PTC Therapeutics 2024

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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, 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 presentation 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 presentation except as required by law.

2023

Building the PTC of the Future

Focus

Right Sizing

OPEX Reduction

Royalty Financing

PTC Mission Remains Patient Focused

Discover

Develop

Commercialize

Transformative therapies for patients with rare diseases

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

Innovative Science

Splicing

Ferroptosis and Inflammation

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

Innovative Science

Therapeutic Expertise*

Neurology

Metabolism

*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 Creation

translarna™ ataluren

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

Evrysdi® risdiplam

Upstaza™ (eladocagene exuparvovec)

Tegsedi® (inotersen) injection 284 mg/1.5 mL

waylivra® (volanesorsen) injection 285 mg/1.5 mL

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)

PTC THERAPEUTICS

Strong Commercial Performance and Growth in 2023

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Emflaza® (deflazacort)
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waylivra® (volanesorsen) injection 285 mg/1.5 mL

2023

Unaudited Total Revenue

~$946M

Unaudited DMD Franchise Net Product Revenue

~$610M

PTC THERAPEUTICS

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 284 mg/1.5 mL

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

PTC THERAPEUTICS

2024 Revenue and OPEX Guidance

translarna™ ataluren

Emflaza® (deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
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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.

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Key Expected Regulatory & Clinical Milestones in 2024

Upstaza
BLA Submission

Sepiapterin PKU
MAA Submission

PTC518
PIVOT HD
Interim Results

Sepiapterin PKU
NDA Submission

Utreloxastat
CARDINALS
Topline Results

Q1 | **Q2** | **Q3** | **Q4**

Sepiapterin PKU Program

Patient Living with PKU

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APHENITY Results Demonstrate Meaningful Benefit of Sepiapterin in PKU Patients

aphenity

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.

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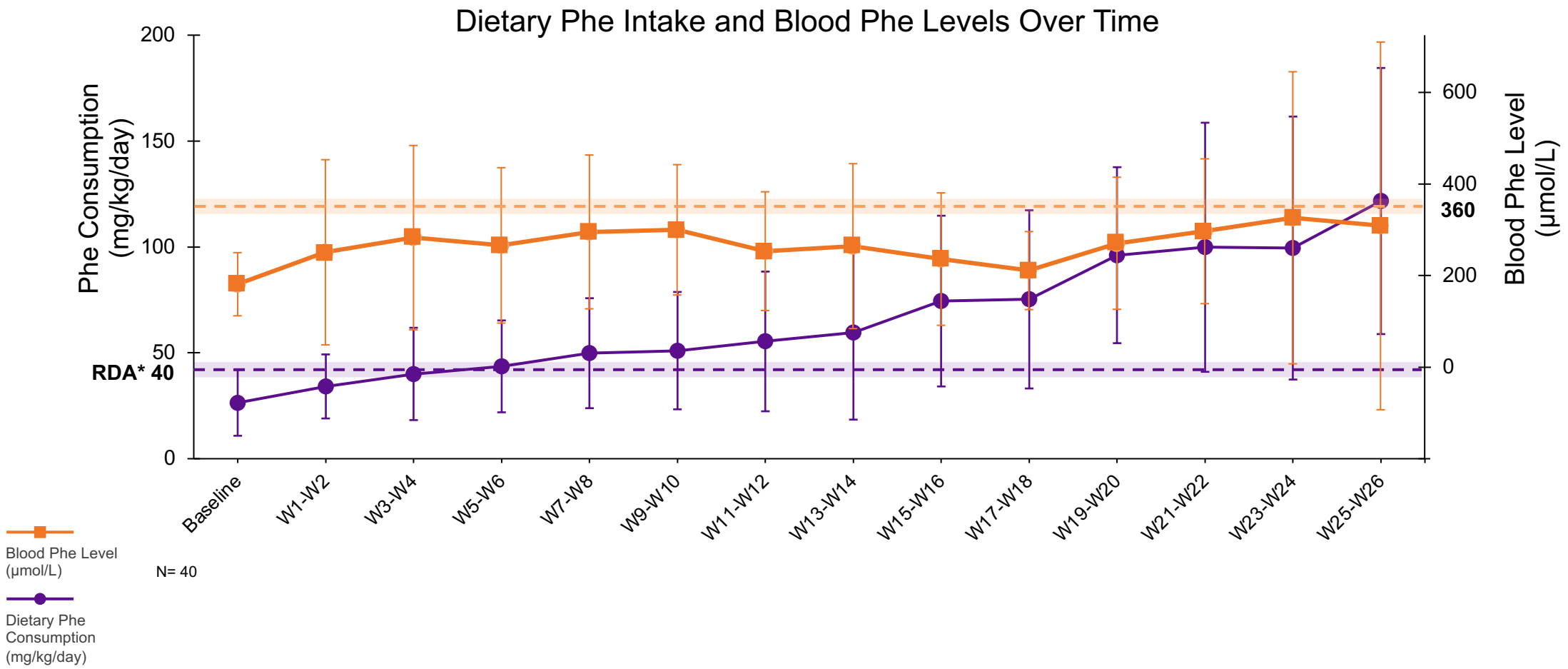
Sepiapterin Enables Increased Dietary Phe Intake in PKU Patients

aphenity extension study



*Recommended dietary allowance

Results based on interim data cut and not full data set

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APHENITY Results Support Potential for Sepiapterin to Address Broad PKU Population

Therapy-Naive Patients Including Classical PKU

Patients Who Have Failed on Current Therapies

Patients Who Are Not Well Controlled by Current Therapies

Greater than $1 Billion Potential Revenue Opportunity

Global Regulatory Submissions and Launch Preparation Planned for 2024

Global Regulatory Submissions

Global Launch Sequence and Continued Launch Preparation

PTC518
HD Program

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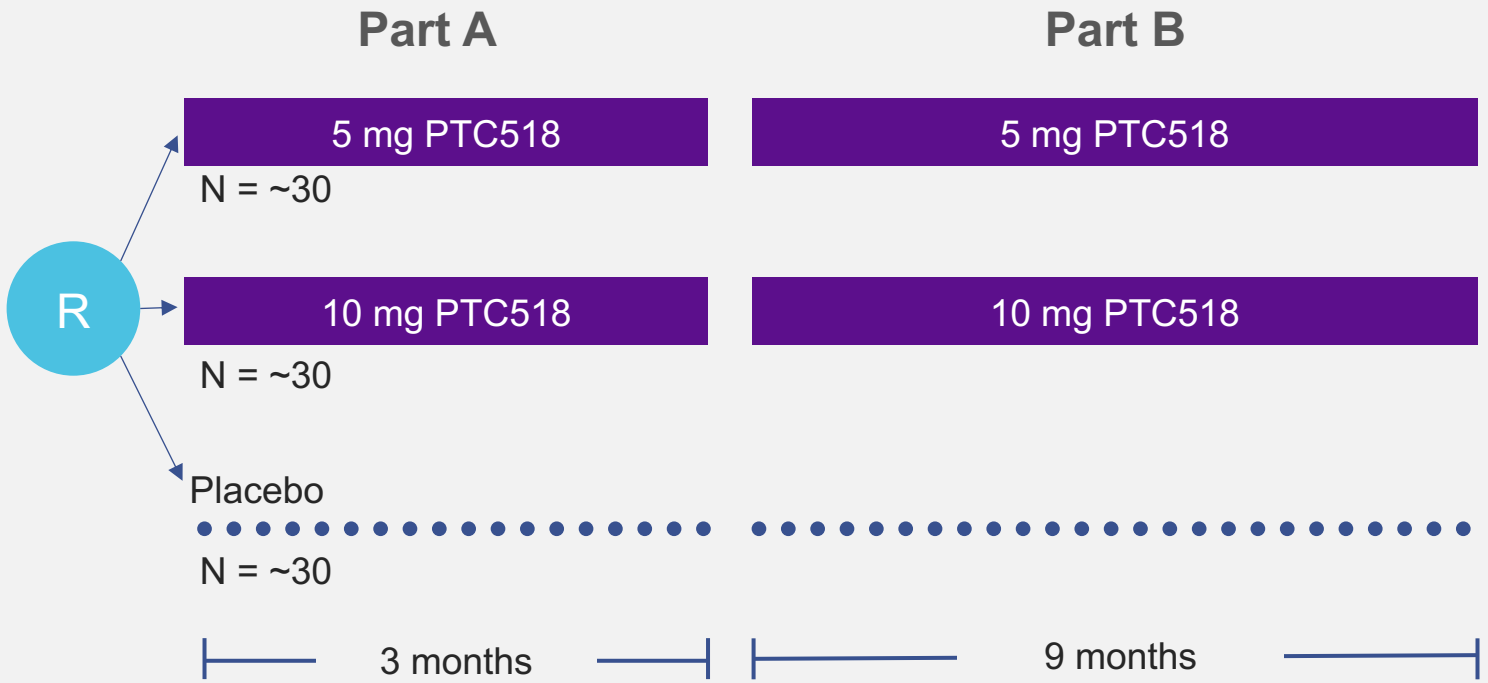
Key Attributes of PTC518 Drive Differentiation

- 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

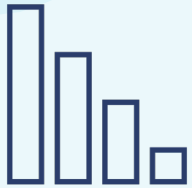
- Percent reduction in mHTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in brain volume on volumetric MRI imaging

12-Week Interim Data Met Key Objectives

PIVOT HD

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

PIVOT HD

- 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

PTC THERAPEUTICS

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

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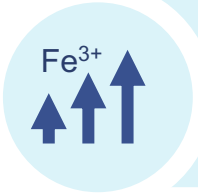
Preclinical and Clinical Evidence Confirm Link Between ALS and Ferroptosis Pathway



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

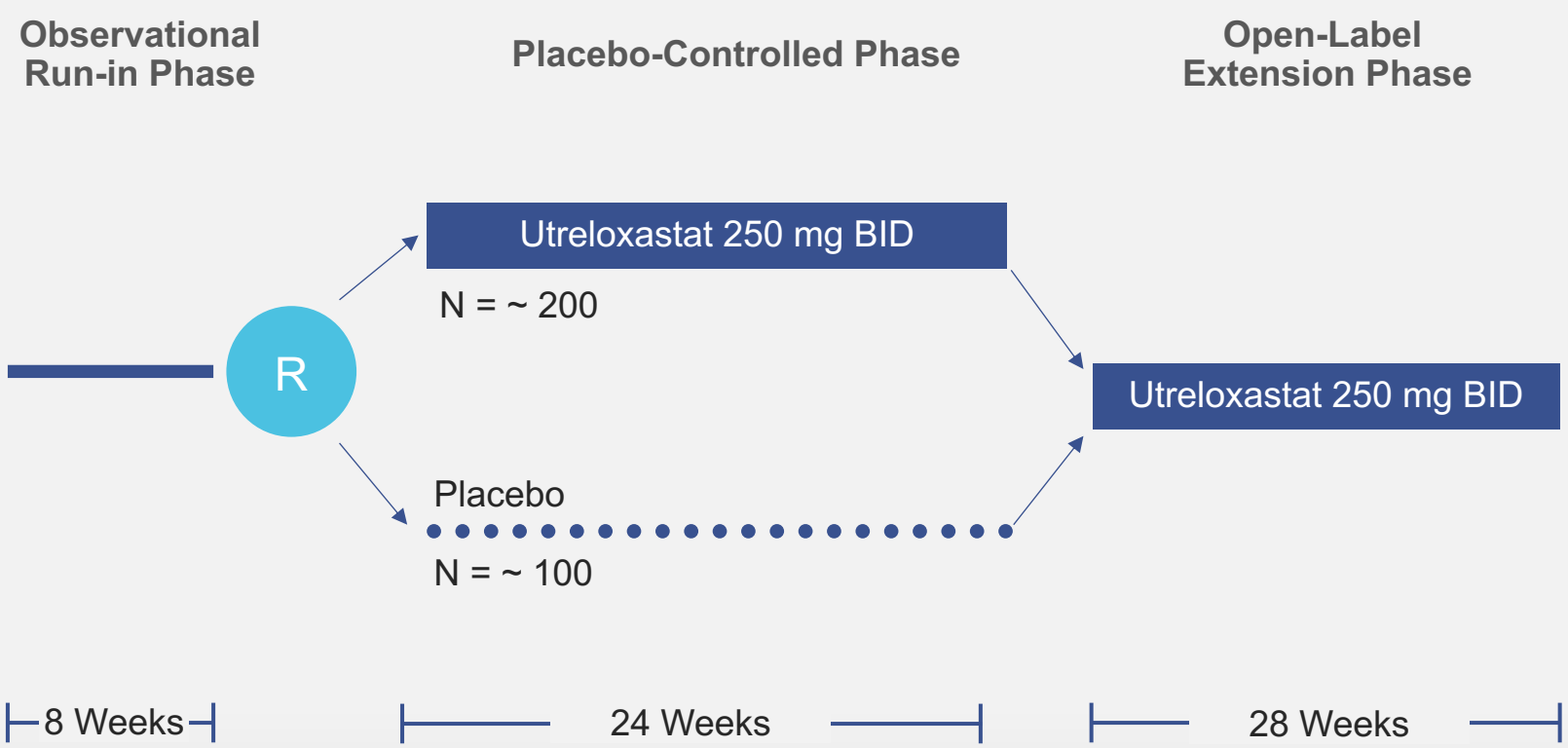
Oxidized lipids and the 15-lipoxygenase end-product, 4-hydroxy-2,3-nonenal (4-HNE), levels are elevated in ALS patients[2]

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



Key Endpoints

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

Study Timeline

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

2023


2024