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

April 27, 2023

– First-quarter 2023 total revenue of $220 million, representing 48% year-over-year growth -

– Readouts from four clinical studies expected in second quarter of 2023, three of which are registration-directed –

SOUTH PLAINFIELD, N.J., April 27, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ending March 31, 2023.

"We are pleased to report strong revenue growth in the first quarter, with double-digit year-over-year revenue increases for all six of our marketed and partnered products," said Matthew Klein, M.D., Chief Executive Officer, PTC Therapeutics, Inc. "In addition, we remain on track to share readouts from four clinical studies, including three registration-directed studies, in the second quarter."

Key Corporate Updates:

- First quarter 2023 revenue for the Duchenne muscular dystrophy (DMD) franchise was $170 million, representing 33% year-over-year growth
  - Translarna™ (ataluren) quarterly net product revenue was $115 million, with growth coming from treatment of new patients, continued high compliance and continued geographic expansion
  - Emflaza® (deflazacort) quarterly net product revenue was $55 million, driven by new patients, high compliance and broader access

Key Clinical and Regulatory Updates:

- PTC continues to make progress in ongoing registration-directed clinical studies:
  - The APHENITY Phase 3 trial of sepiapterin (PTC923) for PKU, with results anticipated in May 2023
  - The MIT-E Phase 2/3 vatiquinone trial for mitochondrial disease associated seizures, with results anticipated in the second quarter of 2023
  - The MOVE-FA Phase 3 vatiquinone trial for Friedreich ataxia, with results anticipated in the second quarter of 2023
- Enrollment in the PIVOT-HD Phase 2 trial of PTC518 for Huntington's disease is active and ongoing at study sites in Europe and Australia. Interim data from the first 12 weeks of the placebo-controlled trial is anticipated in the second quarter of 2023.
- Given the anticipated imminent results from these studies, PTC will not be discussing these programs from April 28 until results are disclosed for each study.
- For Upstaza, the FDA requested additional bioanalytical data in support of comparability analyses between the clinical and commercial drug product. We have received initial feedback from the FDA on these data and are in the process of responding to additional queries prior to submitting the BLA, which could result in a BLA submission occurring in the third quarter of 2023.
- PTC submitted a Type II variation to EMA to support the conversion of the conditional marketing authorization for Translarna to a standard marketing authorization. PTC expects a CHMP opinion in the second quarter of 2023.

First Quarter 2023 Financial Highlights:

- Total revenues were $220.4 million for the first quarter of 2023, compared to $148.7 million for the first quarter of 2022.
- Total revenue includes net product revenue across the commercial portfolio of $187.6 million for the first quarter of 2023, compared to $129.8 million for the first quarter of 2022. Total revenue also includes collaboration, royalty and manufacturing revenue of $32.8 million in the first quarter of 2023, compared to $18.9 million for the first quarter of 2022.
- Translarna net product revenues were $115.1 million for the first quarter of 2023, compared to $79.2 million for the first quarter of 2022. These results were driven by treatment of new patients, continued high compliance and geographic expansion.
- Emflaza net product revenues were $54.6 million for the first quarter of 2023, compared to $48.6 million for the first quarter of 2022. These results reflect new patients, high compliance, and broader access.
- Roche reported Evrysdi 2023 year to date sales of approximately CHF 363 million, resulting in royalty revenue of $30.8 million to PTC for the first quarter of 2023, as compared to $18.9 million for the first quarter of 2022.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $195.1 million for the first quarter of 2023, compared to $140.1 million for the first quarter of 2022. The increase reflects additional investment in research programs and advancement of the clinical pipeline. The increase in R&D expenses includes 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.

- Non-GAAP R&D expenses were $179.8 million for the first quarter of 2023, excluding $15.3 million in non-cash, stock-based compensation expense, compared to $127.0 million for the first quarter of 2022, excluding $13.0 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $86.9 million for the first quarter of 2023, compared to $73.3 million for the first quarter of 2022. The increase reflects our continued investment to support commercial activities, including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $73.4 million for the first quarter of 2023, excluding $13.5 million in non-cash, stock-based compensation expense, compared to $59.7 million for the first quarter of 2022, excluding $13.6 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a loss of $2.4 million for the first quarter of 2023, compared to a gain of $11.7 million for the first quarter of 2022. The change in fair value of deferred and contingent consideration is 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.
- Net loss was $139.0 million for the first quarter of 2023, compared to net loss of $126.7 million for the first quarter of 2022.
- Cash, cash equivalents, and marketable securities was $286.3 million at March 31, 2023, compared to $410.7 million at December 31, 2022.
- Shares issued and outstanding as of March 31, 2023, were 74,012,034.

PTC Reaffirms Full Year 2023 Revenue Guidance as Follows:

- PTC anticipates total revenues for the full year 2023 to be between $940 million and $1.0 billion.
- PTC anticipates net product revenues for the DMD franchise for the full year 2023 to be between $545 and $565 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 March 31,	
	2023	**2022**
Revenues:		
Net product revenue	$ 187,557	$ 129,832
Collaboration revenue	6	7
Royalty revenue	30,831	18,896
Manufacturing revenue	1,988	-
Total revenues	220,382	148,735
Operating expenses:		
Cost of product sales, excluding amortization of acquired intangible assets	14,144	10,135
Amortization of acquired intangible asset	39,415	23,473
Research and development (1)	195,124	140,078
Selling, general and administrative (2)	86,914	73,271
Change in the fair value of deferred and contingent consideration	2,400	(11,700)
Total operating expenses	337,997	235,257
Loss from operations	(117,615)	(86,522)
Interest expense, net	(27,331)	(23,514)
Other income (expense), net	9,956	(11,855)
Loss before income tax expense	(134,990)	(121,891)
Income tax expense	(3,969)	(4,835)
Net loss attributable to common stockholders	$ (138,959)	$ (126,726)

Weighted-average shares outstanding:		
Basic and diluted (in shares)	73,729,284	71,215,105
Net loss per share—basic and diluted (in dollars per share)	$ (1.88)	$ (1.78)
(1) Research and development reconciliation		
GAAP research and development	$ 195,124	$ 140,078
Less: share-based compensation expense	15,314	13,034
Non-GAAP research and development	$ 179,810	$ 127,044
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 86,914	$ 73,271
Less: share-based compensation expense	13,501	13,555
Non-GAAP selling, general and administrative	$ 73,413	$ 59,716

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

	March 31, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 286,303	$ 410,705
Total Assets	**$ 1,608,839**	**$ 1,705,619**
Total debt	$ 572,091	$ 571,722
Total deferred revenue	214	1,351
Total liability for sale of future royalties	763,551	757,886
Total liabilities	**$ 2,066,400**	**$ 2,052,705**
Total stockholders' deficit (74,012,034 and 73,104,692 common shares issued and outstanding at March 31, 2023 and December 31, 2022 respectively)	$ (457,561)	$ (347,086)
Total liabilities and stockholders' deficit	**$ 1,608,839**	**$ 1,705,619**

Acronyms:
BLA: Biologics License Application
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EMA: European Medicines Agency
FA: Friedreich Ataxia
FDA: U.S. Food and Drug Administration
HD: Huntington's Disease
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy

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 science-driven, 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 2023 Revenue Guidance", including with respect to (i) 2023 total revenue guidance, and (ii) 2023 net product revenue guidance for the DMD franchise, 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; expectations with respect to Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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 future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to utilize results from 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, to support a marketing approval for Translarna for the treatment of nmDMD in the United States and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; 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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