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

November 4, 2025

– Initiated US and EU launch of Sephience ™ (sepiapterin) –

– Strong initial Sephience uptake with global revenue of $19.6M and 521 start forms in the US as of September 30 –

– Robust Q3 performance with total revenue of $211M –

– Full-year 2025 revenue guidance narrowed to $750 - $800M –

WARREN, N.J., Nov. 4, 2025 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ended September 30, 2025.

"We achieved an outstanding quarter, highlighted by the strong start to the global Sephience launch," said Matthew B. Klein, M.D., Chief Executive Officer. "The broad initial uptake supports the potential of Sephience to become the standard of care for all individuals with PKU, as well as the foundational product for PTC's sustained growth and success."

Key Corporate Updates

- Third quarter 2025 total revenue of $211.0 million
- Global launch of Sephience™ initiated in US and Europe
  - Third quarter 2025 revenue of $19.6 million, including $14.4 million revenue in the US and $5.2 million revenue ex-US
  - 521 patient start forms received from 141 unique prescribers in the US as of September 30
  - 341 total patients on commercial therapy worldwide as of September 30
- Third quarter 2025 revenue for the DMD franchise of $85.9 million, including net product revenue for Translarna ™ of $50.7 million and for Emflaza® of $35.2 million
- R&D Day planned for Tuesday, December 2nd in New York

Key Clinical and Regulatory Updates

- Additional Sephience marketing authorization reviews ongoing including in Japan where decision is expected in Q4 2025
- FDA meeting for votoplam Huntington's disease program planned for Q4
- FDA meeting planned for vatiquinone Friedreich's ataxia program in Q4
- Translarna NDA remains under FDA review

Third Quarter 2025 Financial Highlights

- Total revenue was $211.0 million for the third quarter of 2025, compared to $196.8 million for the third quarter of 2024.
- Total revenue includes net product revenue across the commercial portfolio of $131.0 million for the third quarter of 2025, compared to $135.4 million for the third quarter of 2024. Total revenue also includes royalty, collaboration and license revenue of $80.1 million for the third quarter of 2025, compared to $61.4 million for the third quarter of 2024.
- Sephience net product revenue was $19.6 million for the third quarter of 2025.
- Translarna net product revenue was $50.7 million for the third quarter of 2025, compared to $72.3 million for the third quarter of 2024.
- Emflaza net product revenue was $35.2 million for the third quarter of 2025, compared to $51.9 million for the third quarter of 2024.
- Roche reported Evrysdi® 2025 year-to-date sales of approximately CHF 1,293 million, resulting in royalty revenue of $70.8 million to PTC for the third quarter of 2025, as compared to $61.4 million for the third quarter of 2024.
- Based on US GAAP (Generally Accepted Accounting Principles), GAAP R&D expense was $100.2 million for the third quarter of 2025, compared to $161.4 million for the third quarter of 2024.
- Non-GAAP R&D expense was $91.0 million for the third quarter of 2025, excluding $9.1 million in non-cash, stock-based compensation expense, compared to $152.0 million for the third quarter of 2024, excluding $9.4 million in non-cash, stock-based compensation expense.
- GAAP SG&A expense was $84.0 million for the third quarter of 2025, compared to $73.5 million for the third quarter of 2024.
- Non-GAAP SG&A expense was $73.8 million for the third quarter of 2025, excluding $10.3 million in non-cash, stock-based compensation expense, compared to $63.1 million for the third quarter of 2024, excluding $10.3 million in non-cash, stock-based compensation expense.
- Net income was $15.9 million for the third quarter of 2025, compared to net loss of $106.7 million for the third quarter of 2024.
- Cash, cash equivalents, and marketable securities were $1,687.8 million as of September 30, 2025, compared to $1,139.7 million as of December 31, 2024. The third quarter cash balance reflects the PTC agreement to purchase approximately 90% of the Sephience annual percentage-based global net sales obligation owed to former Censa shareholders in exchange for an upfront payment of $225.0 million and future sales-based milestone payments.
- Shares issued and outstanding as of September 30, 2025, were 79,931,766.

PTC Full-Year 2025 Financial Guidance

For the full year 2025, PTC anticipates:

- Total revenue of $750 to $800 million, which includes in-line products and royalty revenue from Evrysdi.
- GAAP R&D and SG&A expense of $805 to $835 million.
- Non-GAAP R&D and SG&A expense of $730 to $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 accordance with 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 September 30,		Nine Months Ended September 30,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Net product revenue	$ 130,956	$ 135,421	$ 402,711	$ 446,245
Collaboration and license revenue	9,258	-	998,430	-
Royalty revenue	70,793	61,365	164,837	145,702
Manufacturing revenue	-	-	-	1,661
Total revenues	211,007	196,786	1,565,978	593,608
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	15,781	10,848	40,063	41,115
Amortization of acquired intangible assets	7,534	3,036	15,393	57,431
Research and development (1)	100,158	161,412	322,121	409,710
Selling, general and administrative (2)	84,046	73,456	250,269	216,228
Change in the fair value of contingent consideration	-	700	(800)	5,700
Tangible asset impairment and losses (gains) on transactions, net	472	1,844	648	3,605
Total operating expenses	207,991	251,296	627,694	733,789
Income (loss) from operations	3,016	(54,510)	938,284	(140,181)
Interest expense, net	(32,592)	(41,609)	(97,042)	(125,933)
Other income (expense), net	6,502	(1,872)	(5,540)	(2,306)
(Loss) income before income tax benefit (expense)	(23,074)	(97,991)	835,702	(268,420)
Income tax benefit (expense)	38,970	(8,663)	(18,093)	(28,989)
Net income (loss) attributable to common stockholders	$ 15,896	$ (106,654)	$ 817,609	$ (297,409)
Weighted-average shares outstanding:				
Basic (in shares)	78,471,119	76,925,523	78,642,478	76,716,340
Diluted (in shares)	87,115,369	76,925,523	86,899,727	76,716,340
Net income (loss) per share—basic (in dollars per share)	$ 0.20	$ (1.39)	$ 10.40	$ (3.88)
Net income (loss) per share—diluted (in dollars per share)	$ 0.20	$ (1.39)	$ 9.45	$ (3.88)
(1) Research and development reconciliation				
GAAP research and development	$ 100,158	$ 161,412	$ 322,121	$ 409,710
Less: share-based compensation expense	9,133	9,416	26,827	27,810
Non-GAAP research and development	$ 91,025	$ 151,996	$ 295,294	$ 381,900
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 84,046	$ 73,456	$ 250,269	$ 216,228
Less: share-based compensation expense	10,293	10,339	29,202	29,566

Non-GAAP selling, general and administrative	$ 73,753	$ 63,117	$ 221,067	$ 186,662

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

	September 30, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 1,687,817	$ 1,139,696
Total Assets	**$ 2,643,700**	**$ 1,705,024**
Total debt	$ 286,319	$ 285,412
Total deferred revenue	1,691	5,505
Total liability for sale of future royalties	2,081,921	2,081,776
Total liabilities	**$ 2,799,458**	**$ 2,803,095**
Total stockholders' deficit (79,931,766 and 77,704,188 common shares issued and outstanding at September 30, 2025 and December 31, 2024, respectively)	$ (155,758)	$ (1,098,071)
Total liabilities and stockholders' deficit	**$ 2,643,700**	**$ 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)
DMD: Duchenne Muscular Dystrophy
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
NDA: New Drug Application
nmDMD: Nonsense mutation Duchenne muscular dystrophy
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 and will be archived on the company's website for 30 days.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. PTC is advancing a robust and diversified pipeline of transformative medicines as part of its mission to provide access to best-in-class treatments for patients with unmet medical needs. The company's strategy is to leverage its scientific expertise and global commercial infrastructure to optimize value for patients and other stakeholders. To learn more about PTC, please visit www.ptcbio.com and follow on LinkedIn, X and Facebook.

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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 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, meetings with regulatory agencies, 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," "aim," 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 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; 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 for votoplam for the treatment of Huntington's disease including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; 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 vatiquinone, including with respect to the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses and potential approvals and other matters; 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 Quarterly Report on Form 10-Q and 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 Sephience, Translarna, Emflaza, Upstaza, Kebilidi, Evrysdi, Tegsedi, Waylivra 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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