PTC Therapeutics Provides Corporate Update and Reports Fourth Quarter and Full Year 2023 Financial Results

February 29, 2024

– 34% year-over-year growth in 2023 total revenue –

– Global filings of sepiapterin remain on track with first submission of the EU MAA expected in March –

– Potential NDA submission for vatiquinone for Friedreich ataxia expected in late 2024 –

SOUTH PLAINFIELD, N.J., Feb. 29, 2024 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the fourth quarter and full year ending December 31, 2023.

"We closed out 2023 with strong revenue performance in the fourth quarter," said Matthew Klein, M.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are well-positioned for a successful 2024 with several potential exciting clinical and regulatory milestones ahead. We look forward to initiating the global regulatory submissions for sepiapterin for the treatment of PKU, which we see as a potential billion-dollar opportunity, as well as to advancing our PTC518, vatiquinone, and utreloxastat programs."

Key Corporate Updates:

- 2023 total revenue of $938 million, representing 34% year-over-year growth
- 2023 revenue for the DMD franchise was $611 million
  - Translarna™ (ataluren) net product revenue was $356 million, driven by new patients in existing geographies and continued geographic expansion.
  - Emflaza® (deflazacort) net product revenue was $255 million, resulting from new patient starts and high compliance.

Key Clinical and Regulatory Milestones:

- PTC expects to submit an MAA to the EMA for sepiapterin for the treatment of PKU in March 2024 and expects to submit an NDA to the FDA for sepiapterin no later than the third quarter of 2024.
- PTC had a Type C meeting with the FDA in the first quarter of 2024 to discuss the vatiquinone Friedreich ataxia program. Based on discussions with the FDA, PTC has a potential path to an NDA submission in late 2024 based on the placebo-controlled results of MOVE-FA, along with data from the ongoing open-label extension study.
- PTC expects to submit a BLA to the FDA for Upstaza™ for the treatment of AADC deficiency in March 2024.
- PTC expects to meet with the FDA to discuss a potential NDA resubmission of Translarna in March 2024.
- PTC expects to provide an interim data update for the PIVOT-HD trial of PTC518 for Huntington's disease patients in the second quarter of 2024. This update will include 12-month data on the initial group of subjects for which data was reported in June 2023.
- PTC expects to report topline data for the CardinALS trial of utreloxastat for ALS in the fourth quarter of 2024.

Fourth Quarter and Full Year 2023 Financial Highlights:

- Total revenue was $307.1 million for the fourth quarter of 2023, compared to $167.4 million for the fourth quarter of 2022. Total revenue was $937.8 million for full year 2023, compared to $698.8 million for full year 2022.
- Total revenue included net product revenue across the commercial portfolio of $155.1 million for the fourth quarter of 2023 and $661.2 million for full year 2023, compared to $127.5 million for the fourth quarter of 2022 and $535.2 million for full year 2022. Total revenue also included collaboration, royalty, and manufacturing revenue of $152.0 million in fourth quarter of 2023 and $276.6 million for full year 2023, compared to $39.9 million for the fourth quarter of 2022 and $163.6 million for full year 2022.
- Translarna net product revenue was $75.2 million for the fourth quarter of 2023, compared to $55.8 million for the fourth quarter of 2022. Translarna net product revenue was $355.8 million for full year 2023, compared to $288.6 million for full year 2022. These results were driven by treatment of new patients in existing geographies and continued geographic expansion.
- Emflaza net product revenue was $67.4 million for the fourth quarter of 2023, compared to $58.1 million for the fourth quarter of 2022. Emflaza net product revenue was $255.1 million for full year 2023, compared to $218.3 million for full year 2022. These results were driven by new patient starts and high compliance.
- Roche reported Evrysdi® full year 2023 sales of approximately CHF 1,419 million, resulting in full year 2023 royalty

revenue of $168.9 million to PTC, as compared to $113.5 million for full year 2022. 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 expense was $121.4 million for the fourth quarter of 2023, compared to $188.7 million for the fourth quarter of 2022. GAAP R&D expense was $666.6 million for full year 2023, compared to $651.5 million for full year 2022. The decrease in R&D expense for the fourth quarter of 2023 reflects the strategic portfolio prioritization as the Company continues to focus its resources on its differentiated, high-potential R&D programs. The increase in R&D expense for full year 2023 is primarily due to the achievement of a $30.0 million success-based development milestone for the completion of enrollment of a Phase 3 clinical trial for sepiapterin for PKU, partially offset by the Company's strategic portfolio prioritization.
- Non-GAAP R&D expense was $113.2 million for the fourth quarter of 2023, excluding $8.1 million in non-cash, stock-based compensation expense, compared to $174.7 million for the fourth quarter of 2022, excluding $14.0 million in non-cash, stock-based compensation expense. Non-GAAP R&D expense was $613.6 million for full year 2023, excluding $52.9 million in non-cash, stock-based compensation expense, compared to $595.6 million for full year 2022, excluding $55.9 million in non-cash, stock-based compensation expense.
- GAAP SG&A expense was $76.3 million for the fourth quarter of 2023, compared to $92.7 million for the fourth quarter of 2022. GAAP SG&A expense was $332.5 million for full year 2023, compared to $326.0 million for full year 2022. The decrease in SG&A expense for the fourth quarter of 2023 was primarily due to lower employee costs as a result of the reduction in workforce. The increase in SG&A expense for full year 2023 reflected the Company's continued investment to support commercial activities, including the expanding commercial portfolio, and restructuring costs from the reduction in workforce in the year ended December 31, 2023.
- Non-GAAP SG&A expense was $67.9 million for the fourth quarter of 2023, excluding $8.4 million in non-cash, stock-based compensation expense, compared to $79.3 million for the fourth quarter of 2022, excluding $13.4 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expense was $283.8 million for full year 2023, excluding $48.7 million in non-cash, stock-based compensation expense, compared to $271.5 million for full year 2022, excluding $54.5 million in non-cash, stock-based compensation expense.
- The intangible asset impairment was $217.8 million for full year 2023, which represented a non-cash charge. This was a result of the Company's strategic portfolio prioritization and its decision to discontinue its preclinical and early research programs in its gene therapy platform, which included FA and AS, which was announced in May 2023. No intangible asset impairment was recorded in the fourth quarter of 2023. The intangible asset impairment was $33.4 million for the fourth quarter and full year 2022, which represented a non-cash charge related to a decrease in projected cash flows for Upstaza due to refinements in market assumptions.
- The change in the fair value of deferred and contingent consideration was a gain of $2.7 million for the fourth quarter of 2023, compared to a loss of $6.3 million for the fourth quarter of 2022. The change in the fair value of deferred and contingent consideration was a gain of $127.7 million for full year 2023, compared to a gain of $25.9 million for full year 2022. The change in the fair value of deferred and contingent consideration was primarily related to the fair valuation of potential future consideration to be paid to former equity holders of Agilis Biotherapeutics, Inc. (Agilis) in connection with PTC's acquisition of Agilis, which closed in August 2018. The Company's strategic portfolio prioritization and its decision to discontinue its preclinical and early research programs in its gene therapy platform, which included FA and AS, was announced in May 2023. As a result, PTC determined the fair value for all the contingent consideration payable related to FA and AS was $0.
- The loss on extinguishment of debt was $137.6 million for the fourth quarter of 2023 and full year 2023, compared to $0.0 million for the fourth quarter of 2022 and full year 2022. The increase was primarily due to the early termination of the Company's Blackstone Credit Agreement, which resulted in a loss on the extinguishment of debt of $92.7 million for the period ended December 31, 2023. In addition, the Company recorded a $44.9 million loss on extinguishment of debt for the period ended December 31, 2023, relating to the A&R Royalty Purchase Agreement, which represented a non-cash charge.
- The net loss was $155.8 million for the fourth quarter of 2023, compared to a net loss of $170.9 million for the fourth quarter of 2022. The net loss was $626.6 million for full year 2023, compared to a net loss of $559.0 million for full year 2022.
- Cash, cash equivalents, and marketable securities was $876.7 million on December 31, 2023, compared to $410.7 million at December 31, 2022.
- Shares issued and outstanding as of December 31, 2023, were 75,708,889.

PTC Updates 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 million and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense 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 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 December 31,		Twelve Months Ended December 31,	
	2023	**2022**	**2023**	**2022**
Revenues:				
Net product revenue	$ 155,062	$ 127,508	$ 661,249	$ 535,228
Collaboration revenue	100,024	28	100,030	50,052
Royalty revenue	50,999	39,876	168,856	113,521
Manufacturing revenue	971	-	7,687	-
Total revenues	307,056	167,412	937,822	698,801
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	29,118	10,893	65,486	44,678
Amortization of acquired intangible asset	77,174	35,764	222,635	116,554
Research and development (1)	121,353	188,694	666,563	651,496
Selling, general and administrative (2)	76,291	92,718	332,540	325,998
Change in the fair value of deferred and contingent consideration	(2,700)	6,300	(127,700)	(25,900)
Intangible asset impairment	-	33,384	217,800	33,384
Total operating expenses	301,236	367,753	1,377,324	1,146,210
Income (loss) from operations	5,820	(200,341)	(439,502)	(447,409)
Interest expense, net	(44,274)	(24,500)	(129,180)	(90,871)
Other income (expense), net	18,961	35,147	10,130	(49,207)
Loss on extinguishment of debt	(137,558)	-	(137,558)	-
Loss before income tax benefit	(157,051)	(189,694)	(696,110)	(587,487)
Income tax benefit	1,259	18,805	69,506	28,470
Net loss attributable to common stockholders	$ (155,792)	$ (170,889)	$ (626,604)	$ (559,017)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	75,490,569	72,656,790	74,838,392	71,728,634
Net loss per share—basic and diluted (in dollars per share)	$ (2.06)	$ (2.35)	$ (8.37)	$ (7.79)
(1) Research and development reconciliation				
GAAP research and development	$ 121,353	$ 188,694	$ 666,563	$ 651,496
Less: share-based compensation expense	8,113	13,973	52,941	55,869
Non-GAAP research and development	$ 113,240	$ 174,721	$ 613,622	$ 595,627
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 76,291	$ 92,718	$ 332,540	$ 325,998
Less: share-based compensation expense	8,395	13,370	48,695	54,464
Non-GAAP selling, general and administrative	$ 67,896	$ 79,348	$ 283,845	$ 271,534

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets

(in thousands, except share data)

	December 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 876,739	$ 410,705
Total Assets	**$ 1,895,698**	**$ 1,705,619**
Total debt	$ 284,213	$ 571,722
Total deferred revenue	801	1,351
Total liability for sale of future royalties	1,814,097	757,886
Total liabilities	**$ 2,714,253**	**$ 2,052,705**
Total stockholders' deficit (75,708,889 and 73,104,692 common shares issued and outstanding at December 31, 2023 and December 31, 2022, respectively)	$ (818,555)	$ (347,086)
Total liabilities and stockholders' deficit	**$ 1,895,698**	**$ 1,705,619**

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:
AS: Angelman Syndrome
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
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.

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 "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 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 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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