PTC Therapeutics Provides Update at J.P. Morgan Annual Healthcare Conference

January 12, 2026

– Strong Sephience™ (sepiapterin) launch continues with unaudited Q4 global revenue of $92.5M –

– Unaudited 2025 total product and royalty revenue of approximately $823M, exceeding guidance –

– 2026 product revenue guidance of $700 – 800M, representing 19 – 36% year-over-year growth –

– Strong cash position of approximately $1.94B as of December 31 –

WARREN, N.J., Jan. 12, 2026 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided an update on the Company's progress and its outlook for 2026. Matthew B. Klein, M.D., Chief Executive Officer of PTC, will discuss these updates at the Company's presentation at the J.P. Morgan 2026 Healthcare Conference on Monday, January 12, 2026, at 9:00 a.m. PST / 12:00 p.m. EST.

"2025 was a highly successful year for PTC, highlighted by the initial regulatory approvals of Sephience and strong start to the global launch," Dr. Klein said. "In 2026, we look forward to continuing the Sephience launch momentum, advancing our innovative earlier-stage programs and moving the company towards becoming cash flow breakeven."

Key Corporate Highlights

- Unaudited 2025 product and royalty revenue of approximately $823.4 million, exceeding guidance, and unaudited 2025 product revenue of approximately $587.8 million
- Unaudited Sephience Q4 2025 net revenue of approximately $92.5 million, including $81.6 million in the US and $10.9 million ex-US
    - Unaudited Sephience total net revenue of approximately $112.1 million in 2025 since launch
    - 1,134 patient start forms received in the US as of December 31, 2025
    - 946 total patients on commercial therapy worldwide as of December 31, 2025
    - Additional Sephience launches expected in 2026, including in Japan, Brazil and other geographies
- 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 based on single-year Evrysdi sales of $2.5 billion by Roche
- Cash, cash equivalents, and marketable securities of approximately $1.94 billion as of December 31, 2025
- 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, planned to initiate in H1 2026
    - FDA confirmed openness to potential Accelerated Approval pathway given significant unmet need
- Type C meeting with FDA held in December 2025 to discuss vatiquinone Friedreich's ataxia program; FDA requested additional information from MOVE-FA Phase 3 trial prior to providing guidance on next steps
- Translarna ™ (ataluren) NDA remains under FDA review

Unaudited 2025 Financial Results

- Total unaudited product and royalty revenue for full-year 2025 was approximately $823.4 million
- Total unaudited product revenue for full-year 2025 was approximately $587.8 million
- Sephience unaudited total net revenue for 2025 was approximately $112.1 million
- DMD franchise unaudited revenue for full-year 2025 was approximately $381.8 million, including unaudited product revenue for Translarna of approximately $235.5 million and for Emflaza® (deflazacort) of approximately $146.3 million
- PTC expects to report approximately $235.7 million of full-year 2025 royalty revenue associated with Evrysdi

PTC is finalizing its financial results for the 2025 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2025, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on internal estimates and pending accounting treatment of Evrysdi royalty following the December 2025 transaction.

2026 Financial Guidance
For the full year 2026, PTC anticipates:

- Total product revenue of $700 to $800 million, excluding Evrysdi royalty revenue and collaboration revenue
- 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 and financial guidance of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP 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.

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:

DMD: Duchenne Muscular Dystrophy
FA: Friedreich's ataxia
FDA: 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

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and X.

For More Information:

Investors:
Ellen Cavaleri
+1 (615) 618-6228
ecavaleri@ptcbio.com

Media:
Jeanine Clemente
+1 (908) 912-9406
jclemente@ptcbio.com

Forward-Looking Statement
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 "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 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 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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