PTC Therapeutics

November 2024

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 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.

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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, Waylivra or sepiapterin. 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.

Building the PTC of the Future

Focus

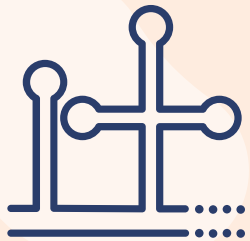
Right Sizing

OPEX Reduction

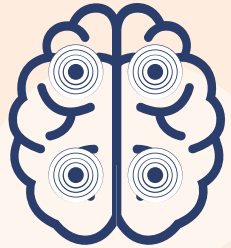
Royalty Financing

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

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

Research

Splicing Platform

SCA-3

MAP-tau

Undisclosed
(Movement Disorders)

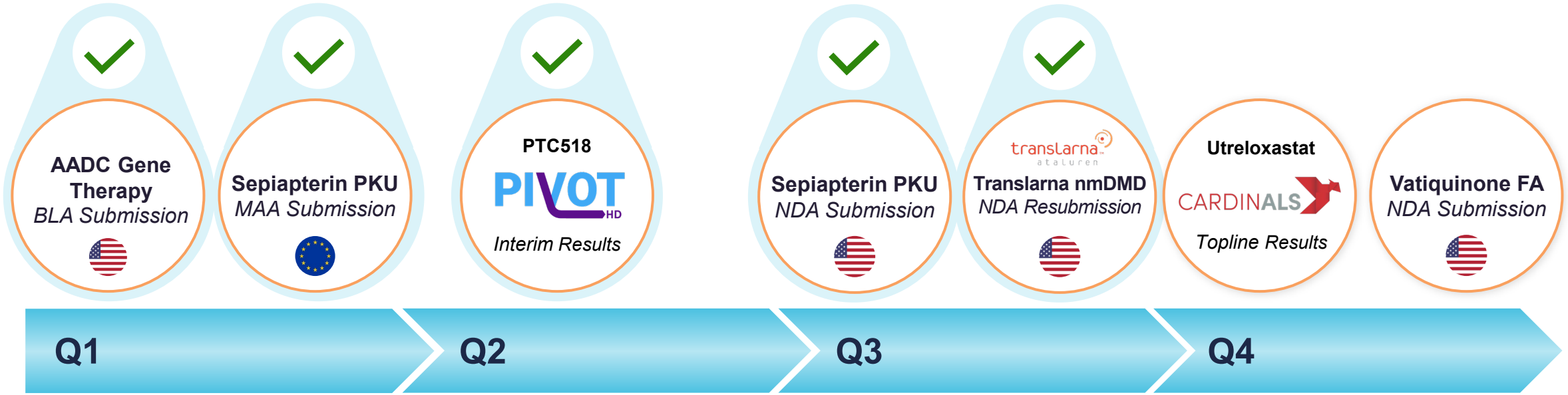
Ferroptosis and Inflammation Platform

Undisclosed
(Neurodegenerative Diseases)

Undisclosed
(Pediatric Neurodevelopment Disorders)

PTC THERAPEUTICS

Key Expected Regulatory & Clinical Milestones in 2024



Key Upcoming Catalysts

- **AADC Gene Therapy** *PDUFA date November 13th, 2024 (PRV eligible)*
- **PTC518 (HD)** *FDA meeting to discuss Accelerated Approval pathway in Q4 2024*
- **Sepiapterin (PKU)** *PDUFA date July 29th, 2025*

Sepiapterin PKU Program

Patient Living with PKU

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%)

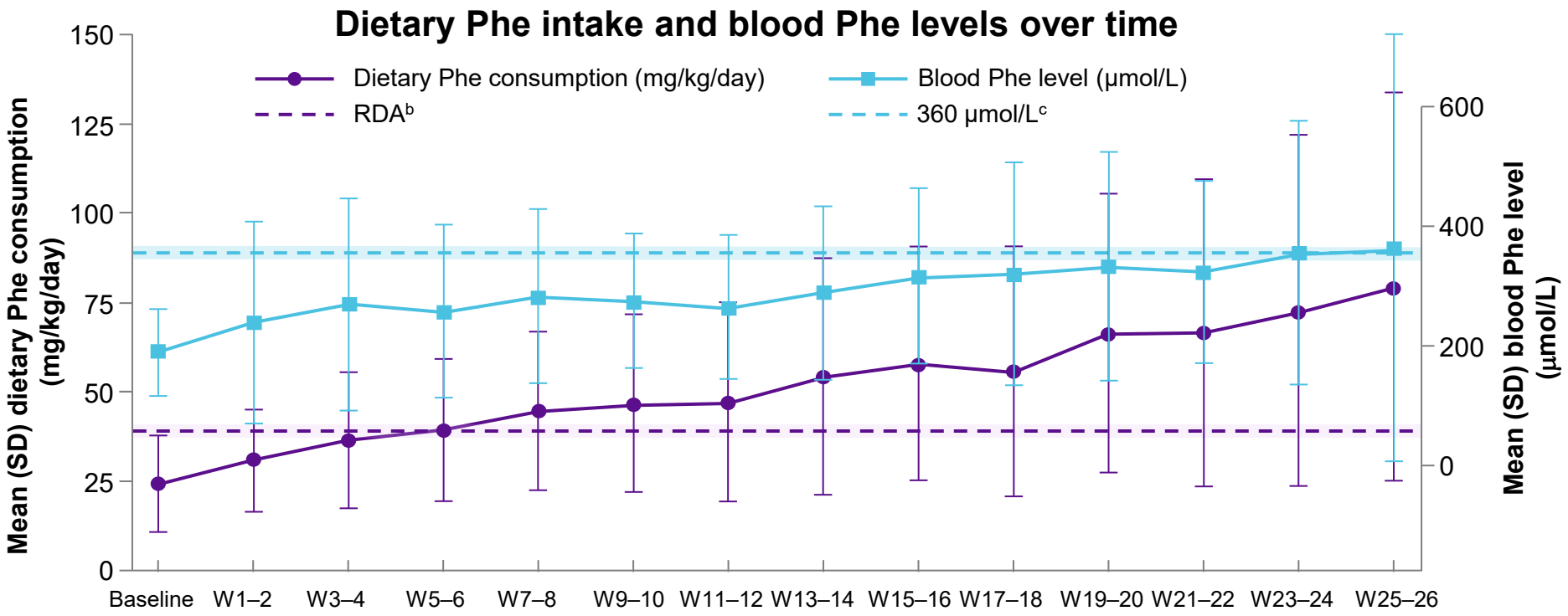
94% of patients ≥ 12 years with blood Phe ≥ 600 µmol/L at baseline achieved a reduction in blood Phe to reach guideline target

Sepiapterin was well tolerated with no serious adverse events

Phe, phenylalanine; PKU, phenylketonuria.

PTC THERAPEUTICS

Interim Results[a]: Sepiapterin Allowed Increased Dietary Phe Intake While Maintaining Blood Phe Within Target Range[1,a]



57% (n = 12/21) of participants reached their age-adjusted RDA by Week 16 and 36% (n = 9/25) reached 2-fold their age-adjusted RDA by Week 20[a,b]

a Data cut-off: 22 September 2023. b The RDA is 0.8 g protein/kg, which is equivalent to approximately 40 mg/kg/day of Phe.
c The US guideline has an upper limit of 360 µmol/L of blood Phe. c 1 g of protein is equivalent to approximately 50 mg of Phe.
Phe, phenylalanine; PKU, phenylketonuria; RDA, recommended dietary allowance; SD, standard deviation; W, Weeks. 1. Sacharow S, et al. Poster presented at SIMD 2024.

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

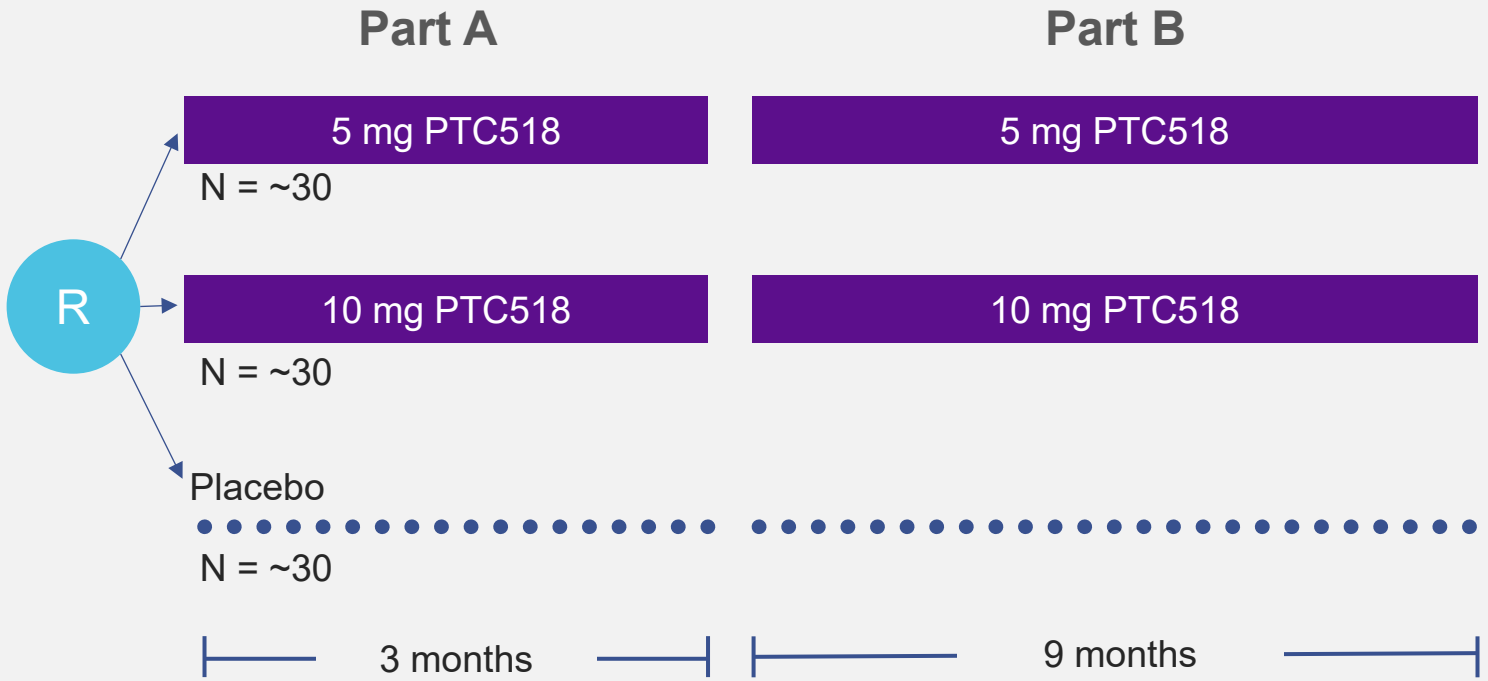
PTC518
HD Program

PTC THERAPEUTICS

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

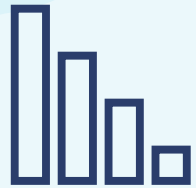
- Percent reduction in mHTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in brain volume on volumetric MRI imaging

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

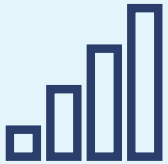
CSF NfL levels remained stable after 12 weeks of treatment with no treatment-related spikes

Evidence of Durability of Effect, Safety and Dose-Dependent Benefit on Clinical Measures

PIVOT HD

Dose-dependent and durable lowering of HTT protein in blood at 12 months

Dose-dependent lowering of CSF mHTT levels



Dose-dependent trends of improvement on key clinical measures including TMS and cUHDRS

PTC518 was well tolerated with no evidence of treatment-related NfL spikes at 12 months

Note: Results based on interim readout

PTC THERAPEUTICS

Vatiquinone FA Program

Patient Living with FA

Meaningful Clinical Results in MOVE-FA

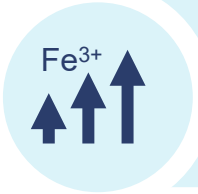
	Change from Baseline to Week 72			
Analysis	**Placebo**	**Vatiquinone**	**Difference**	**P-value**
mFARS Total*	2.83	1.22	-1.61	**0.144**
Upright Stability	2.99	1.73	-1.26	**0.021**
Bulbar	0.22	0.040	-0.18	**0.044**
Fatigue Scale (MFIS)	4.29	-0.76	-5.05	**0.025**

NDA Submission Expected in December 2024

*Study primary endpoint

Utreloxastat ALS Program

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]

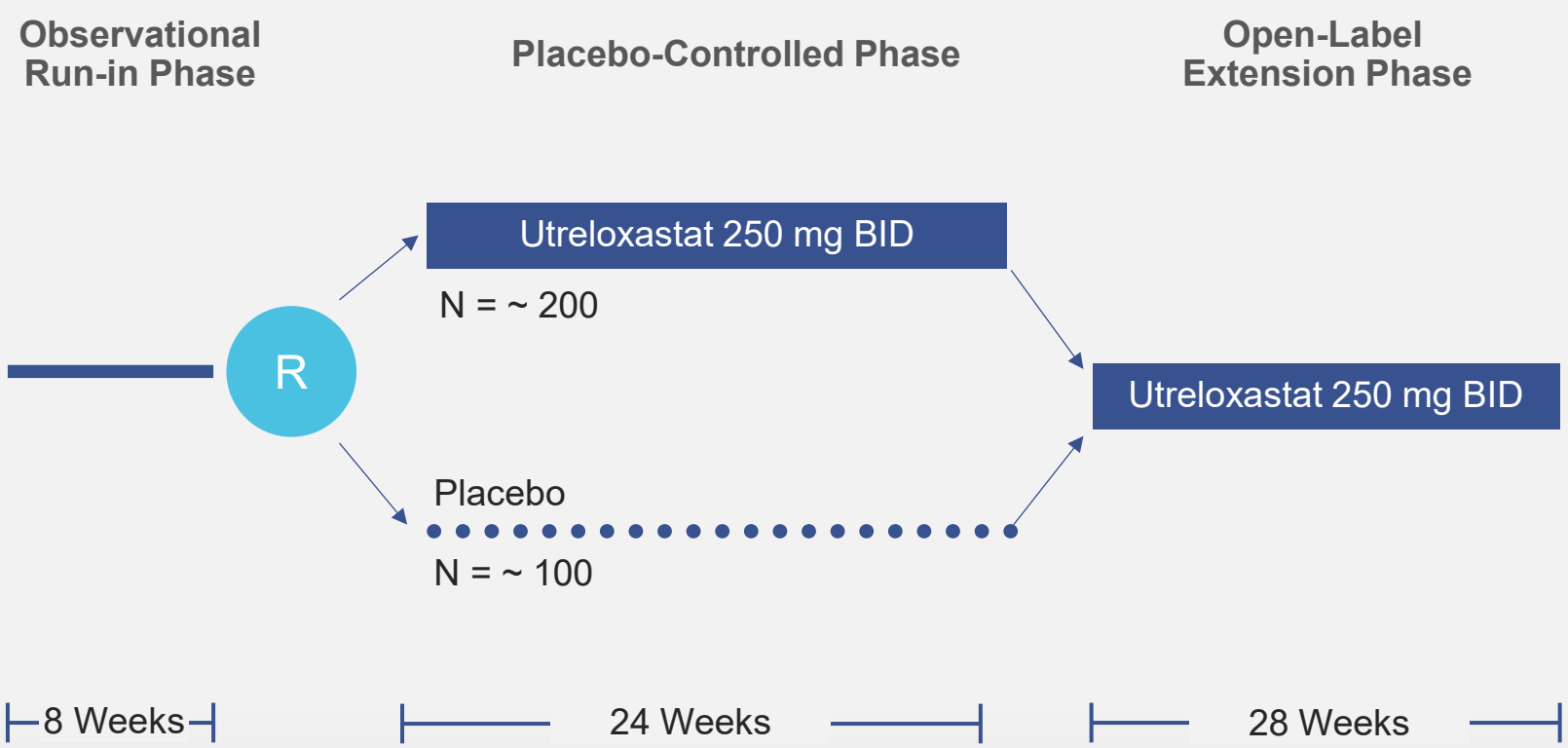
Oxidized lipids and the 15-lipoxygenase end-product, 4-hydroxy-2,3-nonenal (4-HNE), levels are elevated in ALS patients[2]

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

PTC Therapeutics

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