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): **January 13, 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 January 13, 2025, PTC Therapeutics, Inc. (the "Company") issued a press release (the "press release") announcing certain preliminary (unaudited) financial information for its fiscal year ending December 31, 2024, including that the Company expects to report (i) total unaudited net revenue of approximately $814 million, (ii) total unaudited net product revenue of approximately $601 million, (iii) unaudited net product revenue for Translarna™ (ataluren) of approximately $340 million and unaudited net product revenue for Emflaza® (deflazacort) of approximately $207 million and (iv) royalty revenue associated with Evrysdi of approximately $211 million. Final results are subject to completion of the Company's year-end audit.

Item 7.01. Regulation FD Disclosure.

On January 13, 2025, the Company also announced financial guidance for its fiscal year ending December 31, 2025 in the press release, including that the Company anticipates (i) full year total revenues to be between $600 million and $800 million, including in-line products, potential new product launches and royalty revenue from Evrysdi, and (ii) GAAP R&D and SG&A expense for the full year 2025 to be between $805 million and $835 million with non-GAAP R&D and SG&A expense for the full year 2025 to be between $730 million and $760 million, excluding estimated non-cash, stock-based compensation expense of $75 million.

The Company announced that on Monday, January 13, 2025 at 2:15 p.m. EST at the 43rd Annual J.P. Morgan Healthcare Conference (the "Conference"), the Company will provide an update on 2024 accomplishments and highlight upcoming 2024 potential value-creating milestones. The Company will also present its preliminary 2024 unaudited financial results and 2025 financial guidance. The presentation will be webcast live on the Events and Presentations page under the Investors section of the Company's website. Following the completion of the Conference, the Company will enter an investor quiet period until it provides its next corporate update.

A copy of the press release and the slide presentation are attached to this Current Report on Form 8-K as Exhibits 99.1 and 99.2 and are incorporated by reference into this Item 7.01.

This Current Report on Form 8-K and Exhibits 99.1 and 99.2 include a forward-looking financial measure that was not prepared in accordance with accounting principles generally accepted in the United States (GAAP), non-GAAP R&D and SG&A expenses (which excludes non-cash stock-based compensation expense). Management uses this measure when assessing and identifying operational trends and, in management's opinion, this non-GAAP measure is useful to investors and other users of its financial statements by providing greater transparency into the historical and projected operating performance of the Company and the Company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP.

The information set forth in or incorporated by reference into Item 2.02 or this Item 7.01, including 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 Current Report on Form 8-K 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 Current Report on Form 8-K.

Forward Looking Statements: All statements, other than those of historical fact, contained in this Current Report on Form 8-K, are forward-looking statements, including reporting expectations with respect to financial information for fiscal year 2024 and financial guidance for fiscal year 2025. The Company'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 preliminary nature of the Company's 2024 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2025; and the factors discussed in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2023, as well as any updates to these risk factors filed from time to time in the Company's other filings with the Securities and Exchange Commission. You are urged to carefully consider all such factors. The forward-looking statements contained herein and the exhibits hereto represent the Company's views only as of the date of this Current Report on Form 8-K and the Company 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 Current Report on Form 8-K except as required by law.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated January 13, 2025 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – 43rd Annual J.P. Morgan Healthcare Conference
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: January 13, 2025

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

PTC Therapeutics Provides Update on Commercial Performance and R&D Pipeline at 43rd Annual J.P. Morgan Healthcare Conference

– Unaudited 2024 total revenue of approximately $814 million, exceeding guidance –

– Four approval applications submitted to FDA in 2024 –

– Global launch preparations underway for sepiapterin for PKU, a $1 billion market opportunity; CHMP opinion expected Q2 2025, U.S. approval decision in July 2025 –

– License and collaboration agreement with Novartis for PTC518 program closed –

– PIVOT-HD data readout for PTC518 expected Q2 2025 –

WARREN, N.J., Jan. 13, 2025 - PTC Therapeutics, Inc. (NASDAQ: PTCT) today provided an update on the Company's progress and its outlook for 2025. Matthew B. Klein, M.D., Chief Executive Officer of PTC, will discuss these updates at the 43rd Annual J.P. Morgan Healthcare Conference today at 11:15 a.m. PST / 2:15 p.m. EST. The presentation will be webcast live on the Events and Presentations page under the Investors section of PTC Therapeutics' website at https://ir.ptcbio.com/events-presentations and will be archived for 30 days following the presentation.

"I am very proud of our team's performance across every part of the company in 2024," Dr. Klein said. "We met every one of our planned clinical and regulatory milestones on schedule. Commercially, we outperformed guidance. And we begin 2025 with a strong cash balance, ready to build on our successes. PTC, now stronger and more innovative than ever, will continue to work to bring transformative therapies to the patient communities we serve in 2025 and beyond."

Key 2024 Corporate Highlights:

- Unaudited net product revenue of approximately $814 million, exceeding guidance.
  - Strong performance was driven by in-line products, including the DMD franchise with unaudited net product revenue of approximately $340 million for Translarna™ (ataluren) and approximately $207 million for Emflaza® (deflazacort) in 2024.

- Cash balance of approximately $1.1 billion as of December 31, 2024, with an additional $1.0 billion in upfront proceeds from PTC518 collaboration agreement with Novartis following closing.

- PTC submitted four regulatory approval applications to FDA:
  - Kebilidi™ (eladocagene exuparvovec-tneq) gene therapy for AADC deficiency, approved in November 2024.
  - Sepiapterin for children and adults with PKU, accepted with an FDA action date of July 29, 2025.
  - Vatiquinone for children and adults with FA, with filing acceptance decision anticipated Q1 2025.
  - Translarna for nmDMD, accepted with no action date provided.

- PTC signed a global license and collaboration agreement with Novartis for the research, development and commercialization of PTC518 for HD, which has now closed. Key aspects of the transaction include the following:
  - PTC to receive $1.0 billion in upfront proceeds following closing.
  - PTC is eligible to receive up to $1.9 billion in development, regulatory and sales milestones.

    - PTC to receive 40% profit share on U.S. sales, and double-digit tiered royalties on ex-U.S. sales.
    - Novartis will assume global development, manufacturing and commercial responsibilities following the completion of the placebo-controlled portion of PIVOT-HD, which is expected in 1H 2025.
- PTC received $150 million from the sale of the Rare Pediatric Disease PRV it received with FDA approval of Kebilidi.
- In December 2024, PTC had a Type C meeting with FDA to discuss the potential for HTT lowering to serve as a surrogate endpoint to support accelerated approval for PTC518 for HD. The Agency was aligned with the scientific rationale for HTT lowering as a potential surrogate endpoint and asked that PTC provide additional clinical data, such as those being collected in PIVOT-HD, to show associations between HTT lowering and changes in clinical outcome measures.

2025 Potential Key Clinical and Regulatory Events:

- CHMP opinion on sepiapterin MAA expected in Q2 2025.
- FDA approval decision on sepiapterin NDA expected July 29, 2025.
- Results from the PIVOT-HD Phase 2 study of PTC518 expected in Q2 2025.
- NDA acceptance of vatiquinone expected in Q1 2025, with potential regulatory approval in 2H 2025.

Unaudited 2024 Financial Results:

- Total unaudited net revenue for full-year 2024 was approximately $814 million.
- Total unaudited net product revenue for full-year 2024 was approximately $601 million.
- DMD franchise unaudited revenue for full-year 2024 was approximately $547 million, including unaudited net product revenue for Translarna of approximately $340 million and for Emflaza of approximately $207 million.
- PTC expects to report approximately $211 million of full-year 2024 royalty revenue associated with Evrysdi®.

  PTC is finalizing its financial results for the 2024 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2024, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on internal estimates.

2025 Financial Guidance:

- PTC anticipates total revenues for the full-year 2025 to be between $600 million and $800 million, including in-line products, potential new product launches and royalty revenue from Evrysdi.
- PTC anticipates GAAP R&D and SG&A expense for the full-year 2025 to be between $805 million and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full-year 2025 to be between $730 million 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 and financial guidance of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP 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.

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:

AADC: Aromatic l-Amino Acid Decarboxylase
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
FA: Friedreich's ataxia
FDA: Food and Drug Administration
HD: Huntington's Disease
MAA: Marketing Authorization Application
NDA: New Drug Application
nmDMD: nonsense mutation Duchenne Muscular Dystrophy
PKU: Phenylketonuria
PRV: Priority Review Voucher
R&D: Research and Development
SG&A: Selling, General and Administrative

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company that discovers, develops and commercializes clinically differentiated medicines that provide benefits to children and adults living with rare disorders. PTC's ability to innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Facebook, Instagram, LinkedIn and X.

For More Information:

Investors:
Ellen Cavaleri
+1 (615) 618-6228
ecavaleri@ptcbio.com

Media:
Jeanine Clemente
+1 (908) 912-9406
jclemente@ptcbio.com

Forward-Looking Statement
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 "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," 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, the European Economic Area (EEA) and other regions, including whether the European Commission adopts the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) for the conditional marketing authorization for Translarna in the EEA, or PTC's ability to identify other potential mechanisms by which it may provide Translarna to nmDMD patients in the EEA; PTC's ability to use the clinical data from its international drug registry study and real-world evidence concerning Translarna's benefits to support a continued marketing authorization for Translarna for the treatment of nmDMD in the EEA; 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 any upfront payment, development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; expectations with respect to Kebilidi and Upstaza, 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 sepiapterin, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of development, 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 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 Translarna, Emflaza, Kebilidi, Upstaza, Evrysdi, Tegsedi, Waylivra, sepiapterin or vatiqunone.

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 2025

Matthew B. Klein, MD
CEO

Pam and Kelsey living with PKU


The FUTURE Is NOW

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, other than stateme
historic fact, are forward-looking statements, including statements with respect to 2025 total revenue guidance and 2025 operating expenditure guidance and statements regarding: the future expect
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 matte
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-l
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of PTC's most recent 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

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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-looking state
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 required by law.

Execution in 2024 Provides Foundation for Success

All Development Milestones Achieved

Outstanding Revenue Performance

Strong Cash Position

Advanced Programs from Innovative Scientific Platforms

Four U.S. Regulatory Approval Applications Submitted in 2024

AADC Gene Therapy BLA	*Approved*
Sepiapterin PKU NDA	*Accepted*
Translarna nmDMD NDA	*Accepted*
Vatiquinone Friedreich's Ataxia NDA	*Submitted*

Outstanding 2024 Revenue Performance Driven by Inline Products

translarna ataluren | Emflaza (deflazacort) | Upstaza (eladocagene exuparvovec) | Tegsedi (inotersen) | waylivra (volanesorsen) | Evrysdi risdiplam

2024

Unaudited Total Revenue

~$814M

Strong Cash Position Enables Future Revenue Growth and R&D Innovation

Reach Cashflow Breakeven Without Additional Capital

Support Commercial Launches and Innovative R&D Programs

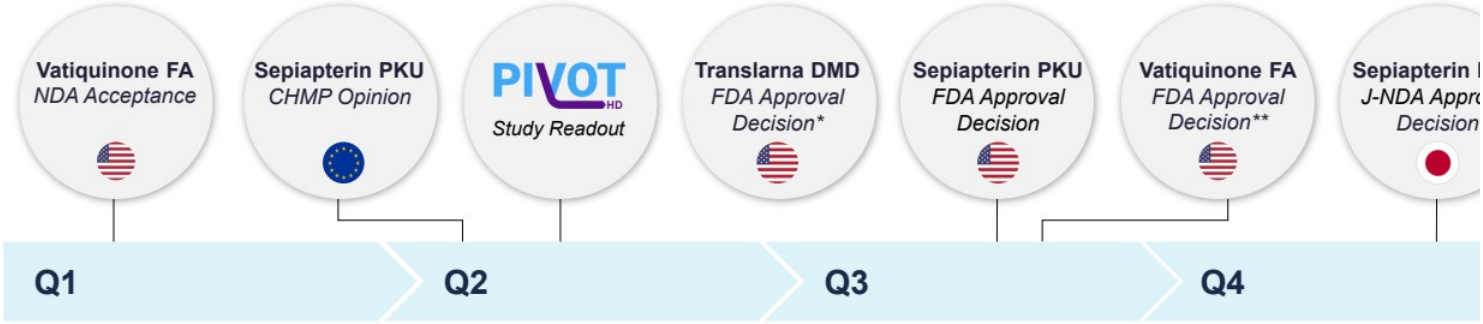
Fund BD Activities to Complement Product Portfolio

Research Platforms Provide Continuous Source of Innovative Therapies

Splicing

Inflammation & Ferroptosis

Numerous Potential Value-Creating Milestones Expected in 2025



*No action date provided
**If priority review granted

2025 Revenue and OPEX Guidance

translarna ataluren

Emflaza (deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

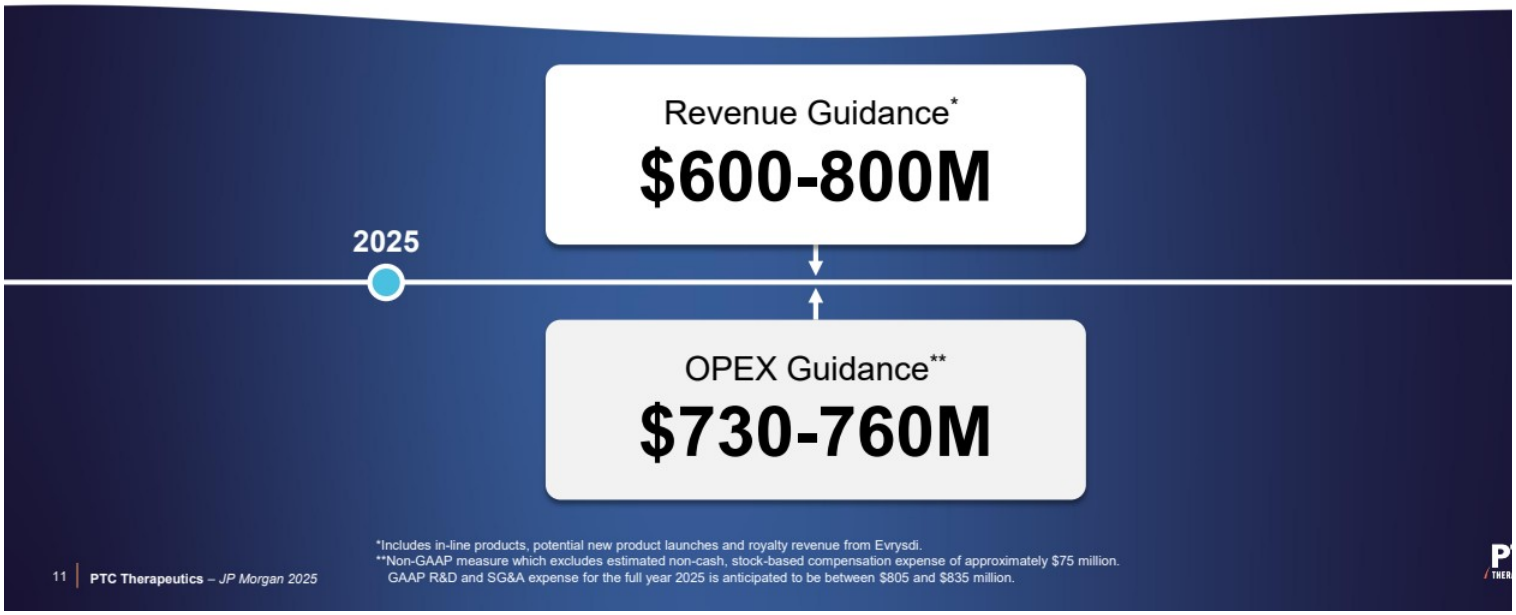
Kebilidi (eladocagene exuparvovec-tneq)

Upstaza (eladocagene exuparvovec)

Tegsedi (inotersen) injection 284 mg/1.5 mL

waylivra (volanesorsen) injection 285 mg/1.5 mL

Evrysdi risdiplam



*Includes in-line products, potential new product launches and royalty revenue from Evrysdi.
**Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $75 million. GAAP R&D and SG&A expense for the full year 2025 is anticipated to be between $805 and $835 million.

Sepiapterin PKU Program

Owen living with PKU

Sepiapterin Demonstrates Potential to Meet Unmet Need for Majority of PKU Patients

apher

Dual mechanism of action provides stronger cofactor effect and enables efficacy in non-BH4 responsive mutations

Significant and meaningful Phe lowering with 84% of patients reaching target Phe levels (<360µmol/L)

Efficacy across all key subgroups in Phase 3 APHENITY trial including patients with classical PKU

Evidence of diet liberalization with ~50% of patients exceeding Recommended Daily Allowance for protein

Physician and Patient Statements Reflect Enthusiasm for Sepiapterin

"I'm excited about the opportunity to offer sepiapterin to all my patients"

– Ania C. Muntau, MD, Professor of Pediatrics, Chair, University Children's Hospital, University Medical Center, Hamburg Eppendorf, Germa

"Impressively, several participants with classical PKU…who had not shown response to sapropterin…showed clinically meaningful response to sepiapte

– Cary O. Harding, MD, *The Lancet*, Volume 404, Issue 10460, 1284 - 1286

Anecdotes on social media from patients about being able to liberalize their diets and "enjoy meat, fish, and chicken" while maintaining Phe control

APHENITY Results Support Potential to Address All Key PKU Population Segments

Patients Who Have Failed Current Therapies

Patients Who Are Not Well Controlled by Current Therapies

Therapy-Naive Patients Including Classical PKU

Greater than $1 Billion Potential Revenue Opportunity

Experienced U.S. Team Preparing for Planned 2025 Launch

- Centers of Excellence & prescribers mapped
- Payer & access discussions initiated
- HCPs engaged through multifaceted approach
- Patient & community partnerships established

Vatiquinone FA Program

Olivia living with FA

Vatiquinone Has Potential to be First Approved Treatment for Children with Friedreich's Ataxia

Vatiquinone data support safety and efficacy for **children and adults** with FA

NDA filing acceptance expected in February 2025 with approval decision potentially by August 202

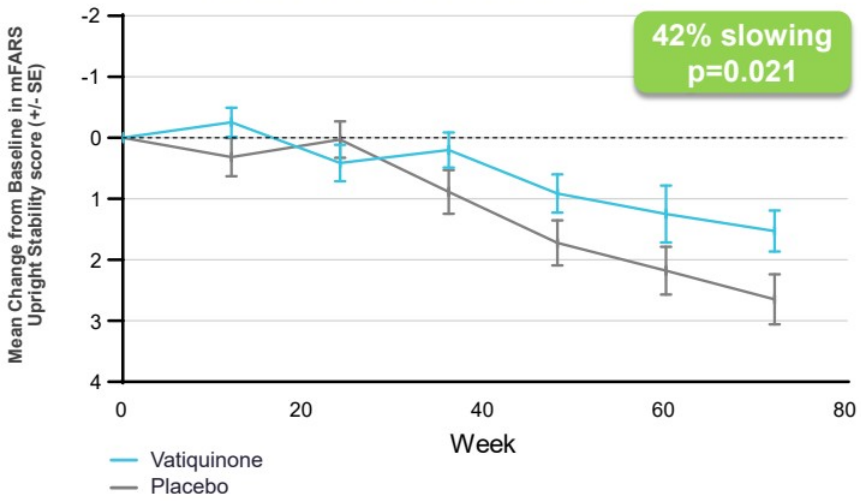
NDA based on findings of efficacy from MOVE-FA placebo-controlled study and two long-term studies

Additional marketing authorization submissions planned in 2025

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

MOVE-FA

72-week Placebo-Controlled Trial*



Long-term Extension Studies

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

4.8-point benefit (p<0.0001) 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

Vatiquinone Profile Supports Significant Commerci Opportunity Including All Friedreich's Ataxia Patien

~6,000 U.S. patients, ~1/3 are pediatric; global prevalence of ~25,000

Efficacy and safety data support opportunity for patients of all ages

Launch preparations underway including CoE and physician mapping

Strong 10+ year partnership with FA patient advocacy groups

PTC518
HD Program

Key Attributes of PTC518 Drive Differentiation

- Orally bioavailable
- Achieves excellent and broad CNS exposure
- Highly selective and specific for HTT target
- Uniform mHTT lowering in all regions of the brain
- Reduces HTT protein in the CNS & periphery
- Safe and well tolerated in clinical trials

Month 12 Interim Readout Met All Key Safety and Efficacy Objectives (June 2024)

Dose-dependent and durable lowering of mHTT protein in blood

Dose-dependent lowering of CSF mHTT protein levels

Dose-dependent trends of benefit on key clinical scales (TMS, cUHDRS)

PTC518 was well tolerated with no treatment-related NfL spikes

Planned Data Update in Q2 2025 to Include 12-month Results for Additional ~100 Subjects

Safety and tolerability of PTC518	Percent reduction in blood mHTT protein	Percent reduction in CSF mHTT protein	Changes in clinical scores (cUHDRS, TMS, TFC)	Readout will include both Stage 2 and 3 subjects

Results to Support Regulatory Discussions on Accelerated Approval Potential

Development and Commercialization Collaboration

$1 billion upfront payment

Up to **$1.9 billion** in development, regulatory and sales milestones

40% U.S. profit share, **double-digit** tiered royalties on ex-U.S. sales

Novartis to fund development activities following completion of PIVOT-HD

Expected to close in Q1 2025, subject to customary closing conditions, including regulatory clearance.
Refer to press release dated December 2, 2024 for additional details.

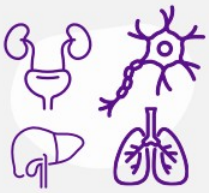
Validated Splicing Platform Provides Source of Innovative and Valuable Therapies

PTC has pioneered discovery and development of oral splicing therapies

PTSeek™

Platform innovations such as PTSeek™ accelerate discovery of novel splicing therapies

Multiple active CNS programs advancing towards clinic



Additional early-stage programs targeting non-CNS indications

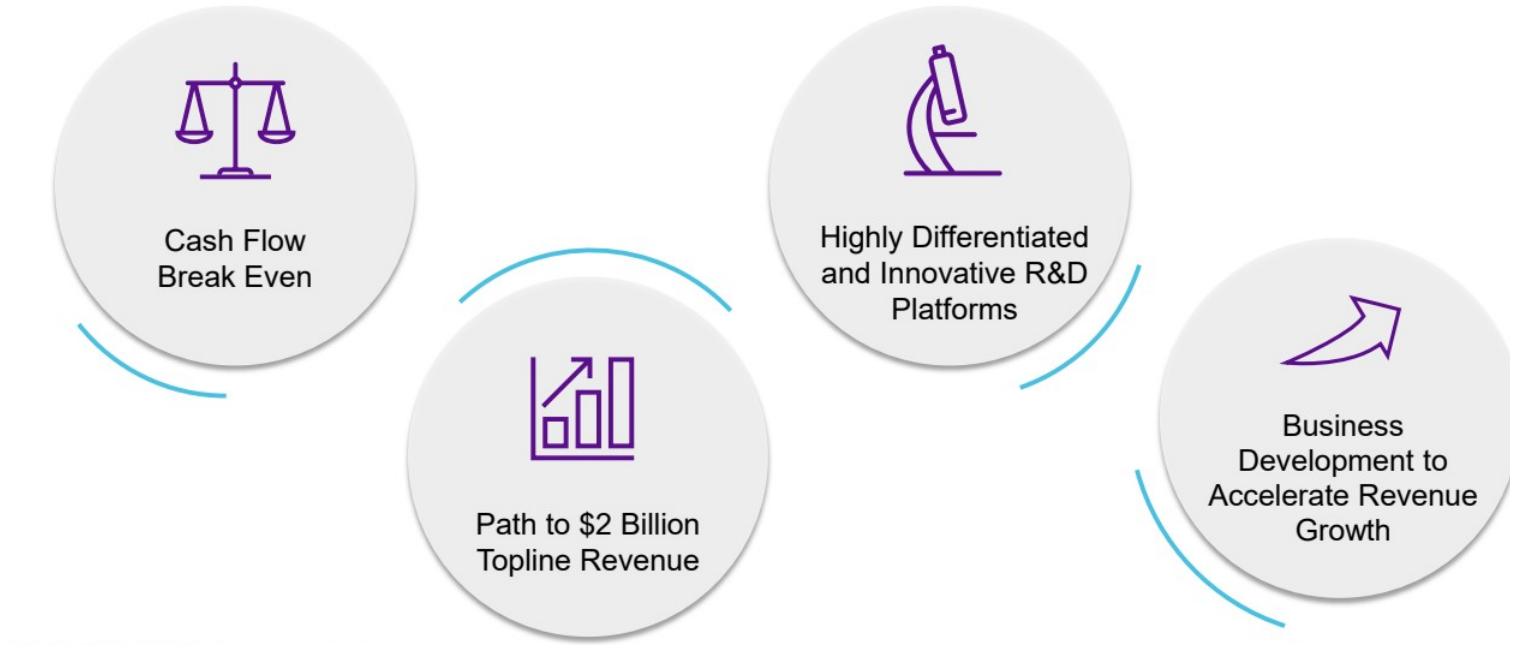
Inflammation & Ferroptosis Programs Targeting CNS and Non-CNS Disorders

Focused on novel targets key to inflammation and oxidative stress

Active programs targeting CNS and non-CNS disorders

- Phase 2 ready DHODH inhibitor program for neuroinflammation indications
- NLRP3 inhibitor program entering IND-enabling studies
- Preclinical program targeting alpha synuclein for Parkinson's disease
- Preclinical program targeting nrf2 activation for both CNS and peripheral indications

PTC Vision for Successful 2025 and Beyond




The FUTURE Is NOW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