UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **August 7, 2025**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

500 Warren Corporate Center Drive	
Warren, NJ	**07059**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code: **(908) 222-7000**

Not applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02. Results of Operations and Financial Condition.

On August 7, 2025, PTC Therapeutics, Inc. (the "Company") announced its financial results for the quarter ended June 30, 2025. The full text of the press release issued in connection with the announcement is furnished as Exhibit 99.1 to this Current Report on Form 8-K (this "Report") and is incorporated by reference into this Item 2.02.

Item 7.01. Regulation FD Disclosure.

The Company will host a conference call on August 7, 2025, at 4:30 p.m. eastern time, as previously announced. During this call the Company expects to review financial results for the quarter ended June 30, 2025, as well as other corporate highlights and updates. Instructions on how to access the conference call are included in the press release furnished as Exhibit 99.1 hereto.

The information in this Report (including Items 2.02 and 7.01 and Exhibit 99.1) shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated August 7, 2025 issued by PTC Therapeutics, Inc.
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: August 7, 2025

By: /s/ Pierre Gravier
Name: Pierre Gravier
Title: Chief Financial Officer

PTC Therapeutics Provides Corporate Update and Reports Second Quarter 2025 Financial Results

– European and FDA approval of Sephience™ (sepiapterin) with broad labeling for PKU –

– Global launch underway in Europe and U.S. –

– Total Q2 Revenue of $179M –

WARREN, N.J., August 7, 2025 – PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ended June 30, 2025.

"We had another strong quarter highlighted by the first approvals of Sephience for the treatment of children and adults with PKU," said Matthew B. Klein, M.D., Chief Executive Officer. "We have initiated the global launch and expect Sephience to be the foundational product for PTC's future growth and path to profitability."

Key Corporate Updates:

- Second quarter 2025 total net product, collaboration and royalty revenue of $179 million
- Second quarter 2025 revenue for the DMD franchise of $96 million, including net product revenue for Translarna™ of $59 million and for Emflaza® of $36 million
- Initiated global launch of Sephience™ in the U.S. and Germany as well as in other countries through early access and named patient programs
- Entered into agreement to purchase the Sephience annual percentage-based global net sales obligation owed to former Censa shareholders in exchange for an upfront payment of $225 million and future sales milestone payments

Key Clinical and Regulatory Milestones:

- Sephience™ (sepiapterin)
  - Marketing authorization granted by the EC on June 19, 2025 with broad label inclusive of all disease subtypes and all ages
  - FDA approval on July 28, 2025 with broad label inclusive of all disease subtypes and all ages, from 1 month of age upwards
  - Japan NDA review is ongoing with decision expected in Q4 2025
- NDA reviews for vatiquinone (Friedreich's ataxia) and Translarna (nonsense mutation DMD) are ongoing, with regulatory action date of August 19, 2025 for vatiquinone
- In May 2025, reported positive Phase 2 PIVOT-HD study results for votoplam (PTC518) in Huntington's Disease patients. PTC continues to collaborate with Novartis on next steps and aims to meet with FDA in Q4 2025 to discuss Phase 3 clinical trial design and potential accelerated approval pathway.

Second Quarter 2025 Financial Highlights:

- Total revenues were $178.9 million for the second quarter of 2025, compared to $186.7 million for the second quarter of 2024.
- Total revenue includes net product revenue across the commercial portfolio of $118.3 million for the second quarter of 2025, compared to $133.2 million for the second quarter of 2024. Total revenue also includes royalty, collaboration and license, and manufacturing revenue of $60.5 million in the second quarter of 2025, compared to $53.5 million for the second quarter of 2024.

- Translarna net product revenues were $59.5 million for the second quarter of 2025, compared to $70.4 million for the second quarter of 2024.
- Emflaza net product revenues were $36.4 million for the second quarter of 2025, compared to $47.3 million for the second quarter of 2024.
- Roche reported Evrysdi® first half 2025 sales of approximately 869 CHF million, resulting in royalty revenue of $57.6 million to PTC for second quarter 2025, as compared to $53.2 million for second quarter 2024.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $113.0 million for the second quarter of 2025, compared to $132.2 million for the second quarter of 2024.
- Non-GAAP R&D expenses were $104.0 million for the second quarter of 2025, excluding $9.0 million in non-cash, stock-based compensation expense, compared to $122.7 million for the second quarter of 2024, excluding $9.4 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $85.3 million for the second quarter of 2025, compared to $69.5 million for the second quarter of 2024.
- Non-GAAP SG&A expenses were $75.7 million for the second quarter of 2025, excluding $9.5 million in non-cash, stock-based compensation expense, compared to $59.7 million for the second quarter of 2024, excluding $9.8 million in non-cash, stock-based compensation expense.
- Net loss was $64.8 million for the second quarter of 2025, compared to net loss of $99.2 million for the second quarter of 2024.
- Cash, cash equivalents, and marketable securities were $1,989.2 million as of June 30, 2025, compared to $1,139.7 million as of December 31, 2024.
- Shares issued and outstanding as of June 30, 2025, were 79,378,145.

PTC Full-Year 2025 Financial Guidance:

- PTC anticipates full-year 2025 revenue to be between $650 million and $800 million, which includes in-line products, new and potential product launches, and royalty revenue from Evrysdi.
- PTC anticipates full-year 2025 GAAP R&D and SG&A expense to be between $805 and $835 million.
- PTC anticipates full-year 2025 non-GAAP R&D and SG&A expense to be between $730 and $760 million, excluding estimated non-cash, stock-based compensation expense of $75 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 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,	
	2025	**2024**	**2025**	**2024**
Revenues:				
Net product revenue	$ 118,329	$ 133,220	$ 271,755	$ 310,824
Collaboration and license revenue	2,941	-	989,172	-
Royalty revenue	57,605	53,183	94,044	84,337
Manufacturing revenue	-	301	-	1,661
Total revenues	178,875	186,704	1,354,971	396,822
Operating expenses:				
Cost of product, collaboration and license sales, excluding amortization of acquired intangible assets	11,420	15,527	24,282	30,267
Amortization of acquired intangible assets	4,061	2,865	7,859	54,395
Research and development (1)	112,990	132,169	221,963	248,298
Selling, general and administrative (2)	85,262	69,500	166,223	142,772
Change in the fair value of contingent consideration	-	5,100	(800)	5,000
Tangible asset impairment and losses (gains) on transactions, net	99	1,761	176	1,761
Total operating expenses	213,832	226,922	419,703	482,493
(Loss) income from operations	(34,957)	(40,218)	935,268	(85,671)
Interest expense, net	(30,358)	(43,490)	(64,450)	(84,324)
Other expense, net	(5,737)	(2,025)	(12,042)	(434)
(Loss) income before income tax benefit (expense)	(71,052)	(85,733)	858,776	(170,429)
Income tax benefit (expense)	6,203	(13,446)	(57,063)	(20,326)
Net (loss) income attributable to common stockholders	$ (64,849)	$ (99,179)	$ 801,713	$ (190,755)
Weighted-average shares outstanding:				
Basic (in shares)	78,151,240	76,725,070	78,438,830	76,610,598
Diluted (in shares)	78,151,240	76,725,070	86,502,578	76,610,598
Net (loss) income per share—basic (in dollars per share)	$ (0.83)	$ (1.29)	$ 10.22	$ (2.49)
Net (loss) income per share—diluted (in dollars per share)	$ (0.83)	$ (1.29)	$ 9.29	$ (2.49)
(1) Research and development reconciliation				
GAAP research and development	$ 112,990	$ 132,169	$ 221,963	$ 248,298
Less: share-based compensation expense	9,030	9,428	17,693	18,395
Non-GAAP research and development	$ 103,960	$ 122,741	$ 204,270	$ 229,903
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 85,262	$ 69,500	$ 166,223	$ 142,772
Less: share-based compensation expense	9,513	9,815	18,910	19,226
Non-GAAP selling, general and administrative	$ 75,749	$ 59,685	$ 147,313	$ 123,546

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

	June 30, 2025	December 31, 2024
Cash, cash equivalents and marketable securities	$ 1,989,150	$ 1,139,696
Total Assets	**$ 2,634,155**	**$ 1,705,024**
Total debt	$ 286,013	$ 285,412
Total deferred revenue	9,760	5,505
Total liability for sale of future royalties	2,096,006	2,081,776
Total liabilities	**$ 2,840,701**	**$ 2,803,095**
Total stockholders' deficit (79,378,145 and 77,704,188 common shares issued and outstanding at June 30, 2025 and December 31, 2024, respectively)	$ (206,546)	$ (1,098,071)
Total liabilities and stockholders' deficit	**$ 2,634,155**	**$ 1,705,024**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2025 R&D and SG&A Expense
(In millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 805	$ 835
Less: projected non-cash, stock-based compensation expense	75	75
Projected non-GAAP R&D and SG&A expense	$ 730	$ 760

Acronyms:
CHF: Confoederatio Helvetica Francs (Swiss francs)
DMD: Duchenne Muscular Dystrophy
EC: European Commission
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
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. To avoid delays, we recommend participants dial in for the conference call 15 minutes prior to the start of the call. 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 approximately two hours after completion of the call and will be archived on the company's website for 30 days.

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 Facebook, X, and LinkedIn.

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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 Full Year 2025 Financial Guidance", including with respect to (i) 2025 total revenue guidance and (ii) 2025 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, 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; 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 including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; 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 Sephience, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of regulatory and sales milestones and contingent payments that PTC may be obligated to make; 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 most recent Quarterly Report on Form 10-Q and 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, Waylivra or vatiquinone.

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.