PTC Therapeutics Provides Corporate Update and Reports Fourth Quarter and Full Year 2025 Financial Results

February 19, 2026

– Full-year 2025 product and royalty revenue of $831M, exceeding guidance –

– Strong Sephience™ (sepiapterin) uptake since 2H 2025 launch with fourth quarter and 2025 revenue of $92M and $111M, respectively –

– Cash of $1.95B as of December 31, 2025 –

WARREN, N.J., Feb. 19, 2026 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the fourth quarter and full year ending December 31, 2025.

"We delivered another strong quarter and finish to 2025, building on the successful global launch of Sephience," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "With our robust commercial engine, innovative R&D programs, and strong financial position, we look forward to continued success as we approach cash flow breakeven."

Key Corporate Highlights

- Full-year 2025 product and royalty revenue of $831 million, exceeding guidance
- Global launch of Sephience off to strong start
  - Q4 2025 revenue of $92 million, including $81 million revenue in the US and $11 million revenue ex-US
  - Sephience total net revenue of $111 million in 2025 since launch
  - 946 total patients on commercial therapy worldwide as of December 31, 2025
  - 1,134 patient start forms received in the US as of December 31, 2025
  - Approval in Japan in December 2025; approval in Brazil in February 2026
  - Sephience global footprint expected to increase to 20 to 30 countries by end of 2026
- In December 2025, PTC sold the remainder of its Evrysdi® (risdiplam) royalty to Royalty Pharma for $240 million upfront and up to $60 million in sales-based milestones; PTC maintains the right to receive a $150 million milestone from Roche based on single-year Evrysdi sales of $2.5 billion
- End-of-Phase 2 meeting with FDA held in Q4 2025 to discuss the votoplam Huntington's disease (HD) program
  - Alignment reached on design of global Phase 3 trial, INVEST-HD, which is planned to initiate in 1H 2026
  - FDA confirmed openness for potential Accelerated Approval pathway given significant unmet need
- Type C meeting with FDA held in December 2025 to discuss the vatiquinone Friedreich's ataxia program; FDA indicated that an additional study would be necessary to support NDA resubmission and meeting minutes stated that this could be an open-label study with a natural history control group

Fourth Quarter and Full Year 2025 Financial Highlights

- Total revenues were $164.7 million for the fourth quarter of 2025, compared to $213.2 million for the fourth quarter of 2024. Total revenues were $1,730.7 million for full year 2025, compared to $806.8 million for full year 2024. Included in total revenues is collaboration and license revenue of $998.4 million for the full year 2025, related to the votoplam license and collaboration agreement with Novartis, which closed in January 2025.
- Total net product revenues were $184.0 million for the fourth quarter of 2025, compared to $150.1 million for the fourth quarter of 2024. Total net product revenues were $586.7 million for full year 2025, compared to $582.1 million for full year 2024.
- Translarna™ (ataluren) net product revenues were $39.0 million for the fourth quarter of 2025, compared to $89.1 million for the fourth quarter of 2024. Translarna net product revenues were $235.3 million for full year 2025, compared to $321.1 million for full year 2024.
- Emflaza® (deflazacort) net product revenues were $27.1 million for the fourth quarter of 2025, compared to $50.5 million for the fourth quarter of 2024. Emflaza net product revenues were $146.4 million for full year 2025, compared to $207.2 million for full year 2024.
- Roche reported Evrysdi full year 2025 sales of approximately 1,757 CHF million, resulting in royalty revenue of $244.2 million to PTC for full year 2025, compared to $203.9 million to PTC for full year 2024.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $133.1 million for the fourth quarter of 2025, compared to $124.8 million for the fourth quarter of 2024. GAAP R&D expenses were $455.2 million for full year 2025, compared to $534.5 million for full year 2024.
- Non-GAAP R&D expenses were $124.3 million for the fourth quarter of 2025, excluding $8.8 million in non-cash, stock-based compensation expense, compared to $116.0 million for the fourth quarter of 2024, excluding $8.8 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $419.6 million for full year 2025, excluding $35.7 million in non-cash, stock-based compensation expense, compared to $497.9 million for full year 2024, excluding $36.6 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $96.9 million for the fourth quarter of 2025, compared to $84.7 million for the fourth quarter of 2024. GAAP SG&A expenses were $347.1 million for full year 2025, compared to $300.9 million for full year 2024.
- Non-GAAP SG&A expenses were $87.2 million for the fourth quarter of 2025, excluding $9.7 million in non-cash, stock-based compensation expense, compared to $76.3 million for the fourth quarter of 2024, excluding $8.4 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $308.3 million for full year 2025, excluding $38.9 million in non-cash,

stock-based compensation expense, compared to $262.9 million for full year 2024, excluding $38.0 million in non-cash, stock-based compensation expense.

- Net loss was $135.0 million for the fourth quarter of 2025, compared to net loss of $65.9 million for the fourth quarter of 2024. Net income was $682.6 million for full year 2025, compared to net loss of $363.3 million for full year 2024.
- Cash, cash equivalents, and marketable securities were $1,945.4 million on December 31, 2025, compared to $1,139.7 million on December 31, 2024.
- Shares issued and outstanding as of December 31, 2025, were 81,474,366.

Full Year 2026 Financial Guidance

- Total product revenue of $700 to $800 million, representing a 19 to 36% increase from 2025, with the majority from Sephience
- GAAP R&D and SG&A expense of $775 to $815 million
- Non-GAAP R&D and SG&A expense of $680 to $720 million, excluding estimated non-cash, stock-based compensation expense of $95 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 December 31,		Twelve Months Ended December 31,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Net product revenue	$ 183,992	$ 150,088	$ 586,703	$ 582,145
Collaboration and license revenue	(73)	304	998,357	304
Royalty revenue	79,387	58,162	244,224	203,864
Translarna France*	(98,629)	4,618	(98,629)	18,806
Manufacturing revenue	-	-	-	1,661
Total revenues	164,677	213,172	1,730,655	806,780
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	6,950	16,283	47,013	57,398
Amortization of acquired intangible assets	9,349	3,307	24,742	60,738
Research and development (1)	133,128	124,770	455,249	534,480
Selling, general and administrative (2)	96,874	84,683	347,143	300,911
Change in the fair value of contingent consideration	-	(10,175)	(800)	(4,475)
Intangible asset impairment	-	159,548	-	159,548
Tangible asset impairment and (gains) losses on transactions, net	(10,275)	(2,855)	(9,627)	750
Total operating expenses	236,026	375,561	863,720	1,109,350
(Loss) income from operations	(71,349)	(162,389)	866,935	(302,570)
Interest expense, net	(55,188)	(41,060)	(152,230)	(166,993)
Other (expense) income, net	(12,546)	8,850	(18,086)	6,544
Gain on sale of priority review voucher	-	99,900	-	99,900
(Loss) income before income tax benefit (expense)	(139,083)	(94,699)	696,619	(363,119)
Income tax benefit (expense)	4,118	28,813	(13,975)	(176)
Net (loss) income attributable to common stockholders	$ (134,965)	$ (65,886)	$ 682,644	$ (363,295)
Weighted-average shares outstanding:				
Basic (in shares)	80,689,810	77,201,783	79,534,290	76,845,055
Diluted (in shares)	80,689,810	77,201,783	88,311,494	76,845,055
Net (loss) income per share—basic (in dollars per share)	$ (1.67)	$ (0.85)	$ 8.58	$ (4.73)
Net (loss) income per share—diluted (in dollars per share)	$ (1.67)	$ (0.85)	$ 7.78	$ (4.73)
(1) Research and development reconciliation				
GAAP research and development	$ 133,128	$ 124,770	$ 455,249	$ 534,480
Less: share-based compensation expense	8,843	8,818	35,669	36,629
Non-GAAP research and development	$ 124,285	$ 115,952	$ 419,580	$ 497,851

(2) Selling, general and administrative reconciliation

GAAP selling, general and administrative	$ 96,874	$ 84,683	$ 347,143	$ 300,911
Less: share-based compensation expense	9,675	8,420	38,878	37,986
Non-GAAP selling, general and administrative	$ 87,199	$ 76,263	$ 308,265	$ 262,925

*In the fourth quarter of 2025, PTC changed its estimates for its sales allowance related to Translarna revenues in France. The $98.6 million change in sales allowance estimate represents a life-to-date adjustment for the historical sales of Translarna in France. The 2024 amounts relate to historical Translarna sales recorded in France.

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

	December 31, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 1,945,371	$ 1,139,696
Total assets	**$ 2,898,767**	**$ 1,705,024**
Total debt	$ 286,631	$ 285,412
Total deferred revenue	2,040	5,505
Total liability for sale of future royalties	2,308,366	2,081,776
Total liabilities	**$ 3,104,080**	**$ 2,803,095**
Total stockholders' deficit (81,474,366 and 77,704,188 common shares issued and outstanding at December 31, 2025 and December 31, 2024, respectively)	$ (205,313)	$ (1,098,071)
Total liabilities and stockholders' deficit	**$ 2,898,767**	**$ 1,705,024**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2026 R&D and SG&A Expense
(in millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 775	$ 815
Less: projected non-cash, stock-based compensation expense	95	95
Projected non-GAAP R&D and SG&A expense	$ 680	$ 720

Acronyms
CHF: Confoederatio Helvetica Francs (Swiss francs)
DMD: Duchenne muscular dystrophy
FA: Friedreich's ataxia
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's disease
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. 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 after completion of the call and will be archived on the company's website.

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, Instagram 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 "Full Year 2026 Financial Guidance", including with respect to (i) 2026 total product revenue guidance and (ii) 2026 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 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 geographies in which it has been approved and the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) on Translarna and the withdrawal of the Translarna NDA in the U.S. on other regulatory bodies; 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 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 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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