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

April 25, 2024

– Strong revenue performance across product portfolio –

– Submitted MAA for sepiapterin and BLA for Upstaza –

– On target to achieve remaining 2024 clinical and regulatory milestones, including global submissions for sepiapterin –

WARREN, N.J., April 25, 2024 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ending March 31, 2024.

"We are off to a strong start in 2024, with outstanding commercial performance and achievement of all planned clinical and regulatory milestones for the first quarter," said Matthew Klein, M.D., Chief Executive Officer, PTC Therapeutics, Inc. "We remain on track to achieve the many planned 2024 clinical and regulatory milestones, including global regulatory submissions for sepiapterin."

Key Corporate Updates:

- First quarter 2024 total revenue of $210 million
- First quarter 2024 revenue for the DMD franchise was $161 million
  - Translarna™ (ataluren) net product revenue was $104 million, driven by new patients in existing geographies and continued geographic expansion.
  - Emflaza® (deflazacort) net product revenue was $57 million, resulting from new patient starts and high compliance.

Key Clinical and Regulatory Milestones:

- PTC submitted an MAA to the EMA for sepiapterin for the treatment of PKU in March 2024. The company expects to submit an NDA to the FDA for sepiapterin no later than the third quarter of 2024 and to complete regulatory submissions in Japan and Brazil in 2024.
- PTC submitted a BLA to the FDA for Upstaza™ for the treatment of AADC deficiency in March 2024.
- Based on FDA feedback, PTC plans to resubmit the NDA for Translarna for the treatment of nmDMD in mid-2024.
- Based on FDA feedback, PTC plans to submit an NDA for vatiquinone for the treatment of FA in late 2024.
- The interim data update for the PIVOT-HD trial of PTC518 for HD patients remains on schedule for the second quarter of 2024. This update will include 12-month data on the initial group of subjects for whom data were reported in June 2023, as well as 12-week data on a larger number of stage 2 and stage 3 HD patients.
- PTC expects to report topline data for the CardinALS trial of utreloxastat for ALS in the fourth quarter of 2024.

First Quarter 2024 Financial Highlights:

- Total revenues were $210.1 million for the first quarter of 2024, compared to $220.4 million for the first quarter of 2023.
- Total revenue includes net product revenue across the commercial portfolio of $177.6 million for the first quarter of 2024, compared to $187.6 million for the first quarter of 2023. Total revenue also includes collaboration, royalty, and manufacturing revenue of $32.5 million in the first quarter of 2024, compared to $32.8 million for the first quarter of 2023.
- Translarna net product revenues were $103.6 million for the first quarter of 2024, compared to $115.1 million for the first quarter of 2023, driven by new patients in existing geographies and continued geographic expansion. The decrease was due to the timing of bulk patient orders.
- Emflaza net product revenues were $57.5 million for the first quarter of 2024, compared to $54.6 million for the first quarter of 2023. These results were driven by new patient starts and high compliance.
- Roche reported Evrysdi® 2024 year-to-date sales of approximately $400 million, resulting in royalty revenue of $31.2 million to PTC for the first quarter of 2024, as compared to $30.8 million for the first quarter of 2023.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $116.1 million for the first quarter of 2024, compared to $195.1 million for the first quarter of 2023. The decrease in research and development expenses reflects strategic portfolio prioritization as the Company continues to focus its resources on its differentiated, high-potential R&D programs.
- Non-GAAP R&D expenses were $107.2 million for the first quarter of 2024, excluding $9.0 million in non-cash, stock-based compensation expense, compared to $179.8 million for the first quarter of 2023, excluding $15.3 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $73.3 million for the first quarter of 2024, compared to $86.9 million for the first 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 $63.9 million for the first quarter of 2024, excluding $9.4 million in non-cash,

stock-based compensation expense, compared to $73.4 million for the first quarter of 2023, excluding $13.5 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a gain of $0.1 million for the first quarter of 2024, compared to a loss of $2.4 million for the first quarter of 2023.
- The net loss was $91.6 million for the first quarter of 2024, compared to a net loss of $139.0 million for the first quarter of 2023.
- Cash, cash equivalents, and marketable securities was $884.8 million on March 31, 2024, compared to $876.7 million on December 31, 2023.
- Shares issued and outstanding as of March 31, 2024, were 76,653,960.

PTC Reaffirms Full Year 2024 Financial Guidance:

- PTC anticipates total revenues for full year 2024 to be between $600 million and $680 million.
- PTC anticipates GAAP R&D and SG&A expense for full year 2024 to be between $740 and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for 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 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 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 March 31,	
	2024	**2023**
Revenues:		
Net product revenue	$ 177,604	$ 187,557
Collaboration revenue	-	6
Royalty revenue	31,154	30,831
Manufacturing revenue	1,360	1,988
Total revenues	210,118	220,382
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	14,740	14,144
Amortization of acquired intangible asset	51,530	39,415
Research and development (1)	116,129	195,124
Selling, general and administrative (2)	73,272	86,914
Change in the fair value of deferred and contingent consideration	(100)	2,400
Total operating expenses	255,571	337,997
Loss from operations	(45,453)	(117,615)
Interest expense, net	(40,834)	(27,331)
Other income, net	1,591	9,956
Loss before income tax expense	(84,696)	(134,990)
Income tax expense	(6,880)	(3,969)
Net loss attributable to common stockholders	$ (91,576)	$ (138,959)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	76,496,127	73,729,284
Net loss per share—basic and diluted (in dollars per share)	$ (1.20)	$ (1.88)
(1) Research and development reconciliation		
GAAP research and development	$ 116,129	$ 195,124
Less: share-based compensation expense	8,967	15,314

Non-GAAP research and development	$ 107,162	$ 179,810

(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 73,272	$ 86,914
Less: share-based compensation expense	9,411	13,501
Non-GAAP selling, general and administrative	$ 63,861	$ 73,413

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

	March 31, 2024	December 31, 2023
Cash, cash equivalents and marketable securities	$ 884,813	$ 876,739
Total assets	**$ 1,789,629**	**$ 1,895,698**
Total debt	$ 284,512	$ 284,213
Total deferred revenue	-	801
Total liability for sale of future royalties	1,838,933	1,814,097
Total liabilities	**$ 2,683,545**	**$ 2,714,253**
Total stockholders' deficit (76,653,960 and 75,708,889 common shares issued and outstanding at March 31, 2024, and December 31, 2023, respectively)	$ (893,916)	$ (818,555)
Total liabilities and stockholders' deficit	**$ 1,789,629**	**$ 1,895,698**

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

	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**

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 Reaffirms 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 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; 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 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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