PTC Therapeutics Provides Corporate Update and Reports Second Quarter Financial Results

August 3, 2023

– Second-quarter 2023 total revenue of $214 million, representing 29% year-over-year growth –

– Positive APHENITY and PIVOT-HD data readouts in the second quarter –

– Numerous regulatory milestones planned for the second half of 2023 –

SOUTH PLAINFIELD, N.J., Aug. 3, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ending June 30, 2023.

"I am extremely proud of the revenue growth in the first half of 2023 providing us confidence that we will meet our full-year total revenue guidance," said Matthew Klein, M.D., Chief Executive Officer, PTC Therapeutics, Inc. "In addition, the great results from the APHENITY and PIVOT-HD trials position us well for future growth."

Key Corporate Updates:

- Second quarter 2023 revenue for the Duchenne muscular dystrophy (DMD) franchise was $162 million, representing 21% year-over-year growth.
    - Translarna™ (ataluren) quarterly net product revenue was $96 million, with growth coming from treatment of new patients and continued geographic expansion.
    - Emflaza® (deflazacort) quarterly net product revenue was $66 million, driven by new patients, and high compliance.

Key Clinical and Regulatory Updates:

- The primary endpoint of blood phenylalanine reduction in the APHENITY trial for sepiapterin in PKU was achieved, with highly statistically significant and clinically meaningful results.
- PTC expects to file an NDA for sepiapterin in the fourth quarter of 2023, pending FDA feedback.
- All key objectives were met in the 12-week interim data analysis of the PIVOT-HD trial of PTC518 in Huntington's disease patients.
- PTC expects to submit the BLA for Upstaza in the third quarter of 2023.
- PTC expects a CHMP opinion on the Type II variation to support the conversion of the conditional marketing authorization for Translarna to a standard marketing authorization in the third quarter of 2023.
- PTC expects additional regulatory meetings in the second half of 2023 including:
    - Type C meeting with the FDA for vatiquinone in FA
    - Type C meeting with the FDA for Translarna in DMD

Second Quarter 2023 Financial Highlights:

- Total revenues were $213.8 million for the second quarter of 2023, compared to $165.5 million for the second quarter of 2022.
- Total revenues include net product revenue across the commercial portfolio of $174.6 million for the second quarter of 2023, compared to $143.7 million for the second quarter of 2022. Total revenues also include royalty and manufacturing revenue of $39.2 million for the second quarter of 2023, compared to $21.8 million for the second quarter of 2022.
- Translarna net product revenues were $96.5 million for the second quarter of 2023, compared to $77.0 million for the second quarter of 2022. These results were driven by treatment of new patients and continued geographic expansion.
- Emflaza net product revenues were $65.7 million for the second quarter of 2023, compared to $56.8 million for the second quarter of 2022. These results reflect new patients and high compliance.
- Roche reported Evrysdi 2023 year-to-date sales of approximately CHF 705 million, resulting in royalty revenue of $36.9 million to PTC for the second quarter of 2023, as compared to $21.8 million for the second quarter of 2022.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $185.9 million for the second quarter of 2023, compared to $157.3 million for the second quarter of 2022. The increase primarily reflects additional investment in advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $170.3 million for the second quarter of 2023, excluding $15.5 million in non-cash, stock-based compensation expense, compared to $143.5 million for the second quarter of 2022, excluding $13.8 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $88.4 million for the second quarter of 2023, compared to $79.9 million for the second

quarter of 2022. The increase reflects our continued investment to support commercial activities, including expanding our commercial portfolio.

- Non-GAAP SG&A expenses were $74.6 million for the second quarter of 2023, excluding $13.8 million in non-cash, stock-based compensation expense, compared to $66.0 million for the second quarter of 2022, excluding $13.9 million in non-cash, stock-based compensation expense.
- During the second quarter of 2023, PTC announced the discontinuation of preclinical and early research programs in gene therapy and a reduction in workforce as part of a strategic portfolio prioritization, which resulted in a one-time charge of approximately $8.0 million recorded to R&D and SG&A expense.
- The change in the fair value of contingent consideration was a gain of $128.9 million for the second quarter of 2023, compared to a gain of $15.2 million for the second quarter of 2022. The change in fair value of 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. As a result of the discontinuation of the Friedreich ataxia and Angelman syndrome gene therapy programs, 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, which is the primary driver of the overall gain during the quarter. An intangible asset impairment of $217.8 million was recorded in the second quarter of 2023 which also related to the discontinuation of Friedreich ataxia and Angelman syndrome gene therapy programs. The net impact of these gene therapy program discontinuations was non-cash expense of $88 million recorded within total operating expenses.
- The net loss was $198.9 million for the second quarter of 2023, compared to a net loss of $152.1 million for the second quarter of 2022.
- Cash, cash equivalents, and marketable securities was $337.9 million on June 30, 2023, compared to $410.7 million at December 31, 2022.
- Shares issued and outstanding as of June 30, 2023, were 75,318,233.

PTC Updates Full Year 2023 Financial 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 $575 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2023 to be between $930 million and $980 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year to be between $810 million and $860 million, excluding estimated non-cash stock-based compensation expense of $120 million.
- PTC also anticipates up to $62 million of one-time expenses, paid in cash or equity ($37 million of which was incurred during the first half of 2023), upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions and expenses associated with a rights exchange agreement.

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,	
	2023	2022	2023	2022
Revenues:				
Net product revenue	$ 174,592	$ 143,701	$ 362,149	$ 273,534
Collaboration revenue	-	-	6	7
Royalty revenue	36,853	21,825	67,684	40,721
Manufacturing revenue	2,363	-	4,351	-
Total revenues	213,808	165,526	434,190	314,262
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	12,731	9,639	26,875	19,774

Amortization of acquired intangible asset	47,397	26,294	86,812	49,767
Research and development (1)	185,874	157,263	380,998	297,341
Selling, general and administrative (2)	88,449	79,892	175,363	153,162
Change in the fair value of contingent consideration	(128,900)	(15,200)	(126,500)	(26,900)
Intangible asset impairment	217,800	-	217,800	-
Total operating expenses	423,351	257,888	761,348	493,144
Loss from operations	(209,543)	(92,362)	(327,158)	(178,882)
Interest expense, net	(29,415)	(21,976)	(56,745)	(45,490)
Other income (expense), net	1,479	(34,357)	11,434	(46,214)
Loss before income tax expense	(237,479)	(148,695)	(372,469)	(270,586)
Income tax benefit (expense)	38,596	(3,392)	34,627	(8,227)
Net loss attributable to common stockholders	$ (198,883)	$ (152,087)	$ (337,842)	$ (278,813)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	74,730,433	71,372,940	74,232,624	71,294,458
Net loss per share—basic and diluted (in dollars per share)	$ (2.66)	$ (2.13)	$ (4.55)	$ (3.91)
(1) Research and development reconciliation				
GAAP research and development	$ 185,874	$ 157,263	$ 380,998	$ 297,341
Less: share-based compensation expense	15,529	13,798	30,842	26,832
Non-GAAP research and development	$ 170,345	$ 143,465	$ 350,156	$ 270,509
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 88,449	$ 79,892	$ 175,363	$ 153,162
Less: share-based compensation expense	13,842	13,932	27,344	27,487
Non-GAAP selling, general and administrative	$ 74,607	$ 65,960	$ 148,019	$ 125,675

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

	June 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 337,943	$ 410,705
Total Assets	**$ 1,338,124**	**$ 1,705,619**
Total debt	$ 572,643	$ 571,722
Total deferred revenue	-	1,351
Total liability for sale of future royalties	766,580	757,886
Total liabilities	**$ 1,917,392**	**$ 2,052,705**
Total stockholders' deficit (75,318,233 and 73,104,692 common shares issued and outstanding at June 30, 2023 and December 31, 2022 respectively)	$ (579,268)	$ (347,086)
Total liabilities and stockholders' deficit	**$ 1,338,124**	**$ 1,705,619**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2023 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 930,000	$ 980,000
Less: projected non-cash, stock-based compensation expense	120,000	120,000
Projected non-GAAP R&D and SG&A expense	$ 810,000	$ 860,000

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
NDA: New Drug Application
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 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 2023 Revenue Guidance as Follows", including with respect to (i) 2023 total revenue guidance, (ii) 2023 net product revenue guidance for the DMD franchise, (iii) 2023 GAAP and non-GAAP R&D and SG&A expense guidance and (iv) 2023 acquisition related one-time expense 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 the discontinuation of PTC's preclinical and early research programs in gene therapy and reduction 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; 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; 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; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and early research programs in gene therapy and reduction 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 preclinical and early research programs in gene therapy and reduction in workforce, which may be materially less than expected; 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; 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; 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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