PTC Therapeutics Provides Corporate Update and Reports First Quarter 2025 Financial Results

May 6, 2025

– Strong revenue performance of $190 million –

– Positive CHMP opinion for Sephience™ (sepiapterin) received in April 2025, NDA review remains on track for July 29, 2025 PDUFA date –

– Global Sephience launch activities progressing well –

– Strong cash position of over $2.0 billion as of March 31, 2025 –

WARREN, N.J., May 6, 2025 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ended March 31, 2025.

"Following a year of outstanding execution across every part of the Company, we have built on this positive momentum with solid revenue performance in the first quarter, allowing us to narrow our full-year revenue guidance," said Matthew B. Klein, M.D., Chief Executive Officer. "Our strong cash balance supports all of our planned commercial and R&D activities and provides the ability to reach cashflow breakeven without raising additional capital. The positive CHMP opinion for Sephience kickstarts our anticipated global launch for what we see as a significant revenue opportunity."

Key Corporate Updates:

- First quarter 2025 total net product and royalty revenue of $190 million.
- First quarter 2025 revenue for the DMD franchise of $134 million, including net product revenue for Translarna ™of $86 million and for Emflaza® of $48 million.

Key Clinical and Regulatory Milestones:

- Sephience
  - Positive CHMP opinion on Sephience MAA for adult and pediatric PKU patients received on April 25, 2025; the opinion includes a broad label inclusive of all ages and disease severities. PTC expects the EC to adopt the opinion in approximately two months
  - NDA currently under review by the FDA, with a target regulatory action date of July 29, 2025
  - Japan regulatory submission under review and a decision is expected in Q4 2025
- Vatiquinone
  - NDA for pediatric and adult patients with Friedreich's ataxia accepted and granted Priority Review by the FDA, with a target regulatory action date of August 19, 2025
- Translarna
  - NDA currently under review by the FDA
- PTC518
  - Phase 2 PIVOT-HD study results announced on May 5, 2025:
    - Met primary endpoint of dose-dependent blood HTT lowering at Week 12
    - Dose-dependent trends of clinical benefit in Stage 2 patients at Month 12
    - Signals of dose-dependent clinical benefit relative to matched natural history cohort and dose-dependent lowering of NfL in Stage 2 subjects at Month 24
    - Continued favorable safety and tolerability with no treatment-related NfL spikes
    - Plans to complete additional analyses and discuss next development and regulatory steps, including potential for accelerated approval

First Quarter 2025 Financial Highlights:

- Total net product and royalty revenue was $189.9 million for the first quarter of 2025, compared to $208.8 million for the first quarter of 2024.
- Total revenue includes net product revenue across the commercial portfolio of $153.4 million for the first quarter of 2025, compared to $177.6 million for the first quarter of 2024. Total revenue also includes royalty, collaboration and license, and manufacturing revenue of $1,022.7 million for the first quarter of 2025, compared to $32.5 million for the first quarter of 2024. The first quarter of 2025 collaboration and license revenue includes $986.2 million, related to the PTC518 license and collaboration agreement with Novartis, which closed in January 2025.
- Translarna net product revenues were $86.2 million for the first quarter of 2025, compared to $103.6 million for the first

quarter of 2024.

- Emflaza net product revenues were $47.8 million for the first quarter of 2025, compared to $57.5 million for the first quarter of 2024.
- Roche reported Evrysdi® full year 2025 sales of approximately 420 CHF million, resulting in royalty revenue of $36.4 million to PTC for first quarter 2025, as compared to $31.2 million for first quarter 2024.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $109.0 million for the first quarter of 2025, compared to $116.1 million for the first quarter of 2024.
- Non-GAAP R&D expenses were $100.3 million for the first quarter of 2025, excluding $8.7 million in non-cash, stock-based compensation expense, compared to $107.2 million for the first quarter of 2024, excluding $9.0 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $81.0 million for the first quarter of 2025, compared to $73.3 million for the first quarter of 2024.
- Non-GAAP SG&A expenses were $71.6 million for the first quarter of 2025, excluding $9.4 million in non-cash, stock-based compensation expense, compared to $63.9 million for the first quarter of 2024, excluding $9.4 million in non-cash, stock-based compensation expense.
- Net income was $866.6 million for the first quarter of 2025, compared to net loss of $91.6 million for the first quarter of 2024.
- Cash, cash equivalents, and marketable securities were $2,027.2 million as of March 31, 2025, compared to $1,139.7 million as of December 31, 2024.
- Shares issued and outstanding as of March 31, 2025 were 79,225,276.

PTC Updates Full-Year 2025 Financial Guidance:

- PTC now anticipates full-year 2025 revenue to be between $650 million and $800 million, which includes in-line products, potential new product launches, and royalty revenue from Evrysdi.
- PTC anticipates full-year 2025 GAAP R&D and SG&A expense to be between $805 and $835 million.
- PTC anticipates full-year 2025 non-GAAP R&D and SG&A expense to be between $730 and $760 million, excluding estimated non-cash, stock-based compensation expense of $75 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,	
	2025	**2024**
Revenues:		
Net product revenue	$ 153,426	$ 177,604
Collaboration and license revenue	986,231	-
Royalty revenue	36,439	31,154
Manufacturing revenue	-	1,360
Total revenues	1,176,096	210,118
Operating expenses:		
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	12,862	14,740
Amortization of acquired intangible assets	3,798	51,530
Research and development (1)	108,973	116,129
Selling, general and administrative (2)	80,961	73,272
Change in the fair value of contingent consideration	(800)	(100)
Tangible asset impairment and losses on transactions, net	77	-
Total operating expenses	205,871	255,571
Income (loss) from operations	970,225	(45,453)
Interest expense, net	(34,092)	(40,834)
Other (expense) income, net	(6,305)	1,591

Income (loss) before income tax expense	929,828	(84,696)
Income tax expense	(63,266)	(6,880)
Net income (loss) attributable to common stockholders	$ 866,562	$ (91,576)
Weighted-average shares outstanding:		
Basic (in shares)	78,115,836	76,496,127
Diluted (in shares)	86,385,922	76,496,127
Net income (loss) per share—basic (in dollars per share)	$ 11.09	$ (1.20)
Net income (loss) per share—diluted (in dollars per share)	$ 10.04	$ (1.20)
(1) Research and development reconciliation		
GAAP research and development	$ 108,973	$ 116,129
Less: share-based compensation expense	8,663	8,967
Non-GAAP research and development	$ 100,310	$ 107,162
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 80,961	$ 73,272
Less: share-based compensation expense	9,397	9,411
Non-GAAP selling, general and administrative	$ 71,564	$ 63,861

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

	March 31, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 2,027,181	$ 1,139,696
Total Assets	**$ 2,655,387**	**$ 1,705,024**
Total debt	$ 285,712	$ 285,412
Total deferred revenue	12,833	5,505
Total liability for sale of future royalties	2,098,463	2,081,776
Total liabilities	**$ 2,841,147**	**$ 2,803,095**
Total stockholders' deficit (79,225,276 and 77,704,188 common shares issued and outstanding at March 31, 2025 and December 31, 2024, respectively)	$ (185,760)	$ (1,098,071)
Total liabilities and stockholders' deficit	**$ 2,655,387**	**$ 1,705,024**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2025 R&D and SG&A Expense
(In millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 805	$ 835
Less: projected non-cash, stock-based compensation expense	75	75
Projected non-GAAP R&D and SG&A expense	$ 730	$ 760

Acronyms:

CHF: Confoederatio Helvetica Francs (Swiss francs)
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EC: European Commission
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's Disease

HTT: Huntingtin protein
MAA: Marketing Authorization Application
NDA: New Drug Application
NfL: Neurofilament light chain
nmDMD: Nonsense mutation Duchenne muscular dystrophy
PDUFA: Prescription Drug User Fee Act
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 for the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investors 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.

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 children and adults living 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 www.ptcbio.com and follow on Facebook, X, and 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 2025 Financial Guidance", including with respect to (i) 2025 total revenue guidance and (ii) 2025 GAAP and non-GAAP R&D and SG&A 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; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia and other regions; the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) on Translarna on other regulatory bodies; 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 its trials in Translarna; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; expectations with respect to Upstaza/Kebilidi, including commercialization, manufacturing capabilities, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to Sephience, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; 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; 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, Kebilidi, Evrysdi, Tegsedi, Waylivra, Sephience or vatiquinone.

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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