PTC Therapeutics Provides a Corporate Update and Reports Fourth Quarter and Full Year 2022 Financial Results

February 21, 2023

– 2022 total revenue of $699 million, representing 30% year-over-year growth, or $740 million and 37% growth at CER –*

– Guidance for full-year 2023 total revenue of $940 million to $1 billion reaffirmed –

– Four important study readouts expected in first half of 2023, three of them registration-directed –

SOUTH PLAINFIELD, N.J., Feb. 21, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the fourth quarter and full year ending December 31, 2022.

"2023 will be a very exciting year at PTC, including a celebration of our 25th anniversary," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Together our marketed products grew 30% in revenue in 2022 despite significant foreign exchange headwinds. We expect our revenue growth to accelerate further in 2023, potentially reaching $1 billion in total revenue. We are also advancing a broad and deep pipeline of new therapies that we expect to provide substantial growth in the coming years."

Key Corporate Updates:

- 2022 total revenue of $699 million, representing 30% year-over-year growth, or $740 million when calculated at CER, representing a 37% growth
- 2022 revenue for the Duchenne muscular dystrophy (DMD) franchise was $507 million, representing 20% year-over-year growth, or $539 million when calculated at CER, representing a 27% growth. The DMD franchise fourth quarter total net product revenue was $114 million
    - Translarna™ (ataluren) total net product revenue was $56 million, with growth coming from new patients in existing geographies and continued geographic expansion
    - Emflaza® (deflazacort) total net product revenue was $58 million, driven by new patient starts, broader access, continued high compliance, and appropriate weight-based dosing

Key Clinical and Regulatory Updates:

- PTC continues to make progress in additional 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. Data from the first 12 weeks of the placebo-controlled trial is anticipated in the second quarter of 2023.

Fourth-Quarter and Full-Year 2022 Financial Highlights:

- Total revenues were $167.4 million for the fourth quarter of 2022, compared to $165.2 million for the fourth quarter of 2021. Total revenues were $698.8 million for the full year 2022, compared to $538.6 million for the full year 2021.
- Total revenue includes net product revenue across the commercial portfolio of $127.5 million for the fourth quarter of 2022 and $535.2 million for the full year 2022, compared to $118.9 million for the fourth quarter of 2021 and $428.9 million for the full year 2021. Total revenue also includes collaboration and royalty revenue of $39.9 million in the fourth quarter of 2022 and $163.6 million for the full year 2022, compared to $46.3 million for the fourth quarter of 2021 and $109.7 million for the full year 2021.
- Translarna net product revenues were $55.8 million for the fourth quarter of 2022, compared to $69.7 million for the fourth quarter of 2021. Translarna net product revenues were $288.6 million for the full year 2022, compared to $236.0 million for the full year 2021. These results were driven by treatment of new patients, continued high compliance, and geographic expansion.
- Emflaza net product revenues were $58.1 million for the fourth quarter of 2022, compared to $47.5 million for the fourth quarter of 2021. Emflaza net product revenues were $218.3 million for the full year 2022, compared to $187.3 million for the full year 2021. These results reflect new patient prescriptions, high compliance, and fewer discontinuations.

- Roche reported Evrysdi 2022 year to date sales of approximately CHF 1,119 million, resulting in full year 2022 royalty revenue of $113.5 million to PTC, as compared to $54.6 million for full year 2021.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $188.7 million for the fourth quarter of 2022, compared to $149.8 million for the fourth quarter of 2021. GAAP R&D expenses were $651.5 million for the full year 2022, compared to $540.7 million for the full year 2021. The increase reflects additional investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $174.7 million for the fourth quarter of 2022, excluding $14.0 million in non-cash, stock-based compensation expense, compared to $136.4 million for the fourth quarter of 2021, excluding $13.4 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $595.6 million for the full year 2022, excluding $55.9 million in non-cash, stock-based compensation expense, compared to $487.1 million for the full year 2021, excluding $53.6 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $92.7 million for the fourth quarter of 2022, compared to $86.5 million for the fourth quarter of 2021. GAAP SG&A expenses were $326.0 million for the full year 2022, compared to $285.8 million for the full year 2021. The increase reflects our continued investment to support commercial activities, including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $79.3 million for the fourth quarter of 2022, excluding $13.4 million in non-cash, stock-based compensation expense, compared to $73.7 million for the fourth quarter of 2021, excluding $12.8 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $271.5 million for the full year 2022, excluding $54.5 million in non-cash, stock-based compensation expense, compared to $235.9 million for the full year 2021, excluding $49.9 million in non-cash, stock-based compensation expense.
- Intangible asset impairment was $33.4 million for the fourth quarter and full year 2022, which represents a non-cash charge. The partial impairment was related to a decrease in projected cash flows due to refinements in current market assumptions and the timing of patient treatments.
- Change in the fair value of deferred and contingent consideration was a loss of $6.3 million for the fourth quarter of 2022, compared to a gain of $12.1 million for the fourth quarter of 2021. Change in the fair value of deferred and contingent consideration was a gain of $25.9 million for the full year 2022, compared to a gain of $0.5 million for the full year 2021. 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 $170.9 million for the fourth quarter of 2022, compared to net loss of $143.3 million for the fourth quarter of 2021. Net loss was $559.0 million for the full year 2022, compared to net loss of $523.9 million for the full year 2021.
- Cash, cash equivalents, and marketable securities was $410.7 million at December 31, 2022, compared to $773.4 million at December 31, 2021.
- Shares issued and outstanding as of December 31, 2022, were 73,104,692.

PTC Reaffirms Full Year 2023 Financial Guidance:

- 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 million and $565 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2023 to be between $1.01 billion and $1.06 billion.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2023 to be between $890 million and $940 million, excluding estimated non-cash, stock-based compensation expense of $120 million. PTC anticipates up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions.

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, and the revenue and percentage changes in revenue growth at CER are presented excluding the impact of changes in foreign currency exchange rates. For financial measures given at CER, the current period's foreign currency revenue values are converted into U.S. dollars using the average exchange rates from the prior period. 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,	
	2022	2021	2022	2021
Revenues:				
Net product revenue	$ 127,508	$ 118,905	$ 535,228	$ 428,904
Collaboration revenue	28	25,029	50,052	55,046
Royalty revenue	39,876	21,294	113,521	54,643
Total revenues	167,412	165,228	698,801	538,593
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	10,893	9,327	44,678	32,328
Amortization of acquired intangible asset	35,764	16,340	116,554	54,751
Research and development (1)	188,694	149,844	651,496	540,684
Selling, general and administrative (2)	92,718	86,548	325,998	285,773
Intangible asset impairment	33,384	-	33,384	-
Change in the fair value of deferred and contingent consideration	6,300	(12,100)	(25,900)	(500)
Total operating expenses	367,753	249,959	1,146,210	913,036
Loss from operations	(200,341)	(84,731)	(447,409)	(374,443)
Interest expense, net	(24,500)	(22,502)	(90,871)	(86,022)
Other income (expense), net	35,147	(31,375)	(49,207)	(57,875)
Loss before income tax expense	(189,694)	(138,608)	(587,487)	(518,340)
Income tax benefit (expense)	18,805	(4,657)	28,470	(5,561)
Net loss attributable to common stockholders	$ (170,889)	$ (143,265)	$ (559,017)	$ (523,901)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	72,656,790	70,669,797	71,728,634	70,466,393
Net loss per share—basic and diluted (in dollars per share)	$ (2.35)	$ (2.03)	$ (7.79)	$ (7.43)
(1) Research and development reconciliation				
GAAP research and development	$ 188,694	$ 149,844	$ 651,496	$ 540,684
Less: share-based compensation expense	13,973	13,416	55,869	53,632
Non-GAAP research and development	$ 174,721	$ 136,428	$ 595,627	$ 487,052
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 92,718	$ 86,548	$ 325,998	$ 285,773
Less: share-based compensation expense	13,370	12,819	54,464	49,881
Non-GAAP selling, general and administrative	$ 79,348	$ 73,729	$ 271,534	$ 235,892

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Revenue and Year-Over-Year Revenue Growth at Constant Exchange Rates
(In thousands, except percentages)

	December 31, 2022 Twelve Months Ended	December 31, 2022 Twelve Months Ended
GAAP DMD revenue as reported; GAAP year-over-year revenue growth	$ 506,846	20 %
Impact of foreign currency translation - DMD net product revenue; year-over year DMD net product revenue growth	32,300	7 %
Non-GAAP DMD revenue at constant exchange rate; Non-GAAP year-over-year revenue growth at constant exchange rate	**$ 539,146**	**27 %**

	December 31, 2022 Twelve Months Ended	December 31, 2022 Twelve Months Ended
GAAP total revenue as reported; GAAP year-over-year total revenue growth	$ 698,801	30 %
Total impact of foreign currency translation – total revenue; year-over-year total revenue growth	40,800	7 %
Non-GAAP total revenue at constant exchange rate; Non-GAAP year over-year total revenue growth at constant exchange rate	**$ 739,601**	**37 %**

Summary Consolidated Balance Sheets
(In thousands, except share data)

	December 31, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	$ 410,705	$ 773,376
Total Assets	**$ 1,705,619**	**$ 1,938,056**
Total debt	$ 571,722	$ 431,434
Total deferred revenue	1,351	-
Total liability for sale of future royalties	757,886	733,985
Total liabilities	**$ 2,052,705**	**$ 1,936,618**
Total stockholders' (deficit) equity (73,104,692 and 70,828,226 common shares issued and outstanding at December 31, 2022 and December 31, 2021, respectively)	$ (347,086)	$ 1,438
Total liabilities and stockholders' (deficit) equity	**$ 1,705,619**	**$ 1,938,056**

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	$ 1,010,000	$ 1,060,000
Less: projected non-cash, stock-based compensation expense	120,000	120,000
Projected non-GAAP R&D and SG&A expense	$ 890,000	$ 940,000

Acronyms:
AADC: Aromatic L-amino acid decarboxylase
ALS: Amyotrophic Lateral Sclerosis
CER: Constant Exchange Rate
DMD: Duchenne Muscular Dystrophy
FDA: U.S. Food and Drug Administration
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy
* Revenue and growth at Constant Exchange Rates, or CER, represents revenue and growth calculated as if the exchange rates had remained unchanged from average exchange rates in 2021. CER is a non-GAAP measure.

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 Financial Guidance", 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; 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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