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

October 29, 2026

– Strong Q3 2026 performance with total revenue of $358 million, including $276 million of product revenue –

– Full-year 2026 expected total revenue increased to $1.29 to $1.32 billion, with full-year product revenue guidance raised to $960 to $990 million –

– Sephience™ (sepiapterin) Q3 2026 revenue of $182 million, with continued growth in the US and ongoing global expansion –

– Acquired ST-920 Fabry disease gene therapy; BLA submission expected to be completed in Q4 2026 –

WARREN, N.J., Oct. 29, 2026 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ended September 30, 2026.

"We delivered another strong quarter of record product revenue led by the continued momentum of the Sephience launch, putting us firmly on track to achieve our cash flow breakeven milestone this year," said Matthew B. Klein, M.D., Chief Executive Officer. "Now at the one-year mark, the launch has been a success by all measures. With a strong patient base, the large global market, and the reports of meaningful treatment benefits, we look forward to continued growth both in the US and internationally."

Key Corporate Updates

- Q3 2026 total revenue of $358 million, including $276 million of product revenue
- Sephience launch momentum continues with steady growth in the US and ongoing global expansion
  - Q3 2026 revenue of $182 million, including $145 million revenue in the US and $37 million revenue ex-US, up over 20% from Q2 2026
  - Over 2,000 active patients worldwide as of September 30, 2026
- ST-920 Fabry disease BLA submission expected to be completed in Q4 2026
  - ST-920 is a one-time administered AAV gene therapy with the potential to reduce the burden associated with chronic enzyme replacement therapy
  - Potential FDA accelerated approval and commercial launch in 2027
- Votoplam Huntington's disease program continues to advance
  - Due to FDA scheduling, Type C meeting to discuss votoplam Phase 2 data and potential accelerated approval path now scheduled for Q1 2027
  - Global Phase 3 INVEST-HD study actively enrolling at sites worldwide with target enrollment of approximately 770 early symptomatic patients
- Vatiquinone PROVE-FA study to support NDA resubmission for treatment of Friedreich's ataxia initiated in Q3 2026
  - Global study will enroll approximately 120 patients ages 7 to 21
  - Study includes open-label treatment arm with matched natural history control arm
- PTC pipeline advances across multiple programs
  - Initiated Phase 2a study of PTC844, a next-generation DHODH inhibitor
  - Ongoing Phase 1 study of PTC612, an oral NLRP3 inhibitor, continues to advance
  - PTC303, a splicing development candidate targeting MSH3, expected to enter clinic in 2027

Third Quarter 2026 Financial Highlights

- Total net product revenue and royalty revenue was $357.6 million for the third quarter of 2026, compared to $201.7 million for the third quarter of 2025.
- Total net product revenue across the commercial portfolio was $275.9 million for the third quarter of 2026, compared to $131.0 million for the third quarter of 2025, representing an over 100% increase.
- Sephience net product revenues were $181.9 million for the third quarter of 2026, representing 20% growth compared to the second quarter of 2026.
- Translarna™ (ataluren) net product revenues were $48.5 million for the third quarter of 2026, compared to $50.7 million for the third quarter of 2025. Translarna third quarter 2026 revenue includes a government purchase order from Russia.
- Emflaza® (deflazacort) net product revenues were $25.1 million for the third quarter of 2026, compared to $35.2 million for the third quarter of 2025.
- Roche reported Evrysdi® (risdiplam) year-to-date sales of approximately CHF 1,470 million, resulting in royalty revenue of $81.7 million to PTC for the third quarter of 2026, compared to $70.8 million to PTC for the third quarter of 2025.
- Based on US GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $105.4 million for the third

quarter of 2026, compared to $100.2 million for the third quarter of 2025.

- Non-GAAP R&D expenses were $94.9 million for the third quarter of 2026, excluding $10.5 million in non-cash, stock-based compensation expense, compared to $91.0 million for the third quarter of 2025, excluding $9.1 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $80.1 million for the third quarter of 2026, compared to $84.0 million for the third quarter of 2025.
- Non-GAAP SG&A expenses were $66.7 million for the third quarter of 2026, excluding $13.3 million in non-cash, stock-based compensation expense, compared to $73.8 million for the third quarter of 2025, excluding $10.3 million in non-cash, stock-based compensation expense.
- Net loss was $51.7 million for the third quarter of 2026, compared to net income of $15.9 million for the third quarter of 2025.
- In Q3 2026, the Company acquired the ST-920 gene therapy for Fabry disease for $111.0 million upfront, with up to an additional $100.0 million in contingent milestone payments based on US regulatory approvals. Acquired in-process research and development expense was $111.0 million for the third quarter of 2026 and related to the purchase of the ST-920 program.
- Cash, cash equivalents, and marketable securities were $2,127.9 million on September 30, 2026, compared to $1,945.4 million on December 31, 2025.
- Shares issued and outstanding as of September 30, 2026, were 83,535,767.

PTC Updates Full-Year 2026 Financial Guidance

- Expected total revenue increased to $1.29 to $1.32 billion, with total product revenue guidance raised to $960 to $990 million from $850 to $950 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 September 30,		Nine Months Ended September 30,	
	2026	2025	2026	2025
Revenues:				
Net product revenue	$ 275,894	$ 130,956	$ 740,286	$ 402,711
Collaboration and license revenue	229	9,258	50,967	998,430
Royalty revenue	81,684	70,793	199,624	164,837
Total revenues	357,807	211,007	990,877	1,565,978
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	23,664	15,781	71,613	40,063
Amortization of acquired intangible assets	12,595	7,534	36,017	15,393
Research and development (1)	105,353	100,158	305,376	322,121
Selling, general and administrative (2)	80,081	84,046	246,894	250,269
Change in the fair value of contingent consideration	-	-	-	(800)
Tangible asset impairment and losses on transactions, net	-	472	927	648
Acquired in-process research and development *	111,000	-	111,000	-
Total operating expenses	332,693	207,991	771,827	627,694
Income from operations	25,114	3,016	219,050	938,284
Interest expense, net	(45,866)	(32,592)	(143,377)	(97,042)
Other (expense) income, net	(1,073)	6,502	(2,415)	(5,540)
Loss on extinguishment of debt	(19,263)	-	(19,263)	-

(Loss) income before income tax (expense) benefit	(41,088)	(23,074)	53,995	835,702
Income tax (expense) benefit	(10,605)	38,970	(25,001)	(18,093)
Net (loss) income attributable to common stockholders	$ (51,693)	$ 15,896	$ 28,994	$ 817,609
Weighted-average shares outstanding:				
Basic (in shares)	83,476,100	78,471,119	83,004,625	78,642,478
Diluted (in shares)	83,476,100	87,115,369	92,253,675	86,899,727
Net (loss) income per share—basic (in dollars per share)	$ (0.62)	$ 0.20	$ 0.35	$ 10.40
Net (loss) income per share—diluted (in dollars per share)	$ (0.62)	$ 0.20	$ 0.34	$ 9.45
(1) Research and development reconciliation				
GAAP research and development	$ 105,353	$ 100,158	$ 305,376	$ 322,121
Less: share-based compensation expense	10,479	9,133	32,155	26,827
Non-GAAP research and development	$ 94,874	$ 91,025	$ 273,221	$ 295,294
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 80,081	$ 84,046	$ 246,894	$ 250,269
Less: share-based compensation expense	13,348	10,293	38,384	29,202
Non-GAAP selling, general and administrative	$ 66,733	$ 73,753	$ 208,510	$ 221,067

* In Q3 2026, the Company purchased the ST-920 asset from Sangamo Therapeutics, Inc. and paid an upfront cash consideration of $111.0 million which was recorded as Acquired in-process research and development expense.

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

	September 30, 2026	December 31, 2025
Cash, cash equivalents and marketable securities	$ 2,127,918	$ 1,945,371
Total Assets	**$ 3,207,715**	**$ 2,898,767**
Total debt	$ 536,268	$ 286,631
Total deferred revenue	1,975	2,040
Total liability for sale of future royalties	2,305,603	2,308,366
Total liabilities	**$ 3,398,924**	**$ 3,104,080**
Total stockholders' deficit (83,535,767 and 81,474,366 common shares issued and outstanding at September 30, 2026 and December 31, 2025, respectively)	$ (191,209)	$ (205,313)
Total liabilities and stockholders' deficit	**$ 3,207,715**	**$ 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
AAV: adeno-associated virus
BLA: Biologics License Application
CHF: Confoederatio Helvetica Francs (Swiss francs)
DHODH: dihydroorotate dehydrogenase
FA: Friedreich's ataxia
FDA: US Food and Drug Administration

GAAP: Generally Accepted Accounting Principles
HD: Huntington's disease
MSH3: MutS Homolog 3 (human gene)
NDA: New Drug Application
NLRP3: NOD-, LRR- and pyrin domain-containing 3 (multi-protein complex)
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 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 ST-920, including the expected timing of regulatory submissions and potential approvals, and the potential achievement of regulatory milestones and contingent payments PTC may be obligated to make; 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 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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