excluding $13.8 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a loss of $5.1 million for the second quarter of 2024, compared to a gain of $128.9 million for the second quarter of 2023. The change in fair value of contingent consideration is primarily related to the strategic portfolio prioritization and the decision to discontinue the Friedreich ataxia and Angelman syndrome gene therapy programs in the second quarter of 2023. As a result, PTC determined that the fair value for all of the contingent consideration payable related to Friedreich ataxia and Angelman syndrome was $0 and recorded a gain of $129.8 million.
- Tangible asset impairment and losses (gains) on transactions, net, was a loss of $1.8 million for the second quarter of 2024, compared to $0 million for the second quarter of 2023. These results were due to the sale of certain assets related to gene therapy manufacturing and fixed asset impairments, partially offset by a gain on lease terminations and a gain on sales of certain fixed assets.
- Net loss was $99.2 million for the second quarter of 2024, compared to net loss of $198.9 million for the second quarter of 2023.
- Cash, cash equivalents, and marketable securities was $1,093.3 million on June 30, 2024, compared to $876.7 million on December 31, 2023. During the second quarter of 2024, PTC amended its agreement with Royalty Pharma and exercised one of its put options in exchange for $250 million in cash, less royalties received.
- Shares issued and outstanding as of June 30, 2024, were 76,900,123.

PTC Updates Full Year 2024 Financial Guidance:

- PTC anticipates total revenues for full year 2024 to be between $700 million and $750 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 June 30, 2024, $35 million in milestones have been achieved, of which $15 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 June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Revenues:				
Net product revenue	$ 133,220	$ 174,592	$ 310,824	$ 362,149
Collaboration revenue	-	-	-	6
Royalty revenue	53,183	36,853	84,337	67,684
Manufacturing revenue	301	2,363	1,661	4,351
Total revenues	186,704	213,808	396,822	434,190

Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	15,527	12,731	30,267	26,875
Amortization of acquired intangible asset	2,865	47,397	54,395	86,812
Research and development (1)	132,169	185,874	248,298	380,998
Selling, general and administrative (2)	69,500	88,449	142,772	175,363
Change in the fair value of deferred and contingent consideration	5,100	(128,900)	5,000	(126,500)
Intangible asset impairment	-	217,800	-	217,800
Tangible asset impairment and losses (gains) on transactions, net	1,761	-	1,761	-
Total operating expenses	226,922	423,351	482,493	761,348
Loss from operations	(40,218)	(209,543)	(85,671)	(327,158)
Interest expense, net	(43,490)	(29,415)	(84,324)	(56,745)
Other (expense) income, net	(2,025)	1,479	(434)	11,434
Loss before income tax (expense) benefit	(85,733)	(237,479)	(170,429)	(372,469)
Income tax (expense) benefit	(13,446)	38,596	(20,326)	34,627
Net loss attributable to common stockholders	$ (99,179)	$ (198,883)	$ (190,755)	$ (337,842)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	76,725,070	74,730,433	76,610,598	74,232,624
Net loss per share—basic and diluted (in dollars per share)	$ (1.29)	$ (2.66)	$ (2.49)	$ (4.55)
(1) Research and development reconciliation				
GAAP research and development	$ 132,169	$ 185,874	$ 248,298	$ 380,998
Less: share-based compensation expense	9,428	15,529	18,395	30,842
Non-GAAP research and development	$ 122,741	$ 170,345	$ 229,903	$ 350,156
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 69,500	$ 88,449	$ 142,772	$ 175,363
Less: share-based compensation expense	9,815	13,842	19,226	27,344
Non-GAAP selling, general and administrative	$ 59,685	$ 74,607	$ 123,546	$ 148,019

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

	June 30, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 1,093,293	$ 876,739
Total Assets	**$ 1,916,355**	**$ 1,895,698**
Total debt	$ 284,806	$ 284,213
Total deferred revenue	-	801
Total liability for sale of future royalties	2,084,880	1,814,097
Total liabilities	**$ 2,896,536**	**$ 2,714,253**
Total stockholders' deficit (76,900,123 and 75,708,889 common shares issued and outstanding at June 30, 2024 and December 31, 2023, respectively)	$ (980,181)	$ (818,555)
Total liabilities and stockholders' deficit	**$ 1,916,355**	**$ 1,895,698**

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

	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**

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
EMA: European Medicines Agency
FA: Friedreich Ataxia
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's Disease
MAA: Marketing Authorization Application
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 patients 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, on Twitter at @PTCBio, and on 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 Revenue 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 for the conditional marketing authorization for Translarna supports renewal of such authorization, 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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