PTC Therapeutics Provides Corporate Update and Reports First Quarter 2026 Financial Results

May 7, 2026

– First quarter 2026 total revenue of $273 million, including $226 million of product revenue, supporting full-year 2026 guidance raise –

– Global Sephience™ (sepiapterin)launch momentum continues with first quarter 2026 revenue of $125 million, representing 36% quarter-over-quarter growth –

– Positive topline results from 24-month interim analysis of PIVOT-HD extension study of votoplam, supporting ongoing global Phase 3 INVEST-HD study and potential regulatory interactions –

– Open-label vatiquinone registration study to be initiated in Q3 2026 based on FDA feedback –

WARREN, N.J., May 7, 2026 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ended March 31, 2026.

"We are off to a strong start to 2026 with outstanding revenue performance this quarter that supports raising our full-year product revenue guidance," said Matthew B. Klein, M.D., Chief Executive Officer. "The Sephience launch continues to be strong, with sustained momentum in the US and growing momentum internationally as more countries contribute to the global launch."

Key Corporate Updates

- Q1 2026 total revenue of $273 million, with $226 million of product revenue
- Sephience global launch continues strong momentum
  - Q1 2026 revenue of $125 million, including $112 million revenue in the US and $13 million revenue ex-US, up 36% from Q4 2025
  - 1,244 total patients on commercial therapy worldwide as of March 31, 2026
  - Continued strong cadence of patient start forms in the US, with ~140 per month over past several months, surpassing 1,500 mark in the quarter
  - First commercial sales in Japan in Q1 2026, with revenue expected from up to 30 countries by year-end 2026
- Reported positive topline results in April 2026 from 24-month interim analysis of PIVOT-HD long-term extension study of votoplam, an oral small molecule splicing agent
  - Dose-dependent benefit on cUHDRS in Stage 2 participants relative to natural history cohort, with 52% slowing of disease progression at 10 mg dose
  - Continued favorable safety and tolerability with no treatment-related NfL spikes
  - PTC and Novartis will complete data review and align on potential regulatory interactions
  - Findings support ongoing global Phase 3 INVEST-HD study of votoplam led by Novartis
  - First patient dosed in Phase 3 INVEST-HD study, triggering $50 million milestone payment from Novartis to PTC in Q2 2026
- Type C meeting with FDA held in April 2026 to discuss vatiquinone study to support NDA resubmission for Friedreich's ataxia program
  - Based on meeting discussion and written feedback, PTC expects to initiate an open-label study using matched natural history control in Q3 2026
  - Primary endpoint will be change in mFARS from baseline to 24 months

First Quarter 2026 Financial Highlights

- Total net product revenue and royalty revenue was $272.4 million for the first quarter of 2026, compared to $189.9 million for the first quarter of 2025.
- Total net product revenue across the commercial portfolio was $225.6 million for the first quarter of 2026, compared to $153.4 million for the first quarter of 2025, representing a 47% increase.
- Sephience net product revenues were $124.6 million for the first quarter of 2026, representing 36% growth compared to fourth quarter of 2025.
- Translarna™ (ataluren) net product revenues were $59.0 million for the first quarter of 2026, compared to $86.2 million for the first quarter of 2025. Translarna first quarter of 2026 revenue includes a large government purchase order from Brazil.
- Emflaza® (deflazacort) net product revenues were $21.5 million for the first quarter of 2026, compared to $47.8 million for the first quarter of 2025, due to continued generic erosion.
- Roche reported Evrysdi® (risdiplam) sales of approximately 464 CHF million, resulting in royalty revenue of $46.8 million to

PTC for the first quarter of 2026, compared to $36.4 million to PTC for the first quarter of 2025.

- Based on US GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $100.9 million for the first quarter of 2026, compared to $109.0 million for the first quarter of 2025.
- Non-GAAP R&D expenses were $89.7 million for the first quarter of 2026, excluding $11.1 million in non-cash, stock-based compensation expense, compared to $100.3 million for the first quarter of 2025, excluding $8.7 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $86.2 million for the first quarter of 2026, compared to $81.0 million for the first quarter of 2025.
- Non-GAAP SG&A expenses were $73.9 million for the first quarter of 2026, excluding $12.3 million in non-cash, stock-based compensation expense, compared to $71.6 million for the first quarter of 2025, excluding $9.4 million in non-cash, stock-based compensation expense.
- Net loss was $2.8 million for the first quarter of 2026, compared to net income of $866.6 million for the first quarter of 2025.
- Cash, cash equivalents, and marketable securities were $1,892.5 million on March 31, 2026, compared to $1,945.4 million on December 31, 2025.
- Shares issued and outstanding as of March 31, 2026, were 82,882,024.

PTC Updates Full-Year 2026 Financial Guidance

- Total product revenue guidance raised to $750 to $850 million, with expected total revenue of $1.08 to $1.18 billion
- GAAP R&D and SG&A expense guidance remains $775 to $815 million
- Non-GAAP R&D and SG&A expense guidance remains $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 March 31,	
	2026	**2025**
Revenues:		
Net product revenue	$ 225,573	$ 153,426
Collaboration and license revenue	143	986,231
Royalty revenue	46,835	36,439
Total revenues	272,551	1,176,096
Operating expenses:		
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	28,028	12,862
Amortization of acquired intangible assets	11,581	3,798
Research and development (1)	100,873	108,973
Selling, general and administrative (2)	86,183	80,961
Change in the fair value of contingent consideration	-	(800)
Tangible asset impairment and losses on transactions, net	927	77
Total operating expenses	227,592	205,871
Income from operations	44,959	970,225
Interest expense, net	(49,030)	(34,092)
Other income (expense), net	1,609	(6,305)
(Loss) income before income tax expense	(2,462)	929,828
Income tax expense	(347)	(63,266)
Net (loss) income attributable to common stockholders	$ (2,809)	$ 866,562
Weighted-average shares outstanding:		
Basic (in shares)	82,521,023	78,115,836
Diluted (in shares)	82,521,023	86,385,922

Net (loss) income per share—basic (in dollars per share)	$ (0.03)	$ 11.09
Net (loss) income per share—diluted (in dollars per share)	$ (0.03)	$ 10.04

(1) Research and development reconciliation		
GAAP research and development	$ 100,873	$ 108,973
Less: share-based compensation expense	11,129	8,663
Non-GAAP research and development	$ 89,744	$ 100,310

(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 86,183	$ 80,961
Less: share-based compensation expense	12,292	9,397
Non-GAAP selling, general and administrative	$ 73,891	$ 71,564

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

	March 31, 2026	December 31, 2025
Cash, cash equivalents and marketable securities	$ 1,892,463	$ 1,945,371
Total assets	**$ 2,869,104**	**$ 2,898,767**
Total debt	$ 286,937	$ 286,631
Total deferred revenue	498	2,040
Total liability for sale of future royalties	2,327,230	2,308,366
Total liabilities	**$ 3,049,569**	**$ 3,104,080**
Total stockholders' deficit (82,882,024 and 81,474,366 common shares issued and outstanding at March 31, 2026, and December 31, 2025, respectively)	$ (180,465)	$ (205,313)
Total liabilities and stockholders' deficit	**$ 2,869,104**	**$ 2,898,767**

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)
cUHDRS: Composite Unified Huntington's Disease Rating Scale
DMD: Duchenne muscular dystrophy
FA: Friedreich's ataxia
FDA: US Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's disease
mFARS: Modified Friedreich's Ataxia Rating Scale
NDA: New Drug Application
NfL: Neurofilament light chain
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 live webcast, please visit the "Events & Presentations" page within the Investors section of the PTC website. A replay of the webcast will be available on the PTC website for 30 days following the event. To participate via phone, please register in advance here to receive dial-in details.

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, Facebook and Instagram.

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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 2026 Financial Guidance", including with respect to (i) 2026 total product revenue guidance and total 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 US 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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