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): **October 29, 2026**

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 October 29, 2026, PTC Therapeutics, Inc. (the "Company") announced its financial results for the quarter ended September 30, 2026. 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 October 29, 2026 at 4:30 p.m. eastern time, as previously announced. During this call the Company expects to review financial results for the quarter ended September 30, 2026, 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 Company has also updated its corporate presentation as of October 29, 2026. The Company's corporate presentation will be posted in the Events and Presentations page under the Investors section of the Company's website. A copy of the corporate presentation is also attached to this Report as Exhibit 99.2 and is incorporated by reference into this Item 7.01.

The information in this Report (including Items 2.02 and 7.01 and Exhibits 99.1 and 99.2) 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 October 29, 2026 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – October 2026
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: October 29, 2026

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

Exhibit 99.1

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

– 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., October 29, 2026 – 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.

PTC Therapeutics

October 2026



PTC THERAPEUTICS

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Forward Looking Statements

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, ot statements of historic fact, are forward-looking statements, including with respect to 2026 total product revenue guidance and total revenue guidance and 2026 operating expense guida 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 sub and responses, meetings with regulatory agencies, commercialization and other matters with respect to its products and product candidates; PTC's strategy, future operations, future position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance," "plan," "anticipate," "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 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 Tra 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 treatme 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 clinic and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; expectations with respect to ST-920, including the expected timin regulatory submissions and potential approvals, and the potential achievement of regulatory milestones and contingent payments PTC may be obligated to make; expectations with respec 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 resp and potential approvals and other matters; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercializatio 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, mar 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 produc 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 reso 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" 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 fa

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Sephience, Translarna, Emflaza, Upstaza, Kebilidi, Evrysdi, Te Waylivra.

The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as rec law.

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PTC: A Global Leader in Rare Disease Drug Development and Commercialization

Robust global commercial infrastructure

Proven track record of achieving regulatory authorization, gaining market access and commercializing rare disease therapies in over 50 countries.

Lead product Sephience supports near- and mid-term growth

Strong initial global launch and positioned to be new standard of care for PKU. IP into 2039 and $2B+ global revenue potential.

Innovative R&D platforms and pipeline

Pioneered field of oral small molecule RNA splicing with proprietary platform engine to accelerate and facilitate next generation of oral small molecule splicing therapies.

Strong financial position

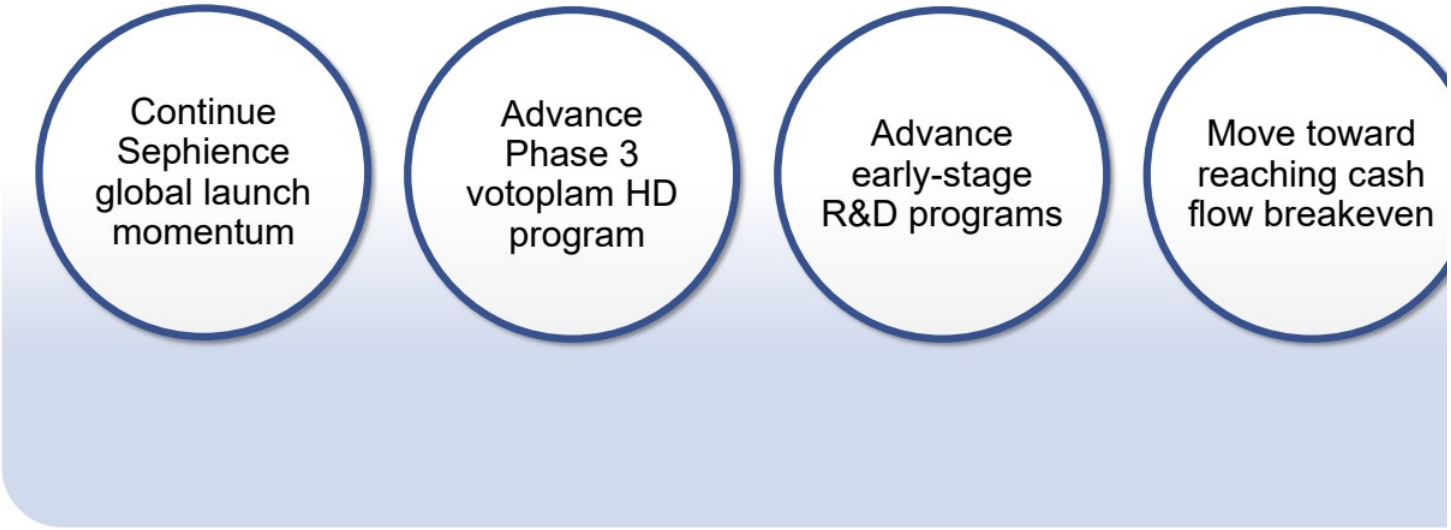
Cash balance of $2.1B[1] and on track to achieve 2026 cash flow breakeven and future sustainable profitability.

PTC THERAPEUTICS

[1]As of September 30, 2026

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2026 Strategic Priorities



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2026 Revenue and OpEx Guidance

Product Revenue[1] **$960 - 990M**

64 - 69% YoY Growth

Total Revenue[2] **$1.29 - 1.32B**

OpEx[3] **$680 - 720M**

Our Products[4]

Sephience™ sepiapterin

translarna™ ataluren

Emfla (deflaza

Kebilidi™ (eladocagene exuparvovec-tneq)

Upsta (eladocagene ex

waylivra® (volanesorsen)

Tegs (inotersen)

1 Product revenue excludes Evrysdi royalty and collaboration revenues; YoY based on $960-990M guidance.
2 Total revenue includes Evrysdi royalty and collaboration revenues.
3 OpEx is a non-GAAP measure that includes only R&D and SG&A expenses for full year 2026, and excludes estimated non-cash, stock-based compensation expense of approximately $95M. GAAP R&D and SG&A expense for full year 2026 is anticipated to be between $775 and $815M.
4 PTC holds the commercialization rights for Tegsedi and Waylivra in Latin America and the Caribbean from Ionis Pharmaceuticals.

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Sephience™
PKU Program



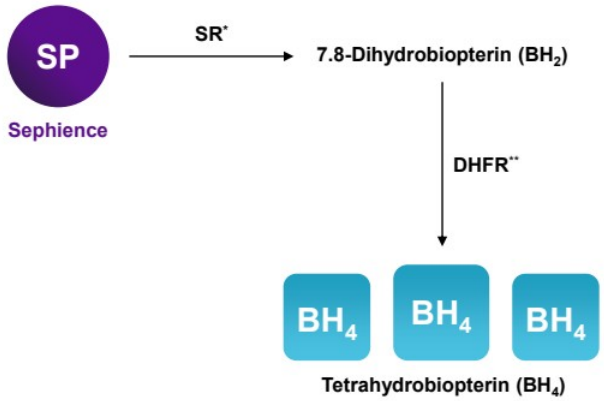
Katie, living with PKU

PTC THERAPEUTICS™

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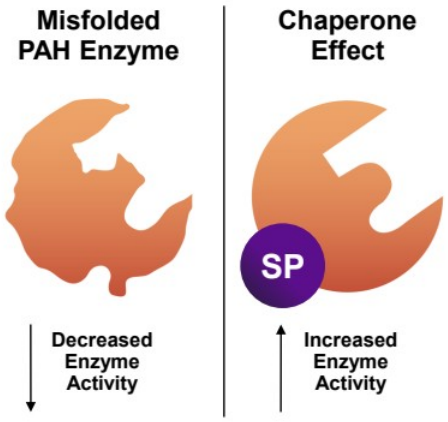
Sephience Dual Mechanism of Action Supports Potential for Broad Uptake

1. Precursor to BH_4



Sephience significantly increases intracellular BH_4 levels

2. Independent Chaperone Function

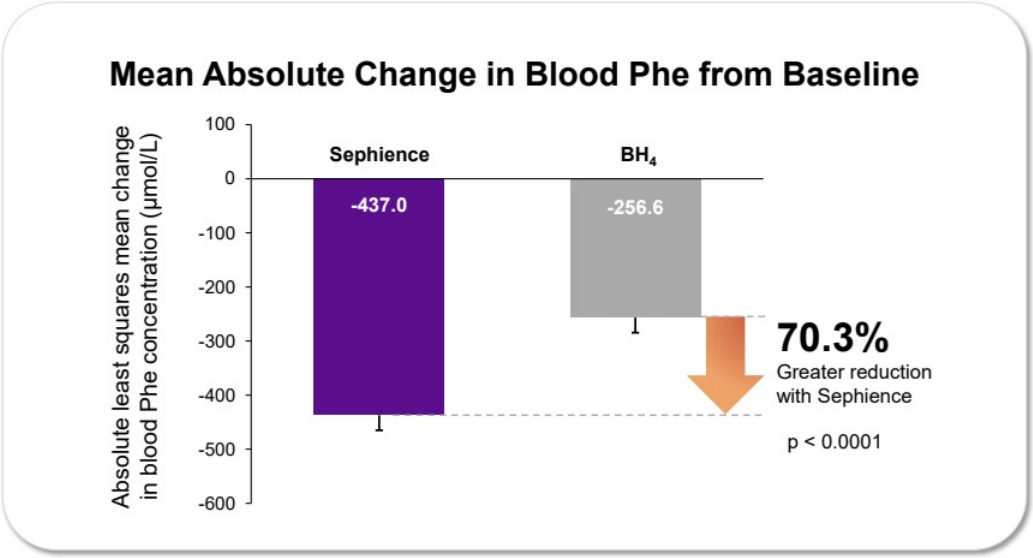


Chaperone effect stabilizes enzyme, increases func and enables treatment of BH_4-non-responsive mutat

* SR, sepiapterin reductase (enzyme that converts Sephience to BH_2)
** DHFR, dihydrofolate reductase (enzyme that converts BH_2 to BH_4)
Lah, M. Advances in Therapy (In press)

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Sephience Provides Significantly Greater Phe Reduction Compared With BH_4



AMPLIPHY was a crossover study allowing for intra-subject comparison of Sephience and BH_4 treatment effect

Lancet. 2024 Oct 5;404(10460):1333-1345. doi: 10.1016/S0140-6736(24)01556-3.

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Sephience Provides Diet Liberalization, Cognitive and Quality of Life Benefits

Meaningful diet liberalization

69% of subjects reached age-adjusted protein RDA

Improved cognitive function and mood

Improved concentration & slow thinking, and decreased irritability

Improved quality of life

Reduced disease impact on emotional, social, and familial wellbeing

Genet Med. 2026 Apr;28(4):101683. doi: 10.1016/j.gim.2026.101683. Epub 2026 Jan 12.

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Sephience Differentiated Profile Enables Penetratic Into All Key Market Segments



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Sephience: Strong Launch Momentum Continues



Broad penetration into all key market segments

$458M Global revenue YTD in first full year of launch

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* As of September 30, 2026

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Sephience Global Launch Footprint Expansion Planned for 2026 and 2027



Anticipated Sephience geographic footprint for 2026

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ST-920

Fabry Disease Program

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Fabry Disease is a Rare, Life-Shortening Disorder with Multiorgan Impact

- Lysosomal storage disorder causing serious organ damage, leading to premature death
- Affects ~11,000 individuals (US)
- Standard of care of enzyme replacement therapies (ERT) have several drawbacks
  - Frequent (up to biweekly) administration
  - Require immunosuppression
  - Risk of antibody formation limiting ERT benefits
- A one-time administered gene therapy has potential to provide benefits of ERT without current burdens and risks



Organ involveme includes kidney, he and brain

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ST-920[1] Provides One-Time Administered Durable Enzyme Production

- AAV gene therapy that provides humanized gene enabling long-term production of the deficient α-Gal A enzyme
- Favorable safety profile with no need for immunosuppression
- Durable α-Gal A activity with longest follow up of over 5 years
- Favorable effects on renal function and other disease aspects following gene therapy administration

BLA submission for accelerated approval expected to be completed in Q4 2026

1 Isaralgagene civaparvovec

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Votoplam

Huntington's Disease Program



April, living with HD

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Phase 2 PIVOT-HD Trial Achieved Key Efficacy and Safety Objectives

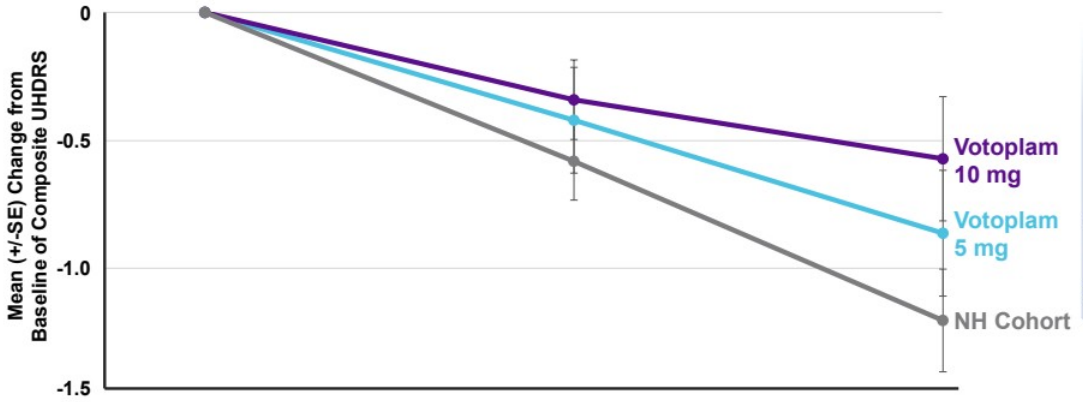
PIVOT-HD study met primary endpoint of blood HTT protein lowering at Week 12 with durable dose-dependent lowering at Month 12

Evidence of dose-dependent disease slowing on cUHDRS in Stage 2 participants at 24 months

Continued evidence of favorable safety profile with no new AE signals identified for both dose levels and stages at 24 months

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Dose-dependent Slowing of Disease Progression on cUHDRS at 24 months in Stage 2 Participants



Mean difference in cUHDRS progression 52% (10mg) and 28% (5 at Month 24

	Baseline	12 Month	24 Month
Treatment Group		**Mean (SE)**	**Mean (SE)**
Natural History (N=73)		-0.58 (0.15)	-1.20 (0.20)
5 mg (N=21)		-0.42 (0.16)	-0.86 (0.24)
10 mg (N=24)		-0.34 (0.21)	-0.57 (0.24)

Results based on observed data
SE = Standard Error

Results from PIVOT-HD LTE (April 2026)

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INVEST-HD Global Phase 3 Trial Enrolling Worldwide

Study Overview

- Individuals with early symptomatic disease
- 3:2 randomization of votoplam 10mg: placebo
- Target enrollment: ~770 participants in >30 countries
- Primary endpoint: Change from baseline in cUHDRS
- Treatment period up to 36 months
- Interim analysis planned for efficacy and futility

*Study sponsored and funded by Novartis; NCT#: NCT07326709

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Vatiquinone

Friedreich's Ataxia Program

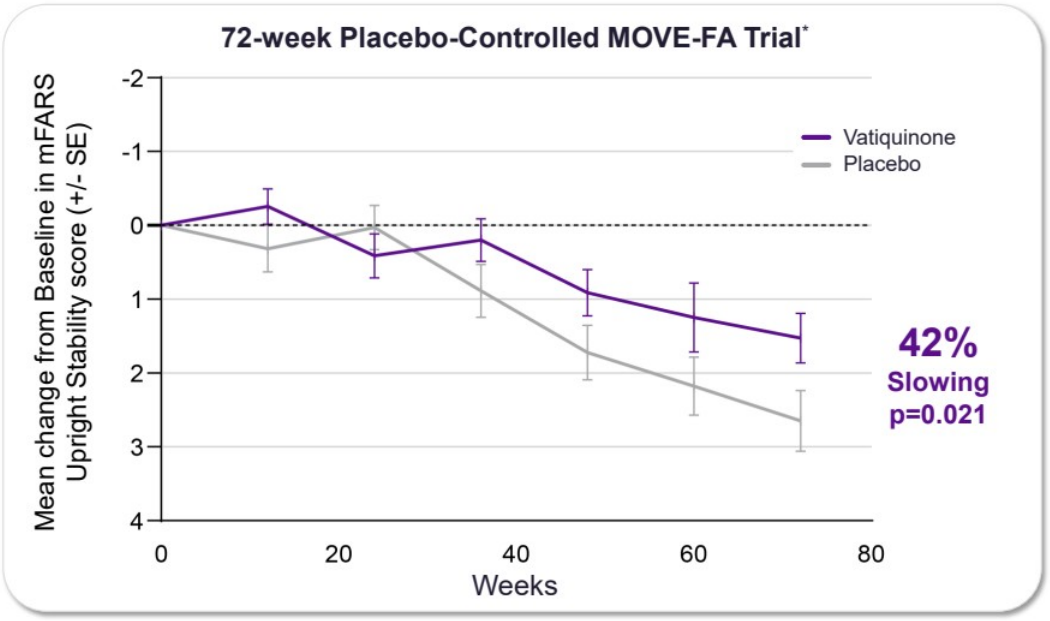


Olivia, living with FA

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Vatiquinone Demonstrated Meaningful Slowing of Short- and Long-term Disease Progression



Long-term Extension Stud

50% slowing (p<0.0001) disease progression over 3 years** in MOVE-FA long-term extension study

4.8-point benefit (p<0.000 on mFARS** over 2 years in ambulatory and non-ambulatory adults

* MOVE-FA did not meet its primary endpoint of statistically significant change in total mFARS at 72 weeks
** Relative to matched FACOMS (Friedreich's Ataxia Clinical Outcome Measures Study) natural history cohort

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PROVE-FA Phase 3 Open-Label Study to Support NDA Resubmission Initiated in Q3 2026

Study Overview

- Open-label study using matched natural history cohort from FACOMS disease registry
- Target enrollment: 120 individuals with FA ages 7 to 21
- Primary endpoint: Change in mFARS from baseline to 24 months



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Innovative Research Platforms



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R&D Pipeline Includes Innovative Programs from Differentiated Scientific Platforms



Oral Small Molecule Splicing Platform

- **MSH3:** Huntington's Disease, Myotonic Dystrophy I
- **Undisclosed:** Brain Tumors & Metastases
- **Undisclosed:** Sickle Cell Disease, β-Thalassemia
- **Undisclosed:** Alzheimer's Disease
- **Undisclosed:** Neurodegenerative Diseases

Inflammation/ Ferroptosis Platform



- **Ferroptosis:** Multisystem Atrophy (MSA) & Parkinson's Dise
- **NRF2 Activation:** CNS, Non-CNS Indications
- **NLRP3 Inflammasome:** Inflammatory Lung Diseases
- **DHODH Inhibition:** T-Cell Mediated Autoimmune Diseas

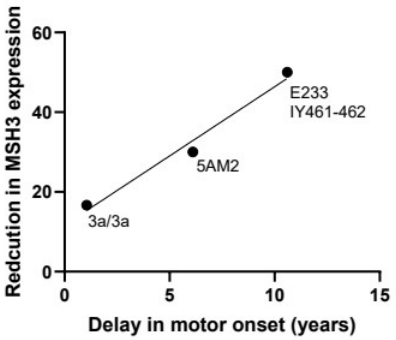
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PTC303 MSH3 Small Molecule Splicing Program

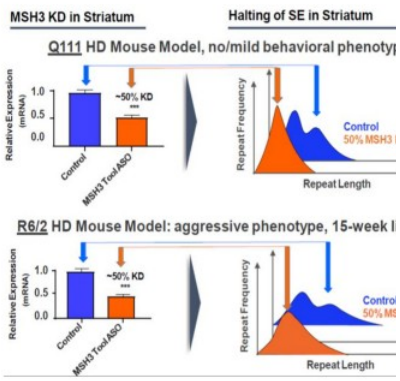
- MSH3 is a genetic modifier of repeat expansion disorders such as HD and DM1
- Validated correlation between MSH3 reduction and HD onset and progression, supporting therapeutic poter of MSH3 reduction
- PTC303 to enter clinic in 2027

Clear linear correlation between magnitude of MSH3 reduction and HD progression in patients[1]



Similar correlation for DM1

In HD mice, 50% knockdown of MSH3 halts somatic expansion in the striatum[2]



1. Flower. M et al. Brain. 2019 Jun 19;142(7):1876–1886. 2. Antonijevic, I. A. et al. (2022)

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PTC612 NLRP3 Inhibitor Program

- NLRP3 is an intracellular sensor protein that detects infection, stress or tissue damage
- Upon detection, NLRP3 is activated, triggering the assembly and activation of the NLRP3 inflammasome leading to elevated levels of IL-1β and IL-18
- Aberrant NLRP3 activation is implicated in a wide range of inflammatory diseases
- PTC612 is differentiated from other NLRP3 inhibitors in terms of potency, selectivity and chemical structure
- PTC612 Phase 1 SAD/MAD healthy volunteer study ongoing including cohort of individuals with metabolic syndrome to provide initial evidence of PK/PD effect

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PTC612: Greater Potency & Selectivity Compared t Other NLRP3 Inhibitors

PTC612 Demonstrates >10x Potency and Selectivity vs Other NLRP3 Inhibitors

Assay	PTC612	Selnoflast[1]	DFV890[2]	Usnoflast[3]	AZD4144[4]
THP-1 IL1β IC_{50} (nM)	**0.99**	220	13	13	23-82

Assay	PTC612	Vent-01[5]	DFV890[2]	VTX-3232[6]	VTX-2735[6]
hWB IL1β IC50 (nM)	**9.7**	124	180	13	80

1. In house data; 2. JMC 2025; 3. Brain Res 2024; 4. JMC 2025; 5. Inflammasome Summit 2022; 6. Ventyx Corporate Presentation June 2023

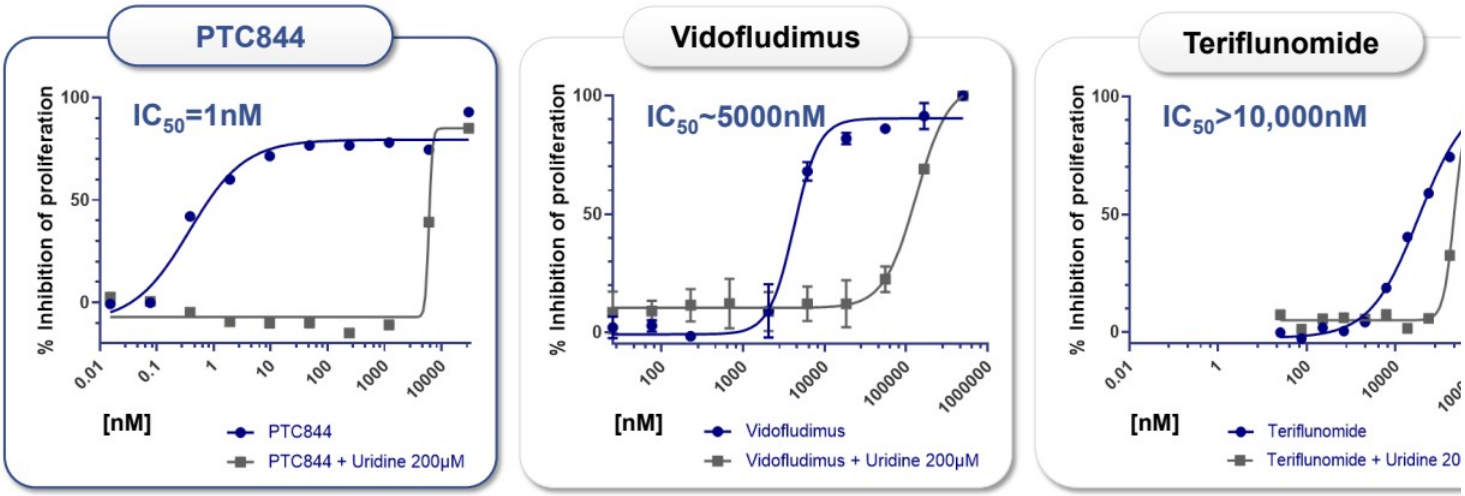
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PTC844 DHODH Inhibitor Program

- DHODH plays a critical role in inflammation by regulating the proliferation of immune cells and modulating cytokine production
- Marketed DHODH inhibitors have limitations due to lack of specificity and associated toxicity
- PTC844 is a more potent and selective DHODH inhibitor, potentially improving efficacy, safety and tolerability
- PTC844 Phase 2a study initiated in Q3 2026
  - 12-week PK/PD study to assess treatment effect on biomarkers and inform target indications

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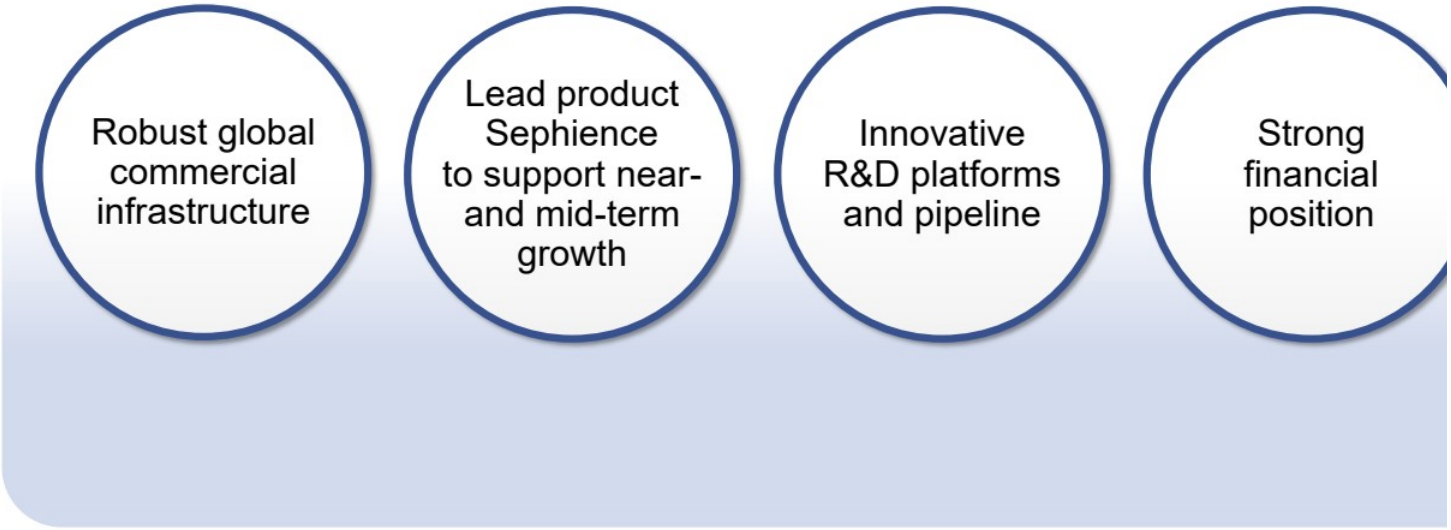
PTC844 Demonstrates Superior Potency and Selectivity Compared to Other DHODH Inhibitors



Phase 2a PK/PD study of PTC844 initiating Q3 2026

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Well Positioned for Success in 2026 & Beyond



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PTC Therapeutics

October 2026



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