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 8, 2024**

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.)

100 Corporate Court **South Plainfield, NJ**	**07080**
(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 8, 2024, 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, 2023, including that the Company expects to report (i) total unaudited net revenue of approximately $946 million, (ii) total unaudited net product revenue of approximately $661 million, (iii) unaudited net product revenue for Translarna™ (ataluren) of approximately $355 million and unaudited net product revenue for Emflaza® (deflazacort) of approximately $255 million and (iv) collaboration and royalty revenue associated with Evrysdi of approximately $278 million. Final results are subject to completion of the Company's year-end audit.

Item 7.01. Regulation FD Disclsoure.

On January 8, 2024, the Company also announced financial guidance for its fiscal year ending December 31, 2024 in the press release, including that the Company anticipates (i) full year total revenues to be between $600 million and $850 million, (ii) GAAP R&D and SG&A expense for the full year 2024 to be between $740 million and $835 million with non-GAAP R&D and SG&A expense for the full year 2024 to be between $660 million and $755 million, including R&D expense milestone payments of up to $65 million and excluding estimated non-cash, stock-based compensation expense of $80 million and (iii) up to $90 million of payments for the full year 2024 upon achievement of potential regulatory success-based milestones from previous acquisition, of which up to $65 million will be recorded as R&D operating expense.

The Company announced that on Monday, January 8, 2024 at 10:30 am EST at the 42nd Annual J.P. Morgan Healthcare Conference (the "Conference"), the Company will provide an update on 2023 accomplishments and highlight upcoming 2024 potential value-creating milestones. The Company will also present its preliminary 2023 unaudited financial results and 2024 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 is attached to this Current Report on Form 8-K as Exhibit 99.1 and is 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 2023 and financial guidance for fiscal year 2024. 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 2023 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2024; 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, 2022 and most recent Quarterly Report on Form 10-Q 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 8, 2024 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – 42nd 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 8, 2024

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

PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 42nd Annual J.P. Morgan Healthcare Conference

– Unaudited 2023 total revenue of $946 million, representing 35% year-over-year growth –

– Regulatory filings in the EU and US for sepiapterin in PKU, a potential $1 billion global commercial opportunity, remain on track for 2024 –

– Multiple study readouts planned for 2024, including 12-month interim data from the PIVOT-HD study of PTC518 in HD patients –

SOUTH PLAINFIELD, N.J., Jan. 8, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 42nd Annual J.P. Morgan Healthcare Conference today, Monday, January 8, at 10:30am EST/7:30am PST. Matthew Klein, M.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2023 accomplishments and highlight potential 2024 value-creating milestones. Preliminary 2023 unaudited financial results and 2024 financial guidance will also be provided. The presentation is being webcast live on the Events and Presentations page of the Investors section of PTC Therapeutics website at www.ptcbio.com.

"2023 was a transformational year for PTC as we made necessary and important changes to become a leaner, more focused and financially stronger company positioned for future success," said Matthew B. Klein, M.D., Chief Executive Officer of PTC. "We look forward to a successful 2024, with anticipated key global regulatory submissions for our PKU program and multiple data readouts, including from our PTC518 HD program."

Key 2023 Corporate Highlights:

- Unaudited net product revenue of $661 million in 2023 representing 23% year-over-year growth.
    - Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $355 million for Translarna™ (ataluren) and $255 million for Emflaza® (deflazacort) in 2023.
        - Translarna growth was driven by new patients in existing geographies and continued geographic expansion.
        - Emflaza growth was due to continued new prescriptions, high compliance, and more favorable access.
- PTC announced positive readouts from key programs in its clinical pipeline in 2023:
    - Highly statistically significant and clinically meaningful results in the Phase 3 registration-directed APHENITY clinical trial of sepiapterin in adult and pediatric patients with PKU.
    - Achievement of all key objectives of the readout of 12-week interim data in the PIVOT-HD study of PTC518 in HD.
- PTC announced strategic portfolio prioritizations, which resulted in reductions in both operating expenses and headcount of approximately 25% and 30%, respectively.
- In October, PTC finalized a royalty agreement, in which Royalty Pharma acquired additional royalties of Evrysdi for $1.0 billion upfront. The agreement included options for PTC to sell the remainder of its royalties of Evrysdi for up to $500 million or for Royalty Pharma to acquire half of such retained royalties for up to $250 million at a later date, less royalties received by PTC. PTC maintains all economics associated with up to $250 million in the remaining commercial sales milestones associated with Evrysdi global net sales. The

proceeds from the financing were used to retire all outstanding debt obligations with Blackstone Life Sciences.

2024 Potential Key Clinical and Regulatory Events:

- Submission of an MAA to the EMA for sepiapterin for the treatment of PKU is expected in the first quarter.
- Submission of an NDA to the FDA for sepiapterin for the treatment of PKU is expected no later than the third quarter.
- Results from 12-month interim data from the PIVOT-HD trial of PTC518 in HD patients are expected in the second quarter.
- Submission of a BLA to the FDA for Upstaza for the treatment of AADC deficiency is expected in the first quarter.
- Topline results from the CardinALS trial of utreloxastat in ALS are expected in the fourth quarter.
- PTC expects the CHMP opinion from the re-examination procedure of the negative opinion on the Translarna conditional marketing authorization renewal in late January 2024, with ratification of that opinion by the European Commission 67 days later.
- FDA meeting for Translarna to align on the specific contents of a potential NDA resubmission is scheduled for the first quarter.
- FDA meeting for vatiquinone to discuss how the MOVE-FA data along with additional clinical and preclinical data could support an NDA submission in FA is scheduled for the first quarter.
- Scientific advice feedback from the EMA on a potential submission of vatiquinone for conditional marketing authorization for Friedreich ataxia is expected in the first quarter.

Unaudited 2023 Financial Results:

- Total unaudited net revenue for full year 2023 was approximately $946 million.
- Total unaudited net product revenue for full year 2023 was approximately $661 million.
- DMD franchise unaudited revenue for full year 2023 was approximately $610 million, including unaudited net product revenue for Translarna of approximately $355 million and for Emflaza of approximately $255 million.
- PTC expects to report approximately $278 million for full year 2023 collaboration and royalty revenue associated with Evrysdi.

PTC is currently in the process of finalizing its financial results for the 2023 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2023, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on internal estimates.

2024 Financial Guidance:

- PTC anticipates total revenues for the full year 2024 to be between $600 million and $850 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2024 to be between $740 and $835 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2024 to be between $660 and $755 million, including expected R&D expense milestone payments of up to $65 million and excluding estimated non-cash, stock-based compensation expense of $80 million.
- PTC anticipates up to $90 million of payments for the full year 2024 upon achievement of potential regulatory success-based milestones from previous acquisitions, of which up to $65 million will be recorded as R&D operating expense.

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 2024 R&D and SG&A Expense
(in millions)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 740	$ 835
Less: Projected Non-Cash, Stock-Based Compensation Expense	80	80
Projected non-GAAP R&D and SG&A Expense	**$ 660**	**$ 755**

PTC Therapeutics, Inc.
Reconciliation of GAAP Projected Full Year 2024 Milestone Payments
(in millions)

Projected GAAP R&D Expense Related Milestone Payments	$ 65
Projected GAAP Contingent Consideration Payable Related Milestone Payments	25
Total Projected GAAP Milestone Payments	**$ 90**

Acronyms:
AADC: Aromatic l-Amino Acid Decarboxylase
ALS: Amyotrophic Lateral Sclerosis
BLA: Biologics License Application
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EMA: European Medicines Agency
FDA: Food and Drug Administration
MAA: Marketing Authorization Application
NDA: New Drug Application
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients 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. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and LinkedIn.

For More Information:

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Jeanine Clemente
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jclemente@ptcbio.com

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 "2024 Financial Guidance", including with respect to (i) 2024 total revenue guidance, (ii) 2024 GAAP and non-GAAP R&D and SG&A expense guidance and (iii) 2024 acquisition related milestone payment 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; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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 Medicines Agency (EMA) determines in the re-examination process that the benefit-risk balance of Translarna authorization supports renewal of such authorization; 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, which was a specific obligation to continued marketing authorization in the EEA, to support a renewal of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA; PTC's ability to utilize results from Study 041 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 our trials in Translarna; expectations with respect to Upstaza, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities, the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and early research programs in gene therapy and reductions in workforce, which may be in different periods and may be materially higher than estimated; the savings that may result from the discontinuation of PTC's strategic pipeline prioritization and reductions in workforce, which may be materially less than expected; 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; the potential financial impact and benefits of PTC's leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of its lease agreements, including for its leased biologics manufacturing facility; 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 Translarna, Emflaza, Upstaza, 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 2024

Matthew B. Klein, MD
CEO

Patient Living with PKU

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 statements of historic fact, are forward-looking statements, including statements with respect to 2024 total revenue guidance, 2024 operating expenditure guidance and 2024 acquisition related milestone payment 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, projec costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; and the objectives of management. Other forward-looking statements may be identified words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

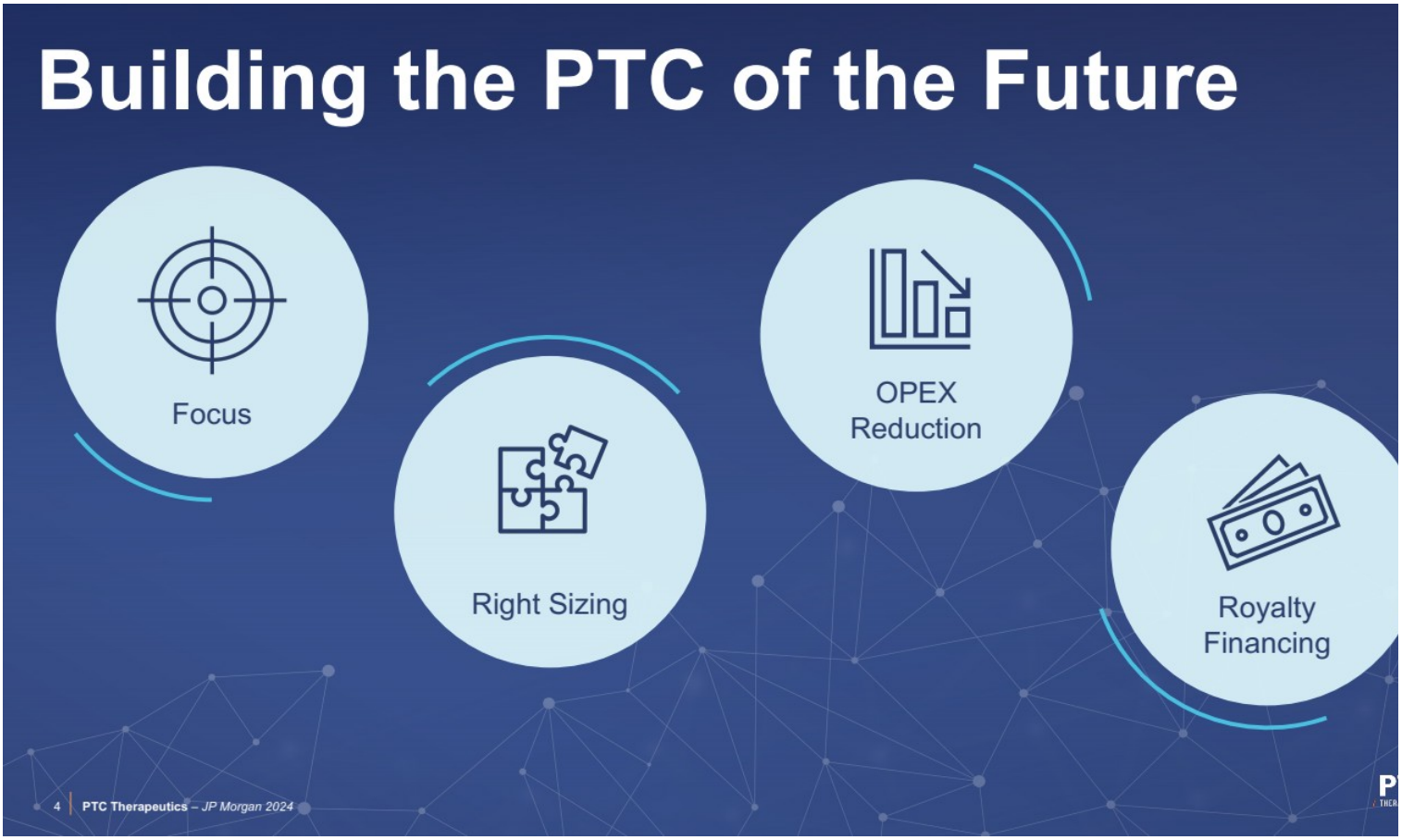
PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, inclu those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in t 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 Medicines Agency (EMA) determines in the re-examination process that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to use the re 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, which was a specific obligation to conti marketing authorization in the EEA, to support a renewal of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA; PTC's ability to utilize results from Study 041 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 clinic data from our trials in Translarna; expectations with respect to Upstaza, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities, the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under our SM collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and ear research programs in gene therapy and reductions in workforce, which may be in different periods and may be materially higher than estimated; the savings that may result from the discontinuation of strategic pipeline prioritization and reductions in workforce, which may be materially less than expected; significant business effects, including the effects of industry, market, economic, political or regul 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 ge development progress; the potential financial impact and benefits of PTC's leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of its lease agreements, includi its leased biologics manufacturing facility; 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 ris 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 wi receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, Upstaza, Evrysdi, Tegsedi or Waylivra.

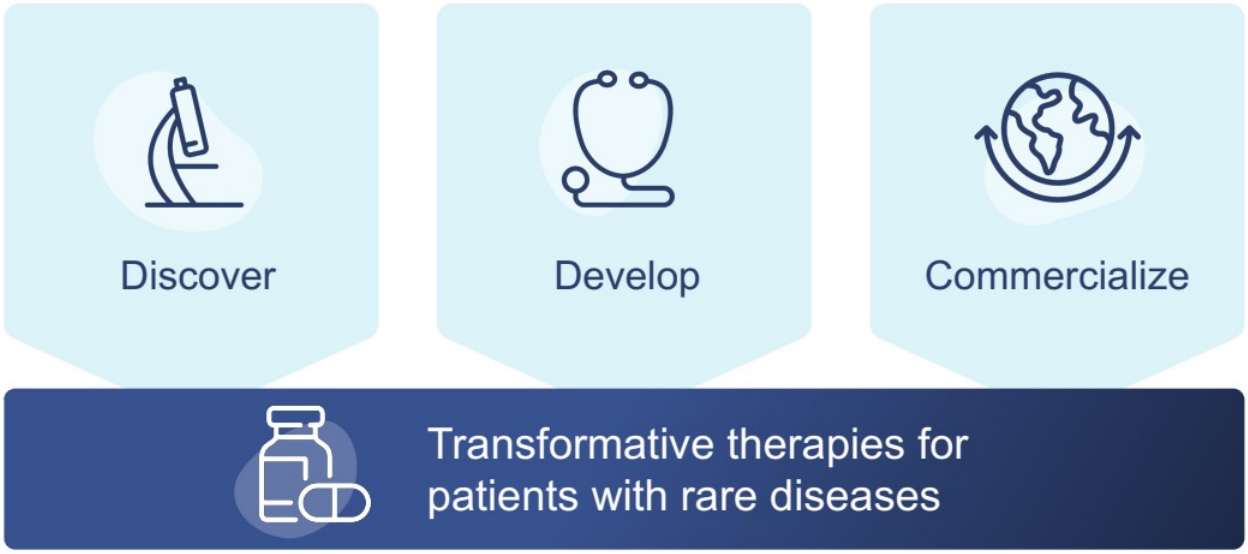
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking state to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other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

2023

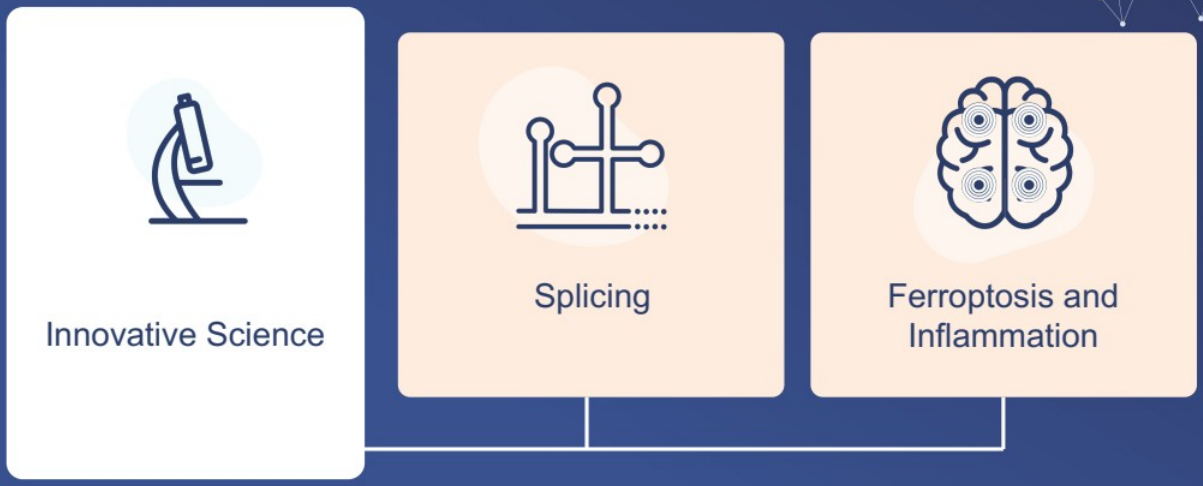
Building the PTC of the Future



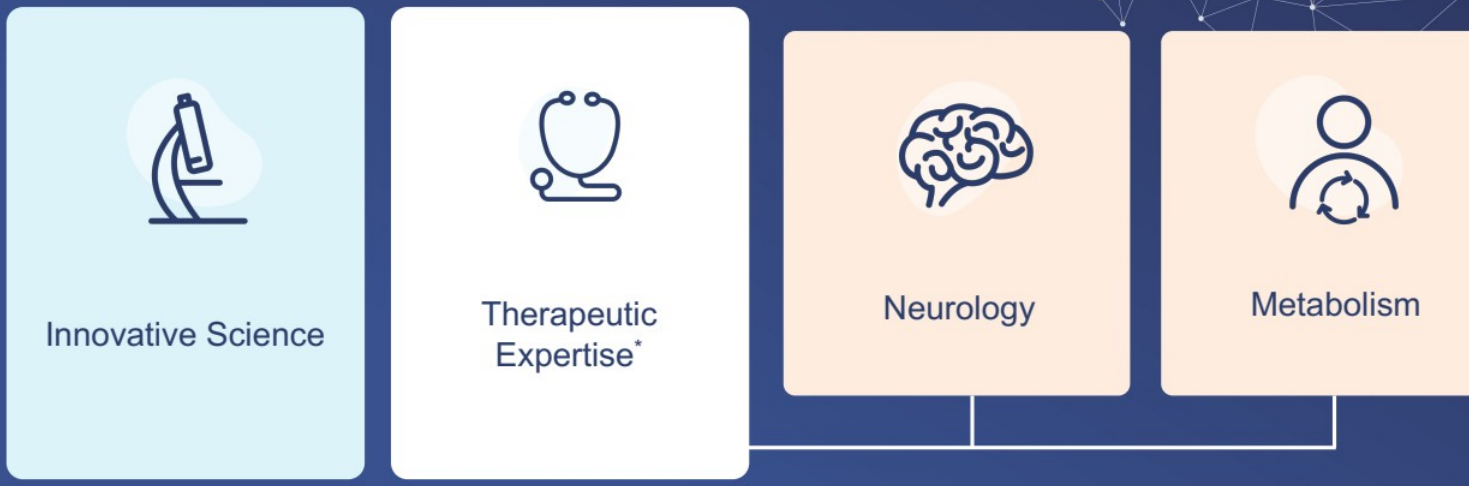
PTC Mission Remains Patient Focused



PTC Strategy Leverages Innovative Science, Passionate Team and Strong Cash Position



PTC Strategy Leverages Innovative Science, Passionate Team and Strong Cash Position



*Unesbulin (PTC596) development discontinued

PTC Strategy Leverages Innovative Science, Passionate Team and Strong Cash Position

Innovative Science

Therapeutic Expertise

Global Commercial Infrastructure

- Europe
- United States
- Asia Pacific
- Latin America
- Middle East & North Africa

Robust Portfolio to Support Growth & Value Creatio

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

Development

Sepiapterin (PKU)	Phase 1	Phase 2	Phase 2/3	Phase 3	**NDA Stage**
Vatiquinone (FA)	Phase 1	Phase 2	Phase 2/3	**Phase 3**	
Utreloxastat (ALS)	Phase 1	Phase 2	**Phase 2/3**		
PTC518 (HD)	Phase 1	**Phase 2**			
Undisclosed (Myopathies)	Phase 1				

Research

Splicing Platform

SCA-3

MAP-tau

Undisclosed
(Movement Disorders)

Ferroptosis and Inflammation Platform

Undisclosed
(Neurodegenerative Diseases)

Undisclosed
(Pediatric Neurodevelopment Disorders)

Strong Commercial Performance and Growth in 202

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

2023

Unaudited Total Revenue
~$946M

Unaudited DMD Franchise
Net Product Revenue
~$610M

Strong R&D Execution and Value Creation in 2023

Clinical

aphenity

Highly statistically significant and meaningful APHENITY results

PIVOT HD

Positive interim data for PIVOT-HD

MOVE-FA

Positive upright stability and fatigue scale results for MOVE-FA*

Regulatory

translarna™ ataluren

Additional approvals enabling continued geographic expansion

Tegsedi (inotersen) injection

Additional LATAM approvals enabling geographic expansion

waylivra® (volanesorsen) injection 285 mg/1.5 mL

Additional LATAM approvals enabling geographic expansion

*Primary endpoint did not meet statistical significance

2024 Revenue and OPEX Guidance

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

2024

Total Revenue Guidance

$600-$850M

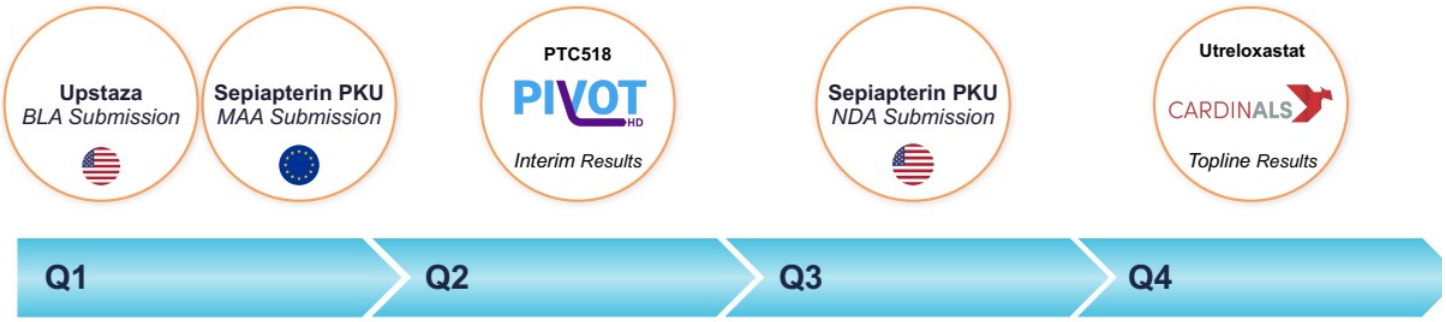
OPEX Guidance*

$660-755M

includes regulatory milestones up to $65M

*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $80 million. GAAP R&D and SG&A expense for the full year 2024 is anticipated to be between $740 and $835 million. The Company anticipates up to $65 million of payments upon achievement of potential regulatory success-based milestones from previous acquisitions.

Key Expected Regulatory & Clinical Milestones in 202



Sepiapterin PKU Program

Patient Living with PKU

APHENITY Results Demonstrate Meaningful Benefit of Sepiapterin in PKU Patients

The primary endpoint was reached in the placebo-controlled portion of the study with statistically significant reductions in blood Phe levels ($p < 0.0001$)

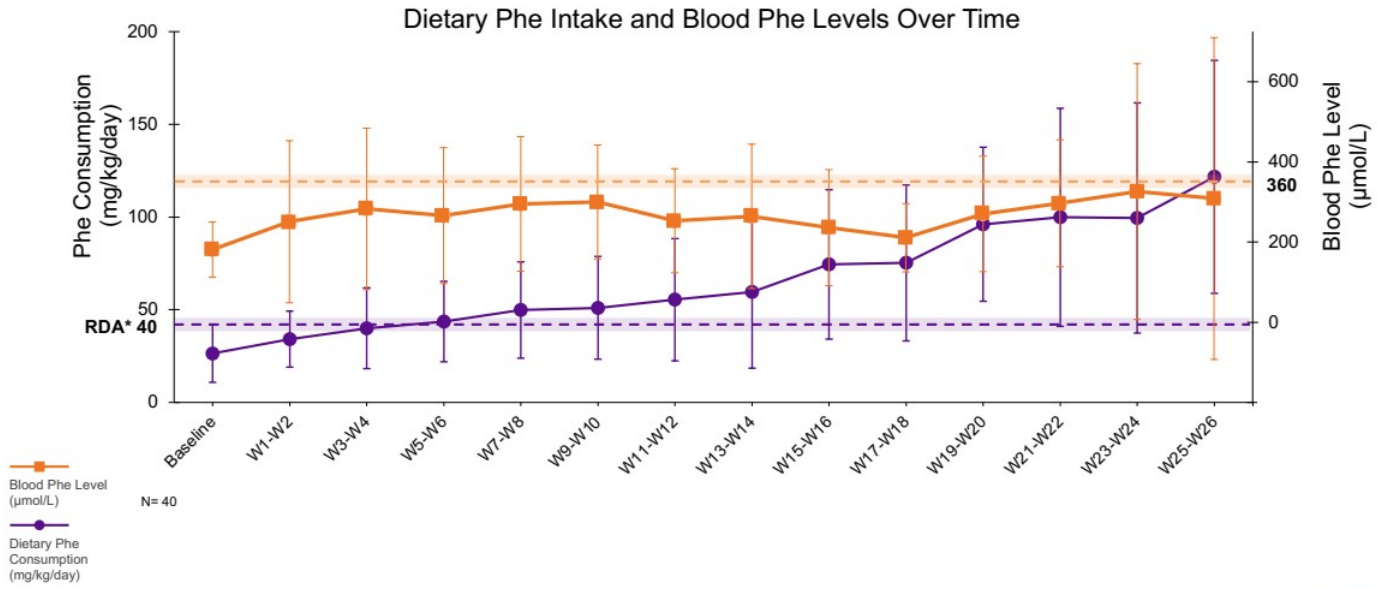
A substantial reduction in blood Phe levels from baseline was observed in both the primary analysis population (63%) and the subset of participants with classical PKU (69%)

94% of patients ≥ 12 years with blood Phe ≥ 600 µmol/L at baseline achieved a reduction in blood Phe to reach guideline target

Sepiapterin was well tolerated with no serious adverse events

Phe, phenylalanine; PKU, phenylketonuria.

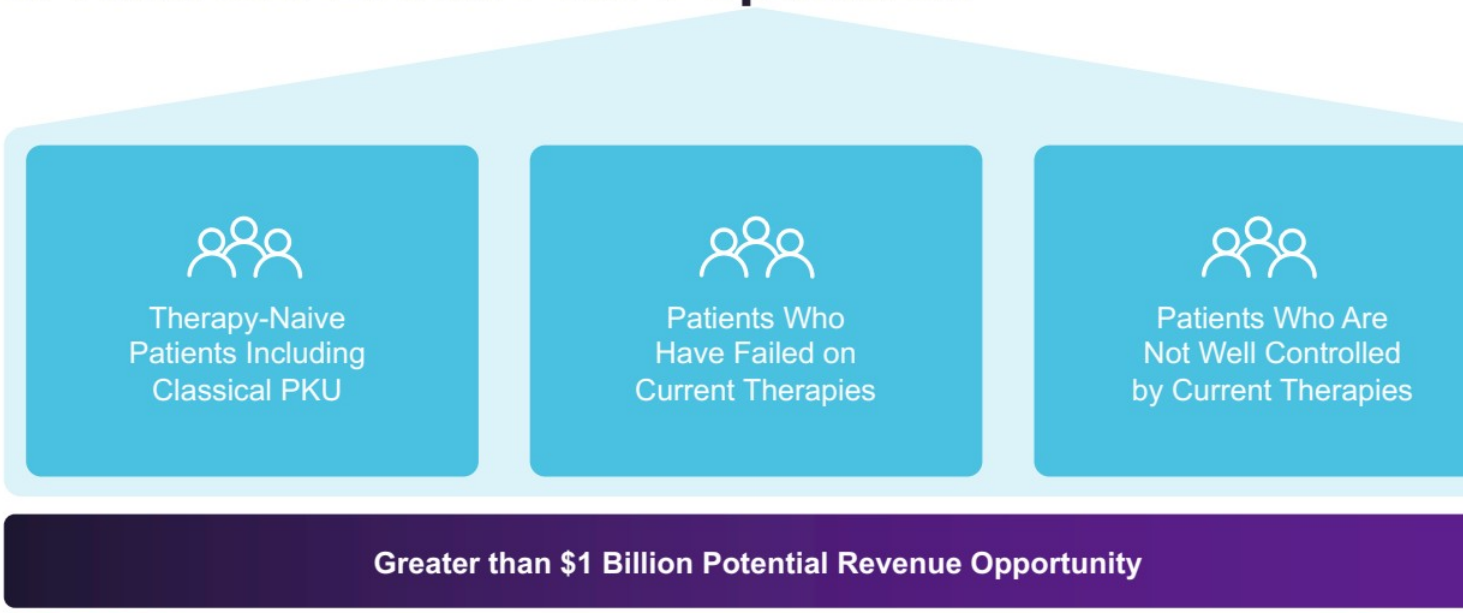
Sepiapterin Enables Increased Dietary Phe Intake in PKU Patients



*Recommended dietary allowance

Results based on interim data cut and not full data set

APHENITY Results Support Potential for Sepiapteri
to Address Broad PKU Population



Global Regulatory Submissions and Launch Preparation Planned for 2024

Global Regulatory Submissions

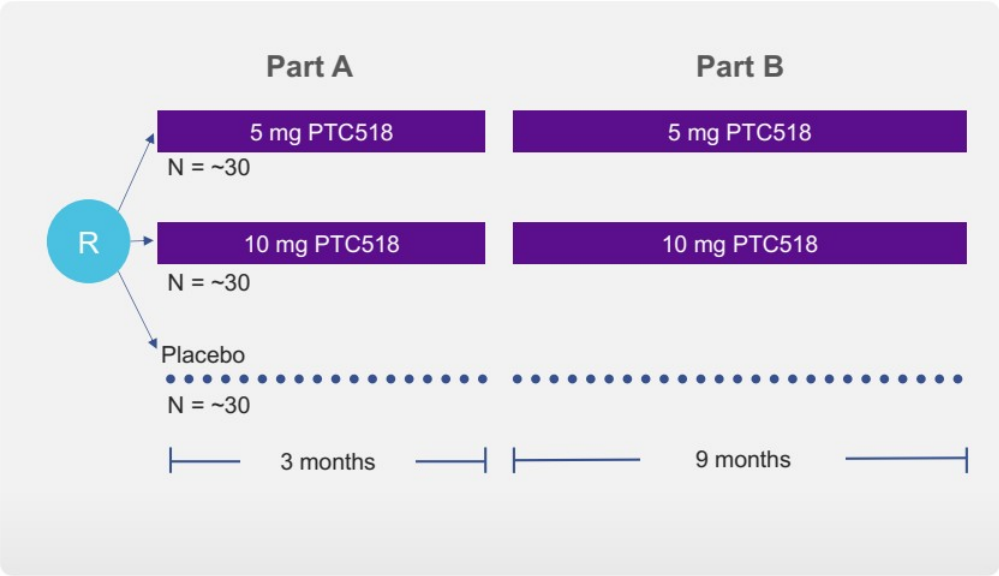
Global Launch Sequence and Continued Launch Preparation


PTC518
HD Program
PIVOT
HD

Key Attributes of PTC518 Drive Differentiation

- Orally bioavailable
- Titratable and reversible
- Highly selective and specific
- Reduces HTT mRNA and protein in the CNS and periphery
- Not effluxed, penetrates blood brain barrier
- Uniform lowering in key regions of the brain

PIVOT-HD Study Design



Primary Endpoints

- Safety and tolerability of PTC518
- Percent reduction in HTT mRNA and protein in blood

Secondary Endpoints

- Percent reduction in mHTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in brain volume on volumetric MRI imaging

12-Week Interim Data Met Key Objectives

PTC518 treatment resulted in dose-dependent lowering of HTT mRNA and protein levels in blood cells

PTC518 demonstrated desired CSF exposure with higher concentrations of free drug in the CSF than plasma

PTC518 was well tolerated with no treatment-related serious adverse events and no reports of peripheral neuropathy

CSF NfL levels remained stable after 12 weeks of treatment with no treatment-related spikes

12 Month Data Update to Include Blood, CSF and Radiographic Biomarkers

PIVO

- Safety and tolerability of PTC518
- Percent reduction in HTT mRNA and protein in blood
- Percent reduction in mHTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in brain volume on volumetric MRI imaging

Interim Results Expected Q2 2024

Vatiquinone FA Program

Patient Living with FA

Meaningful Clinical Results in MOVE-FA

	Change from Baseline to Week 72			
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**
mFARS Total*	2.83	1.22	-1.61	**0.144**
Upright Stability	2.99	1.73	-1.26	**0.021**
Bulbar	0.22	0.040	-0.18	**0.044**
Fatigue Scale (MFIS)	4.29	-0.76	-5.05	**0.025**

Upcoming Regulatory Interactions in Q1 2024

- Live Type C Meeting
- EMA Scientific Advice Feedback

*Study primary endpoint


Utreloxastat
ALS Program
CARDINALS

Preclinical and Clinical Evidence Confirm Link Between ALS and Ferroptosis Pathway

Iron accumulation, a marker of ferroptosis, within spinal cord lesions has been reported as an early event in ALS pathogenesis[1]

Oxidized lipids and the 15-lipoxygenase end-product, 4-hydroxy-2,3-nonenal (4-HNE), levels are elevated in ALS patients[2]

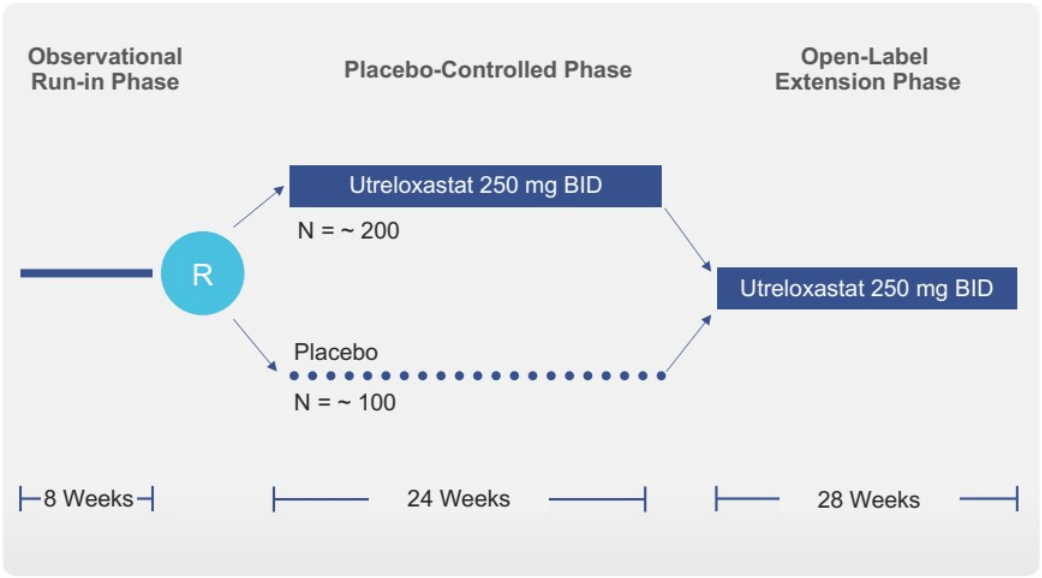
Overexpression of GPX4 protects against motor neuron death[3]

Targeting ferroptosis in ALS in vivo and clinical studies demonstrates improved function and survival

ALS, amyotrophic lateral sclerosis; GPX4, glutathione peroxidase 4. 1. Li Q, et al. Front Neurol. 2022;13:791300. 2. Simpson EP, et al. Neurology. 2004;62:1758–1765. 3. Moreau C, et al. Antioxid Redox Signal. 2018;29:742–748.

CardinALS Study Design

CARDINALS



Key Endpoints

- Change in ALS-FRS Scale
- Respiratory Function
- Survival

Study Timeline

- Enrollment Completed: Q1 202
- Topline Results: Q4 2024

2023


2024