



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

July 30, 2026

– Robust Q2 performance with total revenue of \$361 million, including \$239 million of product revenue –

– Full-year 2026 expected total revenue increased to \$1.18 to \$1.28 billion, with full-year product revenue guidance raised to \$850 to \$950 million –

– Sephience™ (sepiapterin) Q2 2026 revenue of \$151 million, with continued broad uptake and geographical expansion –

– Strong cash position of \$2.2 billion as of June 30, 2026 –

WARREN, N.J., July 30, 2026 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ended June 30, 2026.

"Our teams delivered another strong quarter enabling us to again raise 2026 product revenue guidance," said Matthew B. Klein, M.D., Chief Executive Officer. "We also made a number of advances in our R&D pipeline, including identification of our next small molecule splicing development candidate, PTC303, targeting somatic expansion disorders including Huntington's disease and myotonic dystrophy. In addition, we initiated the Phase 1 study of PTC612, our differentiated NLRP3 small molecule."

Key Corporate Updates

- Q2 2026 total revenue of \$361 million, including \$239 million of product revenue
- Sephience global launch continues with strong momentum
 - Q2 2026 revenue of \$151 million, with the majority from the US and increasing contributions internationally
 - 1,647 patients on commercial therapy worldwide as of June 30, 2026
- Positive results from voptoplam PIVOT-HD long-term extension study reported and enrollment in global Phase 3 INVEST-HD study ongoing
 - PIVOT-HD 24-month results demonstrated dose-dependent disease slowing on cUHRS scale in Stage 2 participants, reaching 52% at 10 mg dose level
 - Plan to engage with FDA in 2H 2026 to discuss PIVOT-HD 24-month results being finalized
 - INVEST-HD study being conducted and funded by partner Novartis with target enrollment of approximately 770 individuals with early symptomatic disease
 - INVEST-HD initiation triggered a \$50 million milestone payment from Novartis to PTC in Q2 2026
- Vatinquinone PROVE-FA study to support NDA resubmission for treatment of Friedreich's ataxia expected to initiate in Q3 2026
 - Global study will enroll approximately 120 individuals with Friedreich's ataxia age 7 to 21
 - Study includes open-label treatment arm with matched natural history control group
- PTC pipeline continues to advance
 - PTC303 named as MSH3 splicing program development candidate targeting Huntington's disease and myotonic dystrophy with plan to enter clinic in 2027
 - Phase 1 study of PTC612, an oral NLRP3 inhibitor, initiated in Q2 2026
 - Phase 2a study of PTC844, a next-generation DHODH inhibitor, expected to initiate in Q3 2026
- Hege Sollie-Zetlmayer has been appointed to PTC's Board of Directors. Ms. Sollie-Zetlmayer has over 30 years of experience in the biopharmaceutical and medical device industry as a global business operations and human resources leader. She most recently served as PTC's Chief Human Resources Officer prior to her retirement in June 2026.

Second Quarter 2026 Financial Highlights

- Total net product revenue and royalty revenue was \$309.9 million for the second quarter of 2026, compared to \$175.9 million for the second quarter of 2025.
- Total net product revenue across the commercial portfolio was \$238.8 million for the second quarter of 2026, compared to \$118.3 million for the second quarter of 2025, representing over 100% increase.
- Sephience net product revenues were \$151.3 million for the second quarter of 2026, representing 21% growth compared to the first quarter of 2026.
- Translarna™ (ataluren) net product revenues were \$42.2 million for the second quarter of 2026, compared to \$59.5 million for the second quarter of 2025.
- Emflaza® (deflazacort) net product revenues were \$24.6 million for the second quarter of 2026, compared to \$36.4 million for the second quarter of 2025, due to continued generic erosion.

- Roche reported Evrysdi® (risdiplam) year-to-date sales of approximately 968 CHF million, resulting in royalty revenue of \$71.1 million to PTC for the second quarter of 2026, compared to \$57.6 million to PTC for the second quarter of 2025.
- In the second quarter of 2026, PTC recorded a development milestone of \$50.0 million from Novartis for the first patient dosed in the ongoing INVEST-HD Phase 3 study. This sales milestone was recorded as collaboration revenue.
- Based on US GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were \$99.2 million for the second quarter of 2026, compared to \$113.0 million for the second quarter of 2025.
- Non-GAAP R&D expenses were \$88.6 million for the second quarter of 2026, excluding \$10.5 million in non-cash, stock-based compensation expense, compared to \$104.0 million for the second quarter of 2025, excluding \$9.0 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were \$80.6 million for the second quarter of 2026, compared to \$85.3 million for the second quarter of 2025.
- Non-GAAP SG&A expenses were \$67.9 million for the second quarter of 2026, excluding \$12.7 million in non-cash, stock-based compensation expense, compared to \$75.7 million for the second quarter of 2025, excluding \$9.5 million in non-cash, stock-based compensation expense.
- Net income was \$83.5 million for the second quarter of 2026, compared to net loss of \$64.8 million for the second quarter of 2025.
- In June 2026, PTC issued \$550.0 million of senior convertible notes due in 2031 at 0% coupon and a conversion price representing a 40% premium over the stock's closing price at the time of the deal. Concurrent with the transaction, PTC repurchased the majority of its 1.5% senior convertible notes due in September 2026.
- Cash, cash equivalents, and marketable securities were \$2,229.1 million on June 30, 2026, compared to \$1,945.4 million on December 31, 2025.
- Shares issued and outstanding as of June 30, 2026, were 83,327,286.

PTC Updates Full-Year 2026 Financial Guidance

- Expected total revenue increased to \$1.18 to \$1.28 billion, with total product revenue guidance raised to \$850 to \$950 million from \$750 to \$850 million
- 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 June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenues:				
Net product revenue	\$ 238,819	\$ 118,329	\$ 464,392	\$ 271,755
Collaboration and license revenue	50,595	2,941	50,738	989,172
Royalty revenue	71,105	57,605	117,940	94,044
Total revenues	360,519	178,875	633,070	1,354,971
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	19,921	11,420	47,949	24,282
Amortization of acquired intangible assets	11,841	4,061	23,422	7,859
Research and development (1)	99,150	112,990	200,023	221,963
Selling, general and administrative (2)	80,630	85,262	166,813	166,223
Change in the fair value of contingent consideration	-	-	-	(800)
Tangible asset impairment and losses on transactions, net	-	99	927	176
Total operating expenses	211,542	213,832	439,134	419,703
Income (loss) from operations	148,977	(34,957)	193,936	935,268

Interest expense, net	(48,481)	(30,358)	(97,511)	(64,450)
Other expense, net	(2,951)	(5,737)	(1,342)	(12,042)
Income (loss) before income tax (expense) benefit	97,545	(71,052)	95,083	858,776
Income tax (expense) benefit	(14,049)	6,203	(14,396)	(57,063)
Net income (loss) attributable to common stockholders	<u>\$ 83,496</u>	<u>\$ (64,849)</u>	<u>\$ 80,687</u>	<u>\$ 801,713</u>

Weighted-average shares outstanding:

Basic (in shares)	<u>83,006,808</u>	<u>78,151,240</u>	<u>82,765,248</u>	<u>78,438,830</u>
Diluted (in shares)	<u>92,020,009</u>	<u>78,151,240</u>	<u>91,824,273</u>	<u>86,502,578</u>
Net income (loss) per share—basic (in dollars per share)	<u>\$ 1.01</u>	<u>\$ (0.83)</u>	<u>\$ 0.97</u>	<u>\$ 10.22</u>
Net income (loss) per share—diluted (in dollars per share)	<u>\$ 0.92</u>	<u>\$ (0.83)</u>	<u>\$ 0.90</u>	<u>\$ 9.29</u>

(1) Research and development reconciliation

GAAP research and development	\$ 99,150	\$ 112,990	\$ 200,023	\$ 221,963
Less: share-based compensation expense	10,547	9,030	21,677	17,693
Non-GAAP research and development	<u>\$ 88,603</u>	<u>\$ 103,960</u>	<u>\$ 178,346</u>	<u>\$ 204,270</u>

(2) Selling, general and administrative reconciliation

GAAP selling, general and administrative	\$ 80,630	\$ 85,262	\$ 166,813	\$ 166,223
Less: share-based compensation expense	12,744	9,513	25,035	18,910
Non-GAAP selling, general and administrative	<u>\$ 67,886</u>	<u>\$ 75,749</u>	<u>\$ 141,778</u>	<u>\$ 147,313</u>

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

	<u>June 30, 2026</u>	<u>December 31, 2025</u>
Cash, cash equivalents and marketable securities	\$ 2,229,082	\$ 1,945,371
Total Assets	<u>\$ 3,250,477</u>	<u>\$ 2,898,767</u>
Total debt	\$ 590,970	\$ 286,631
Total deferred revenue	1,192	2,040
Total liability for sale of future royalties	2,321,434	2,308,366
Total liabilities	<u>\$ 3,415,840</u>	<u>\$ 3,104,080</u>
Total stockholders' deficit (83,327,286 and 81,474,366 common shares issued and outstanding at June 30, 2026, and December 31, 2025, respectively)	\$ (165,363)	\$ (205,313)
Total liabilities and stockholders' deficit	<u>\$ 3,250,477</u>	<u>\$ 2,898,767</u>

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

	<u>Low End of Range</u>	<u>High End of Range</u>
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	<u>\$ 680</u>	<u>\$ 720</u>

Acronyms

CHF: Confoederatio Helvetica Francs (Swiss francs)
cUHDS: Composite Unified Huntington's Disease Rating Scale
DMD: Duchenne muscular dystrophy
FA: Friedrich's ataxia
FDA: US Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's disease
NDA: New Drug Application
nmDMD: Nonsense mutation Duchenne muscular dystrophy
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 Sepience, including 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 vutolam 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; expectations regarding PTC's product candidates, including the timing of clinical trials and studies; 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 Sepience, 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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