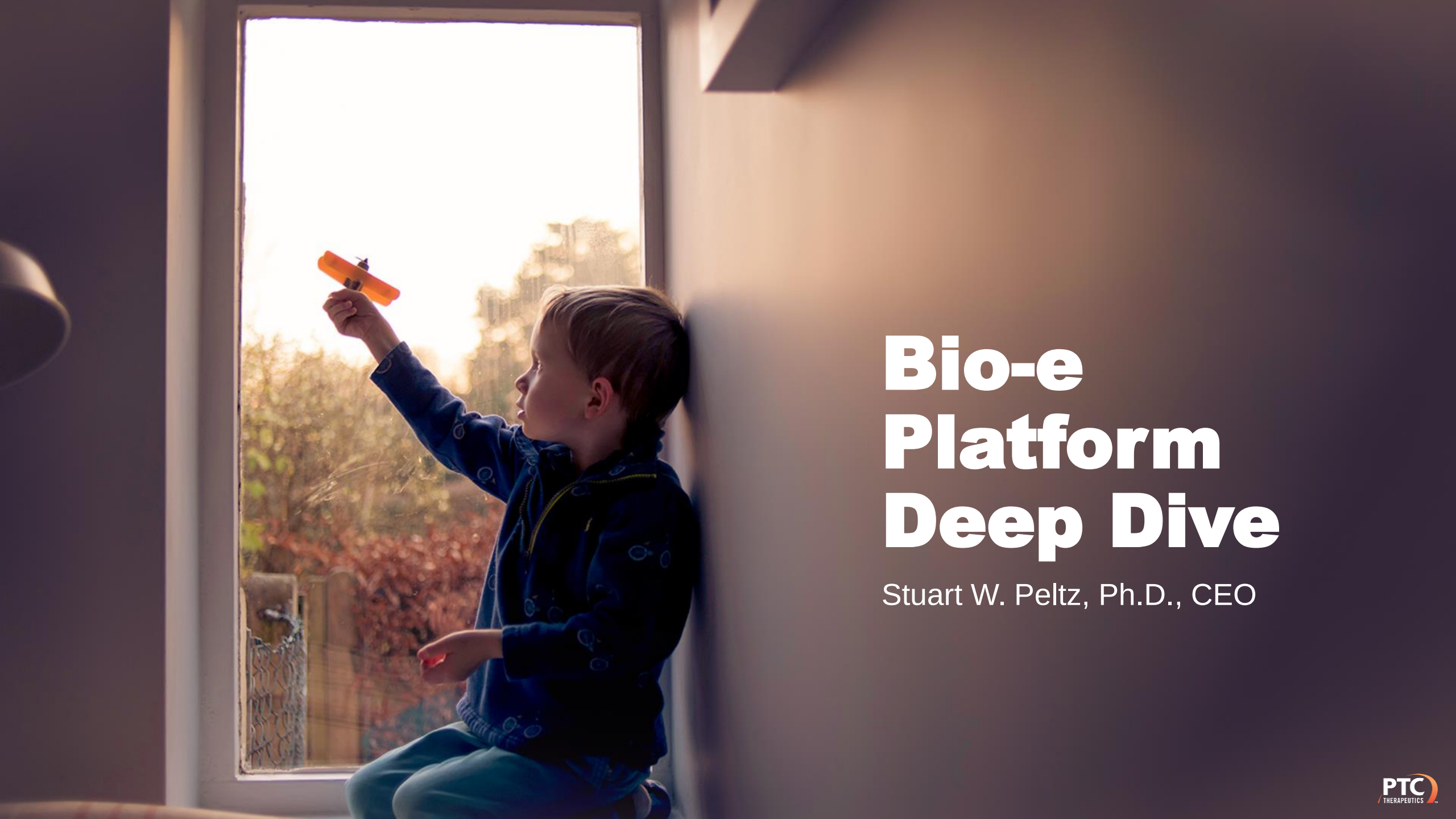


Bio-e Platform Deep Dive

Stuart W. Peltz, Ph.D., CEO

Forward Looking Statement

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 future revenue 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 and other matters; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of 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; 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; 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.

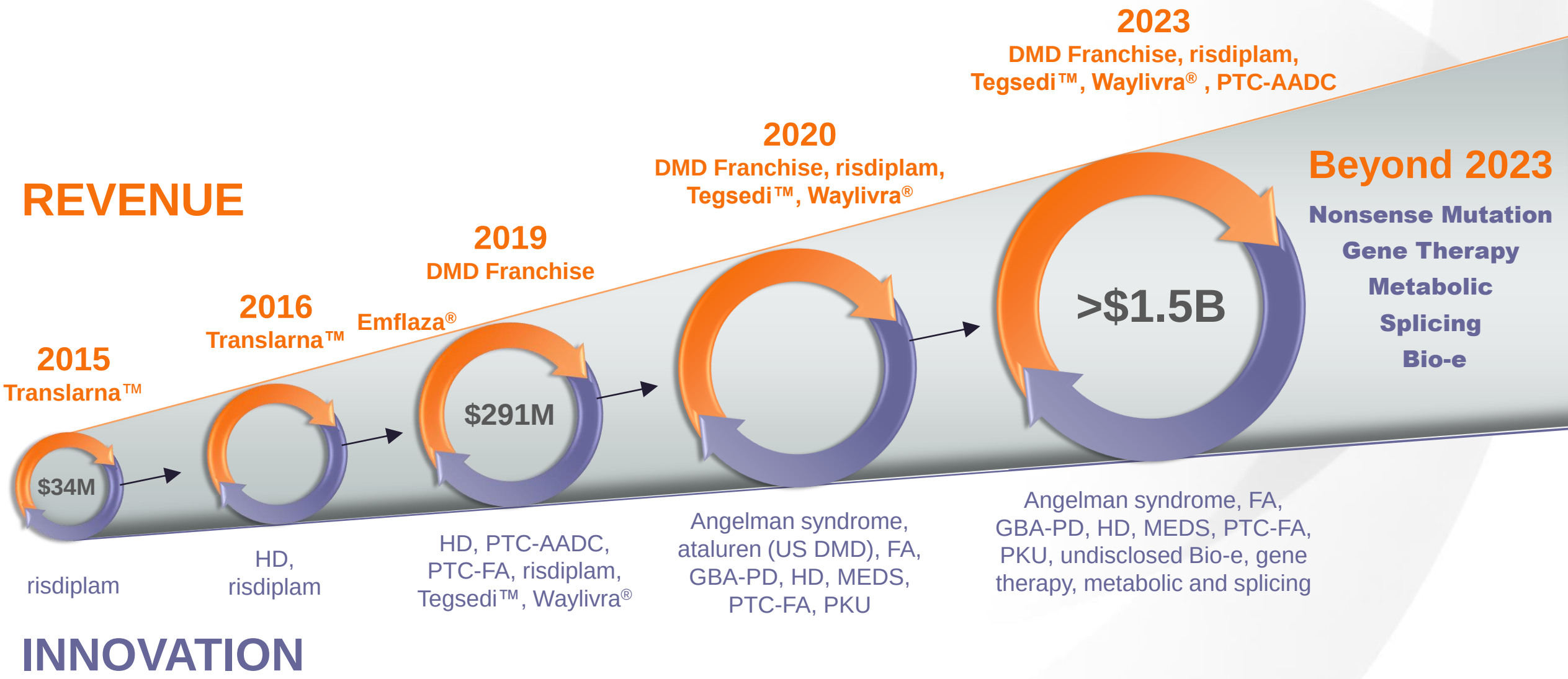
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or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

Multiplatform, Diversified Pipeline Built Through Internal Innovation & Strategic Business Development

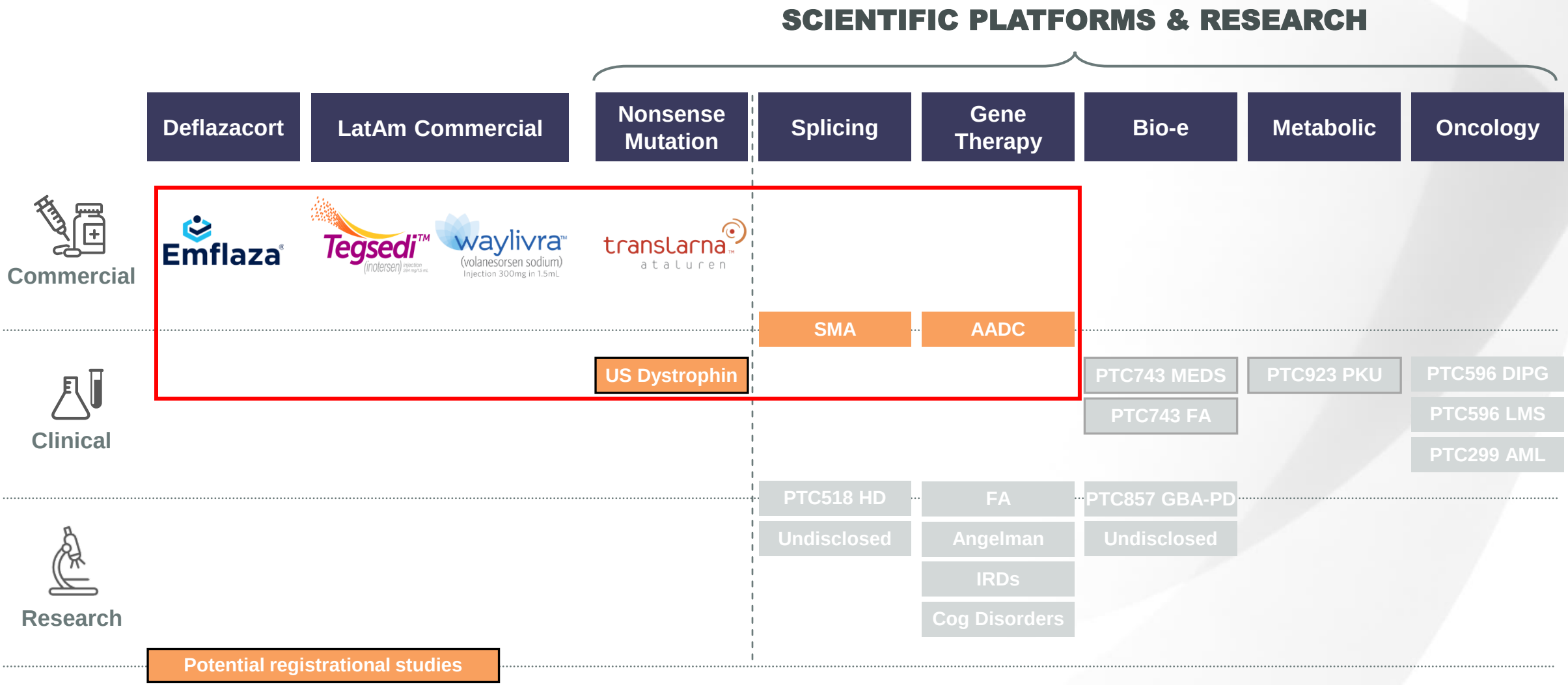
SCIENTIFIC PLATFORMS & RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology
Commercial	Emflaza®	Tegsedi™ (inotersen) injection 284 mg/3.5 mL; waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren					
Clinical			US Dystrophin	SMA	AADC	PTC743 MEDS PTC743 FA	PTC923 PKU	PTC596 DIPG PTC596 LMS PTC299 AML
Research				PTC518 HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed		
	Potential registrational studies							

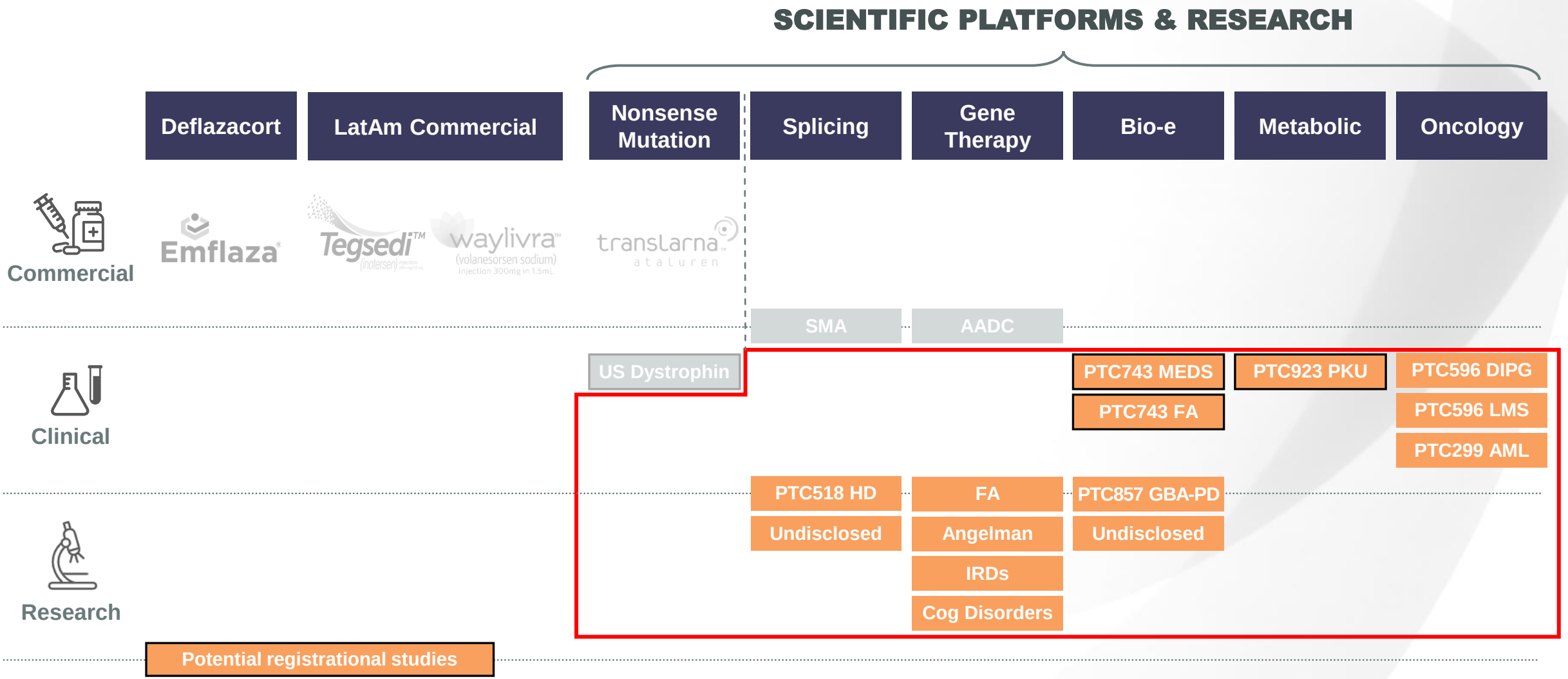
Multiplatform Approach Drives Sustainable Innovation & Continuous Value Creation



>$1.5B Revenue Target Includes Commercial Products & Therapies Nearing Potential Launch



Majority of Pipeline Not Represented in >$1.5B Revenue Target



Bio-e Platform has Potential to Generate Substantial Additional Value

SCIENTIFIC PLATFORMS & RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology
Commercial	Emflaza®	Tegsedi™ (inotersen) injection 284mg/1.5mL waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren					
Clinical			US Dystrophin	SMA	AADC	PTC743 MEDS PTC743 FA	PTC923 PKU	PTC596 DIPG PTC596 LMS PTC299 AML
Research				PTC518 HD Undisclosed	FA Angelman IRDs Cog Disorders	PTC857 GBA-PD Undisclosed		

Potential registrational studies

Bio-e Platform Overview

Platform capabilities

Based on a family of oxidoreductase enzyme targets critical to generation and regulation of energy key to disease pathology

Novel approach

Intersection of electron-transfer chemistry and biology

Validated target and mechanism of action

Lead compound PTC743 targets the enzyme 15-lipoxygenase – a key enzyme hub that regulates inflammation and oxidative stress

Extensive pediatric safety and exposure history

PTC743 has been evaluated in over 500 patients – mostly children – with duration of exposure up to 10 years, and has been safe and well-tolerated

Pipeline potential

Large number of oxidoreductase targets with known biological significance (> 100), and diverse redox small molecule library

Initiating Three Bio-e Clinical Trials in 2020

PTC743

Mitochondrial Epilepsy Trial

Trial Starting 3Q20

- Proof-of-concept established in dozens of patients
- Clinical trials demonstrated reduction in hospitalizations and mortality risk in mitochondrial epilepsy patients
- Enrolling patients with 4 most common sub-types of mitochondrial epilepsy

5-6K

patients in the US and EU

PTC743

Friedreich Ataxia Trial

Trial Starting 4Q20

- Mechanism linked to FA pathology
- >60 subjects treated; Improvement in FARS compared to natural history
- Potentially complementary with FA gene therapy

25K

patients WW

PTC857

Phase 1 Trial

Trial Starting 3Q20

- Targeting GBA Parkinson's disease as first indication
- Inhibits alpha-synuclein oxidation and aggregation in preclinical studies
- Protects dopamine-related motor function in MPTP mouse

~50 – 90K

patients in the US

Matthew B. Klein, MD, MS, FACS

Chief Development Officer at PTC Therapeutics, Inc.

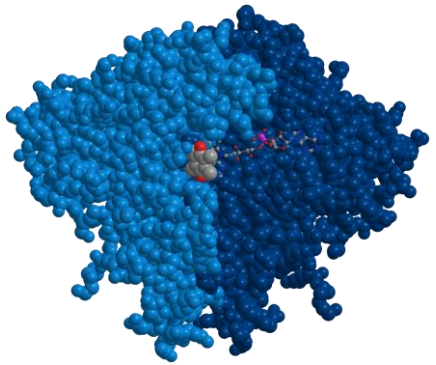
Prior to joining PTC, Dr. Klein was CEO and Chief Medical Officer of BioElectron Technology Corporation, a biotechnology company focused on development of redox active small molecules for mitochondrial disease and related disorders of oxidative stress. Prior to joining BioElectron, Dr. Klein was the Auth-Washington Research Foundation Chair of Restorative Burn Surgery at the University of Washington. Dr. Klein completed his undergraduate degree at the University of Pennsylvania where he graduated summa cum laude and Phi Beta Kappa, and received his MD degree with honors from Yale University.

Bio-e Platform

Matthew Klein, MD, MS, FACS
Chief Development Officer

Bio-e Discovery Platform

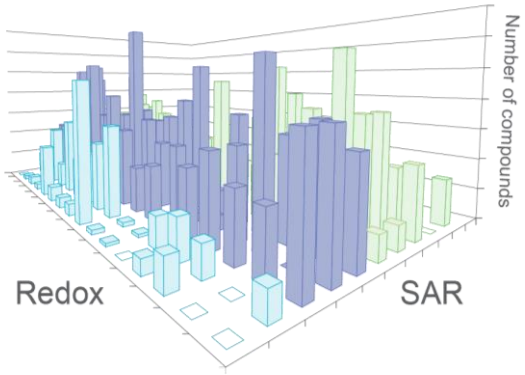
Novel Targets



Oxidoreductase enzymes

Known enzymes that regulate critical biological function through electron-transfer chemical reactions

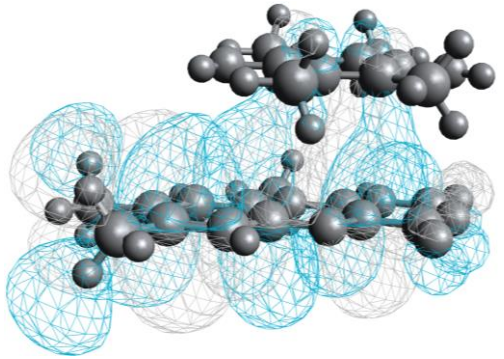
Proprietary Library



Redox active small molecules

Compounds that activate or inhibit oxidoreductase activity through electron-transfer chemistry

Specialized Approach



Redox discovery tools

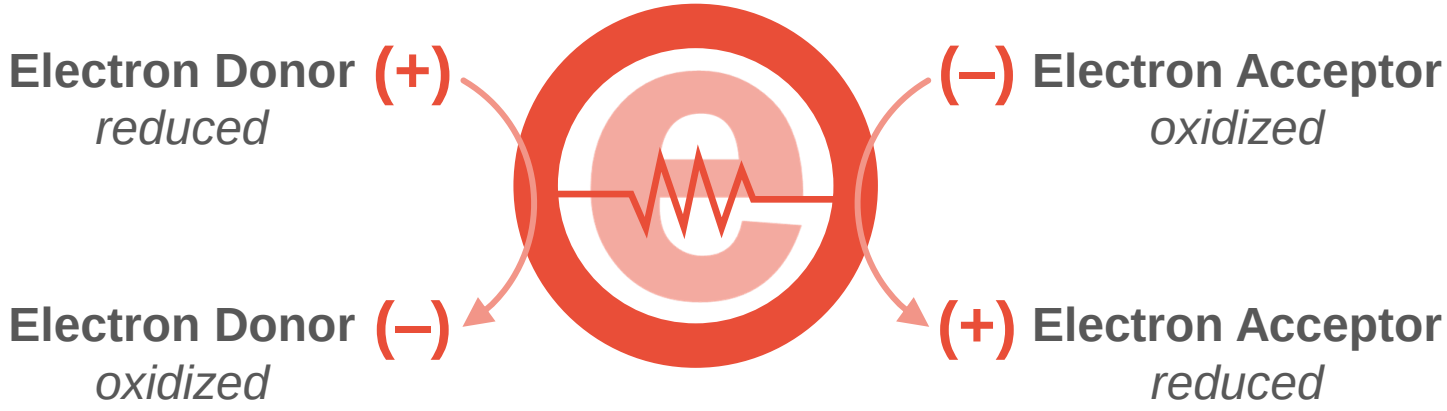
Enzymology, screening assays and medicinal chemistry that account for structural and redox activity

Scientific & Technical Expertise

Unique knowledge to enable the discovery, optimization and development of first-in-class therapeutics

Novel Targets: Oxidoreductases

Oxidoreductase enzymes catalyze the transfer of electrons from one molecule, the electron donor, to another molecule, the electron acceptor

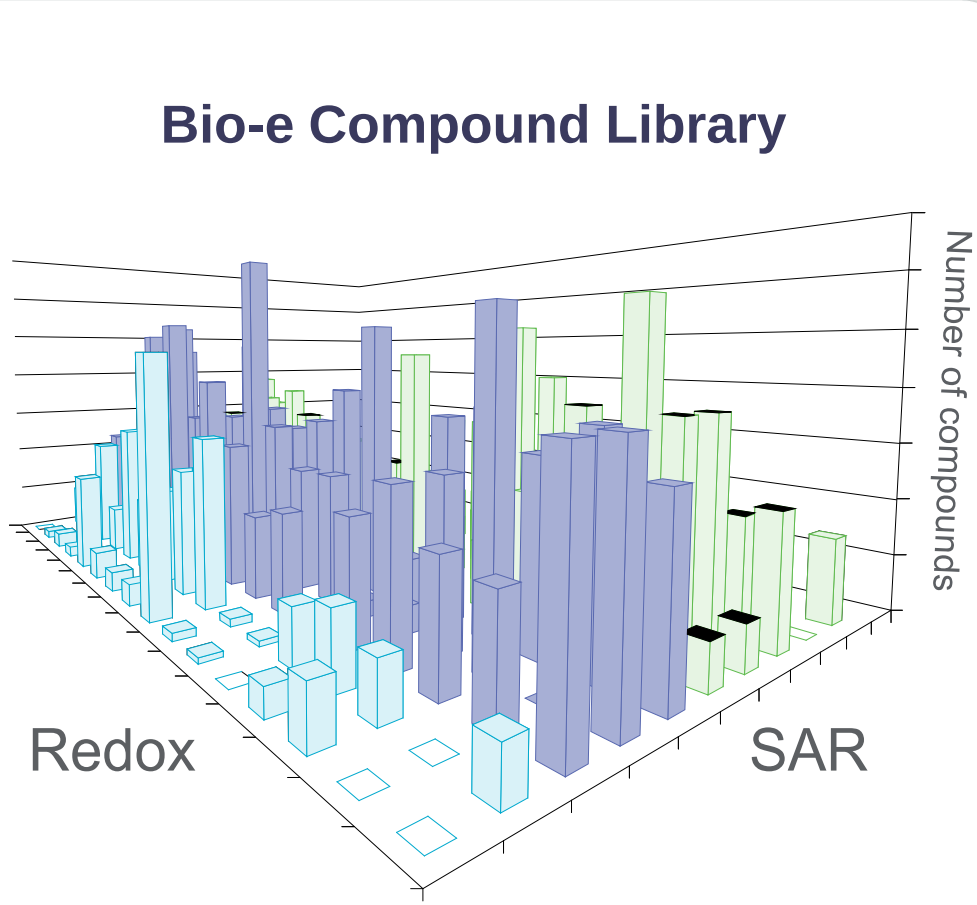


Oxidoreductase enzymes have known biological significance in the regulation of energy generation and oxidative stress

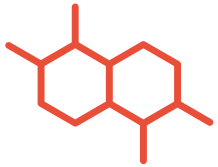
Novel Compounds: Bio-e Active Small Molecules

PTC Bio-e compound library consists of 16 unique chemical series with structural and redox diversity

Bio-e active compounds optimized for properties that improve oxidoreductase enzyme target selectivity to modulate biological function



Compound Characteristics



Small Molecule

Orally Bioavailable

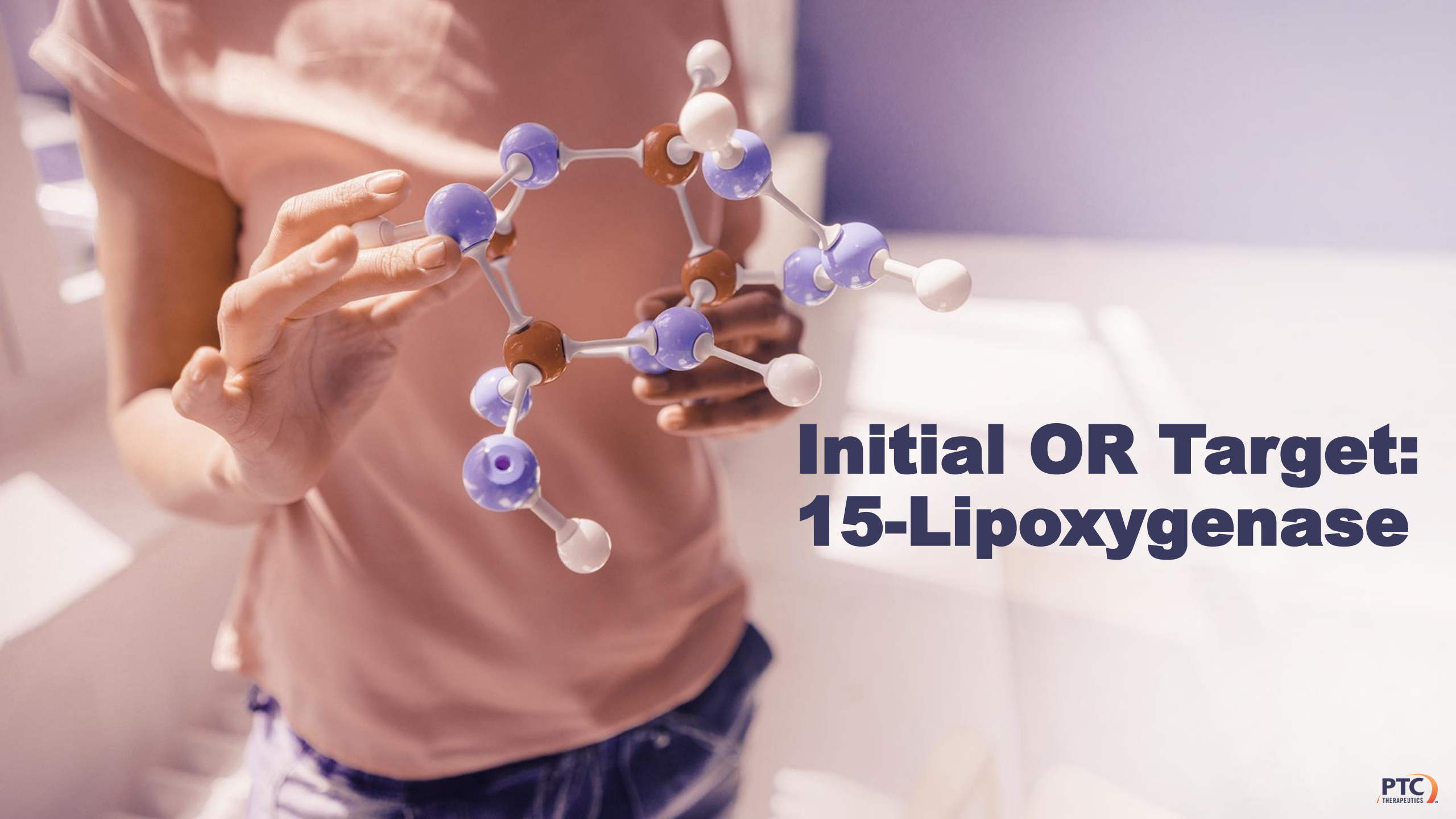
Highly Selective

Defined Redox Activity

Catalytically recycled

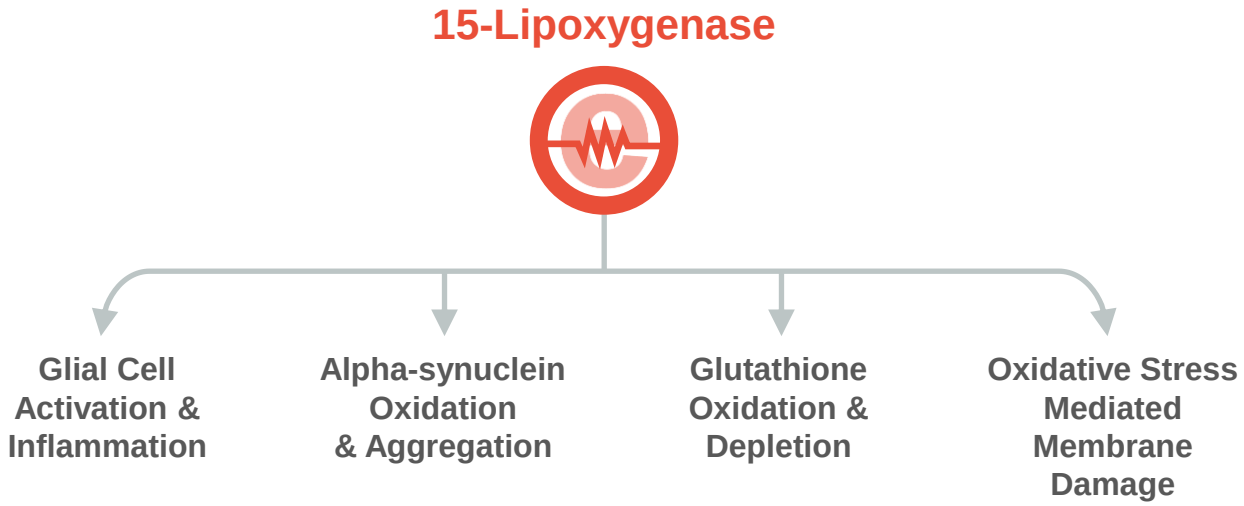
Low nanomolar potency

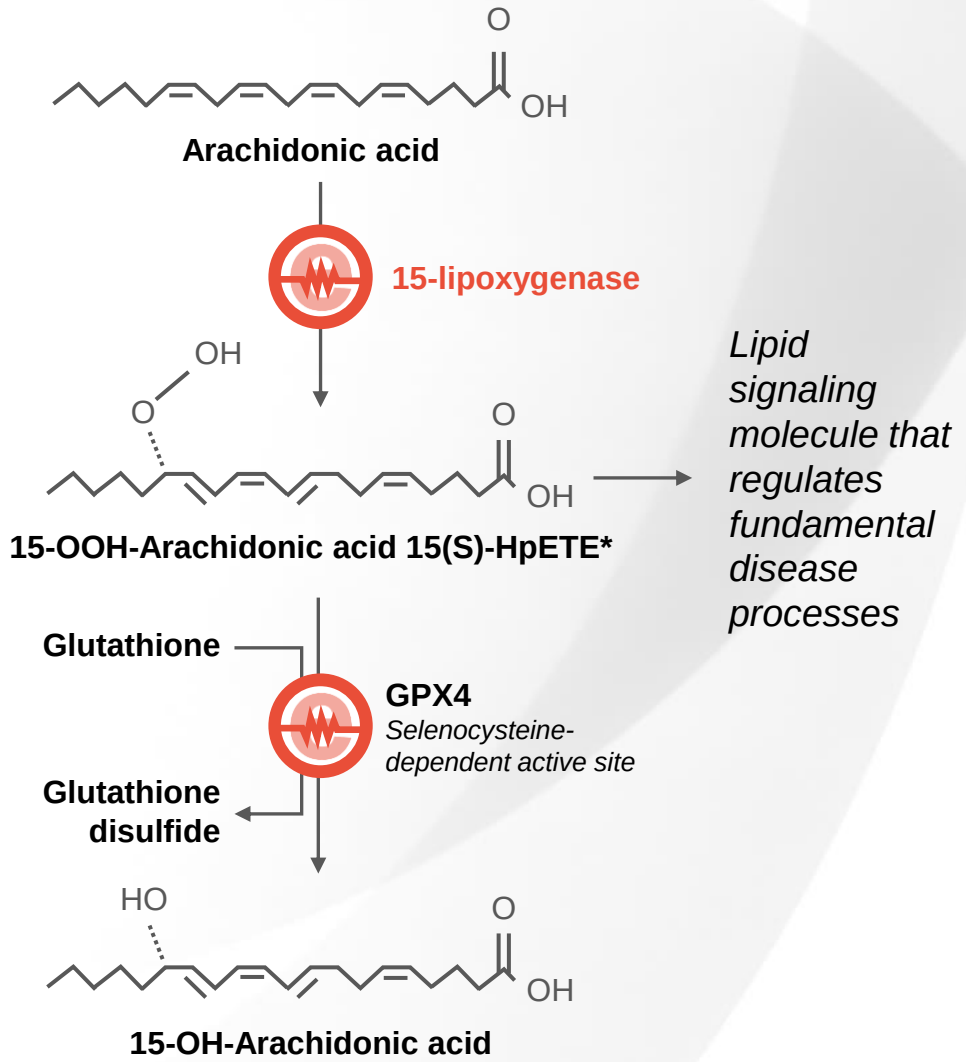
Scalable manufacturing



Initial OR Target: 15-Lipoxygenase

15-Lipoxygenase is a Key Regulator of Inflammation and Oxidative Stress Pathways in CNS Diseases

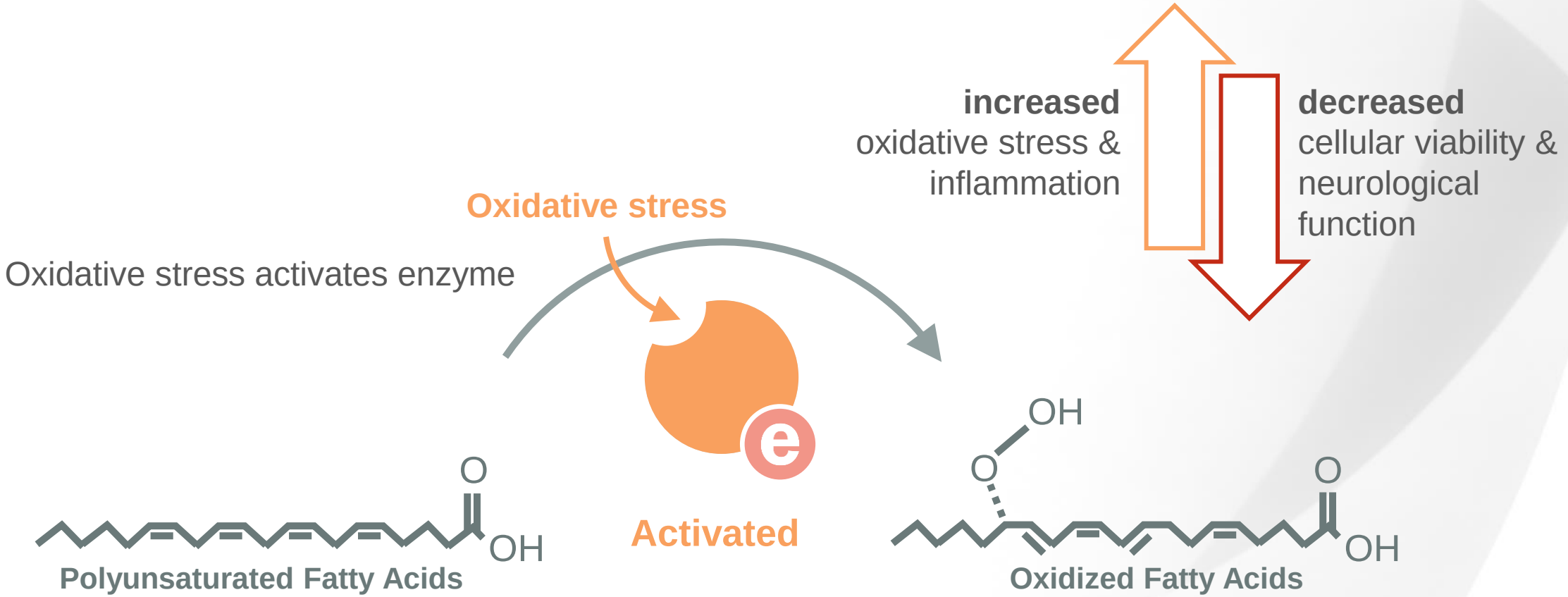




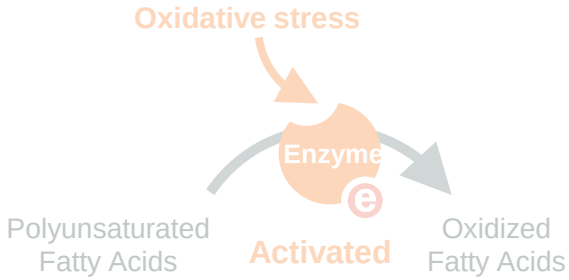
Oxidative Stress

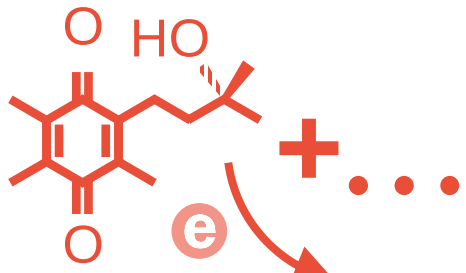
- Results from an imbalance between the production of free radicals (i.e. oxygen, nitrogen) and the ability of the body to detoxify them
- Free radicals can arise from a variety of disease processes
  - Mitochondrial disease: electron transport chain defects
  - Genetic Parkinson's disease: genetic mutation affecting the mitochondria or cell membranes
- Oxidative stress results in the activation of 15-lipoxygenase

Oxidative Stress Activates 15-Lipoxygenase Resulting in Increased Oxidative Stress and Inflammation

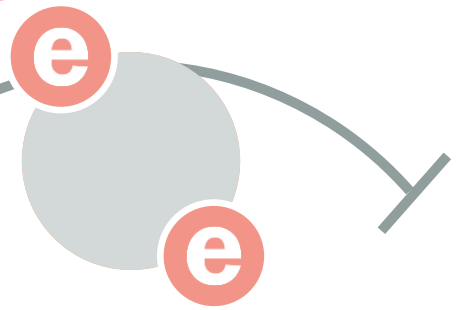


PTC Compounds Inhibit 15-Lipoxygenase to Decrease Oxidative Stress and Inflammation and Improve Cell Viability





PTC Compounds



Inhibited

Polyunsaturated Fatty Acids

Oxidized Fatty Acids

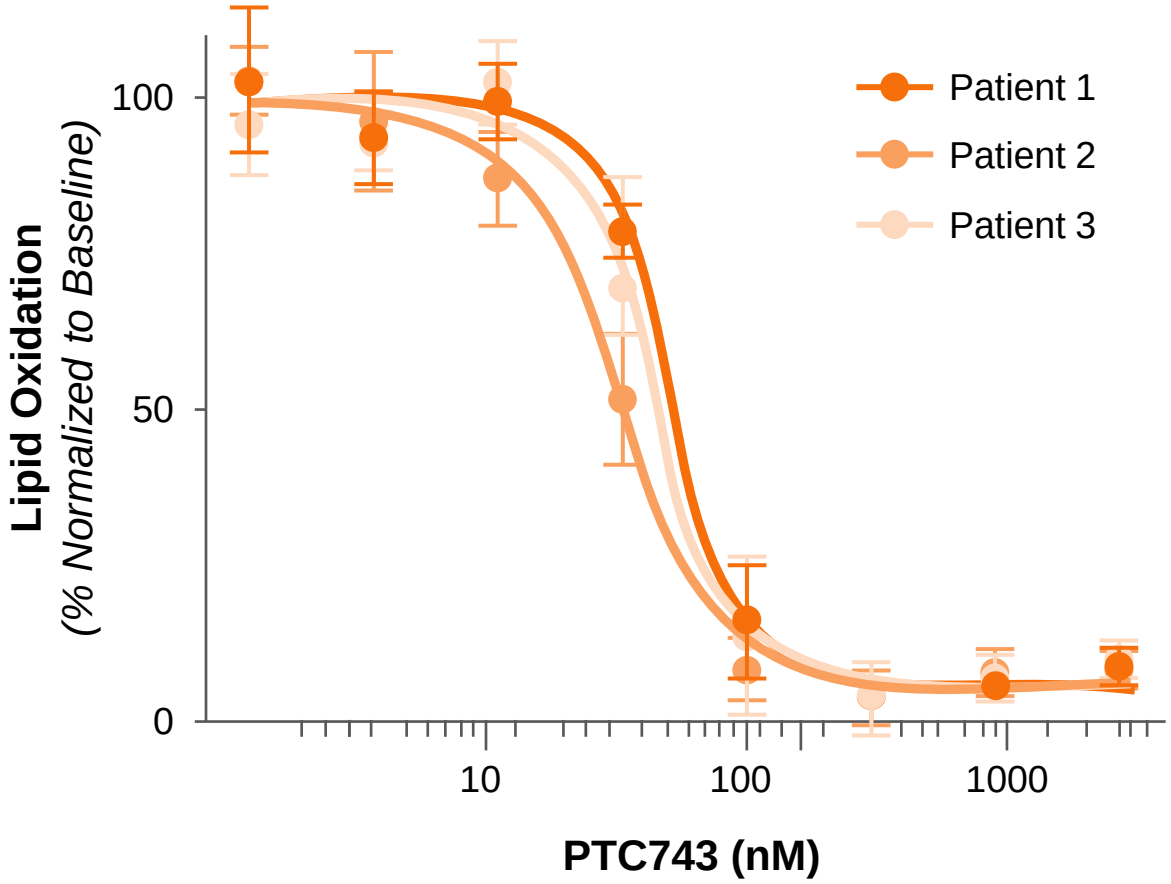
decreased inflammation & oxidative stress

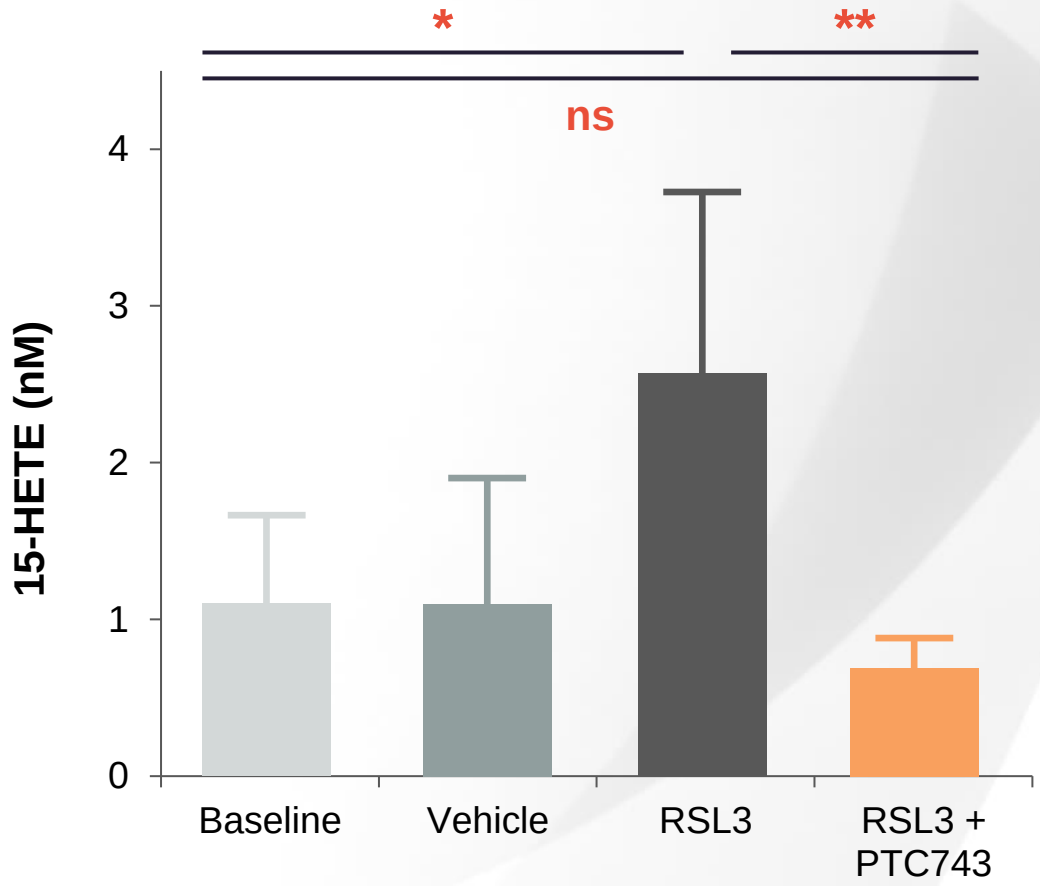
increased cellular viability & neurological function

PTC743: Overview

- Target: 15-Lipoxygenase (15-LO)
- MOA: Regulation of inflammation, oxidative stress, glutathione depletion and cell death
- Route of Administration: Orally bioavailable
- Designations: 7 Orphan, 1 FDA fast track
- Target Indications: Refractory mitochondrial epilepsies (Ph 2/3), Friedreich ataxia (Ph 3)
- Subjects Treated: >500, longest exposure > 10 years
- Safety: Well tolerated with strong safety record

PTC743 Inhibits 15-Lipoxygenase to Prevent Accumulation of Damaging Oxidized Lipids

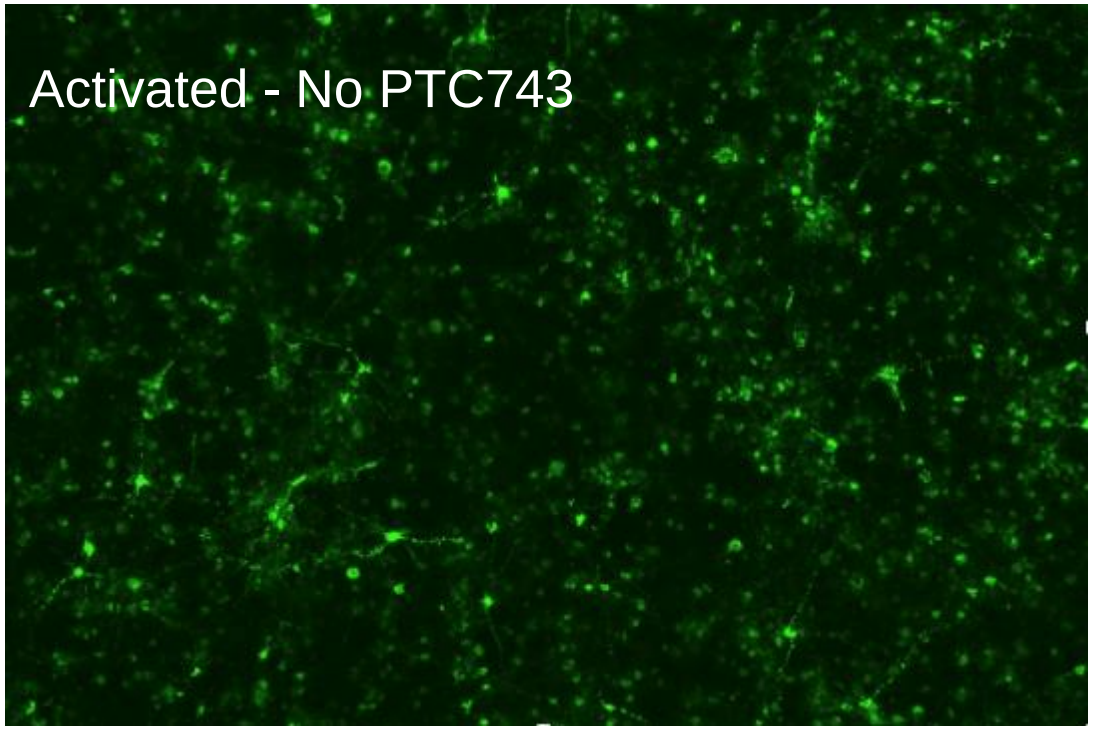


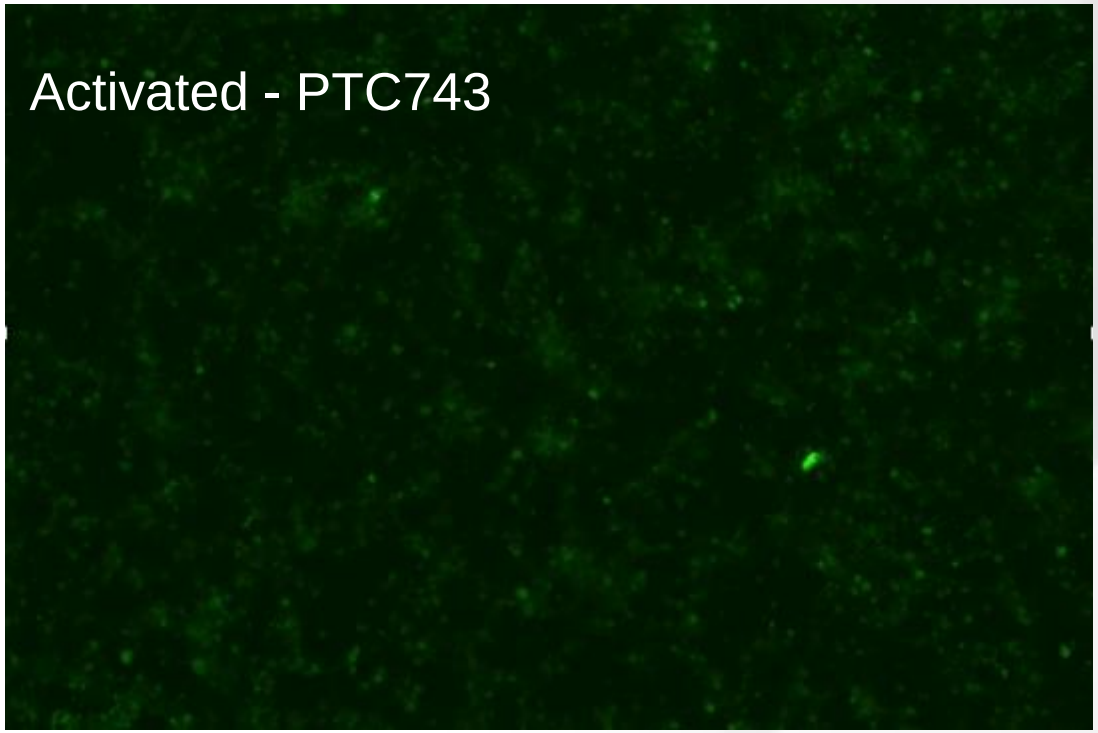


Patient fibroblasts (n=3 donors) treated with RSL3 to induce lipid oxidation and cell death. Lipid oxidation assessed by BODIPY fluorescence by quantitative time-lapse microscopy. Mean +/- SEM, in technical triplicate. The15-lipoxygenase product 15-Hydroxyeicosatetraenoic acid (15-HETE) was measured by LC-MS/MS. Mean ± SEM (N=6 replicates per patient culture).

PTC743 Prevents Lipid Oxidation of Primary Neurons

Rat Cortical Primary Neurons





BODIPY C11 fluorescence images were acquired every 3hrs for 40hrs. Images collected after 18hrs exposure to BSO, immediately before addition of compound.

Mitochondrial Disease

- Family of inherited disorders causing defects in mitochondrial structure and function
- Overall incidence ~1:5000
- Genetic subtypes share common biochemistry of oxidative stress, inflammation and depletion of endogenous antioxidant system
- Typically diagnosed in first 1-2 years of life
- Highly morbid, typically fatal in early childhood
- No approved therapies

Initial PTC743 Study: Expanded Access Program

Study Overview

- Enrolled patients (2009-2012) with inherited mitochondrial disease **within 90 days of end-of-life care**
- Initial treatment of 13 weeks with long-term extension
- Measured survival, drug safety & pharmacokinetics

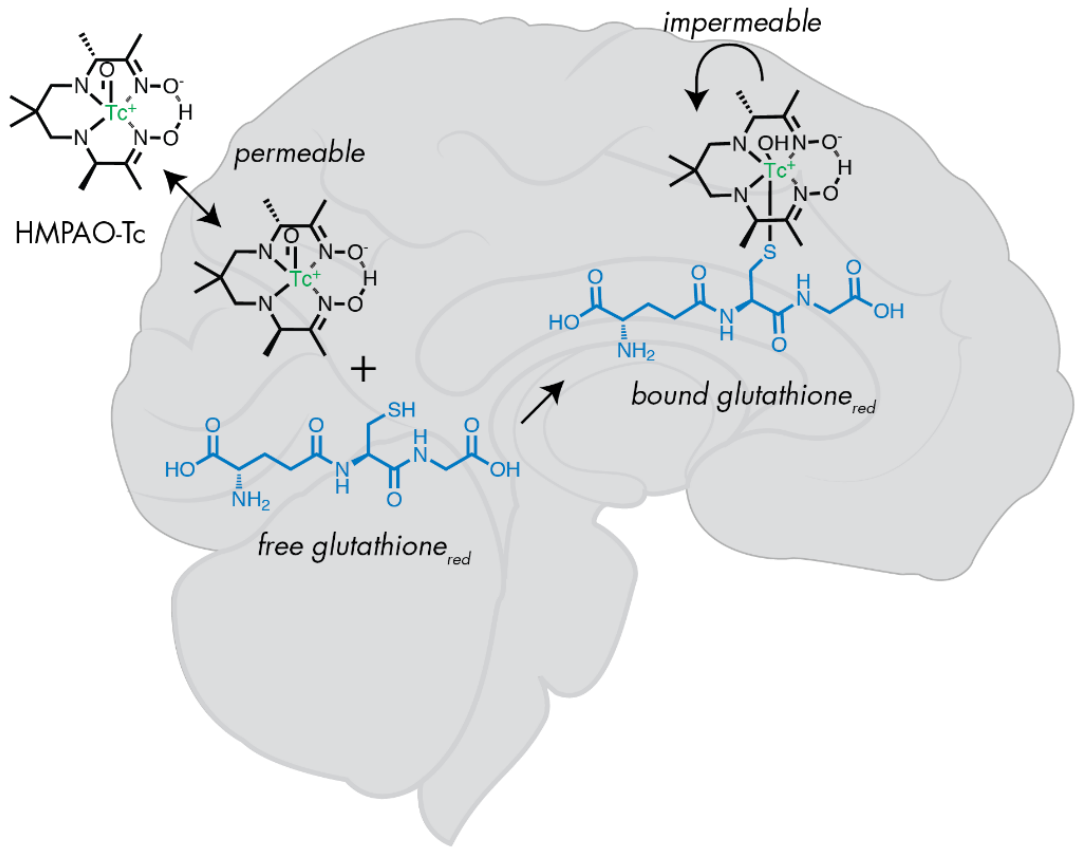
Study Results

- Significant survival effect – **42 of 94 subjects alive and on drug for more than a decade**
- Clinical effects across subtypes includes reduced seizure frequency, improved neuromuscular and neurological function, decreased transfusion requirements, improved liver function
- CNS and blood-based biomarker response consistent with PTC743 MOA and disease pathology
- Safe and well-tolerated

Expanded Access Clinical Results: Physician Reports

- “No seizures, no blackouts, gaining weight, sleeping better, less pain”
- “Significant appetite increase, walking better with minimum support, able to pick her feet up, energy is way up”
- “Seizures under much better control, myoclonus is much better as well. These objective measures holding true for the duration of the trial are rare in this disorder”
- “Fewer illnesses, more alert, recovers more quickly, and no clinical seizures in 6 months”
- “All my POLG patients are stable or improving [on PTC743]. This is not what Dr. Alpers or Huttenlocher thought about the natural history”

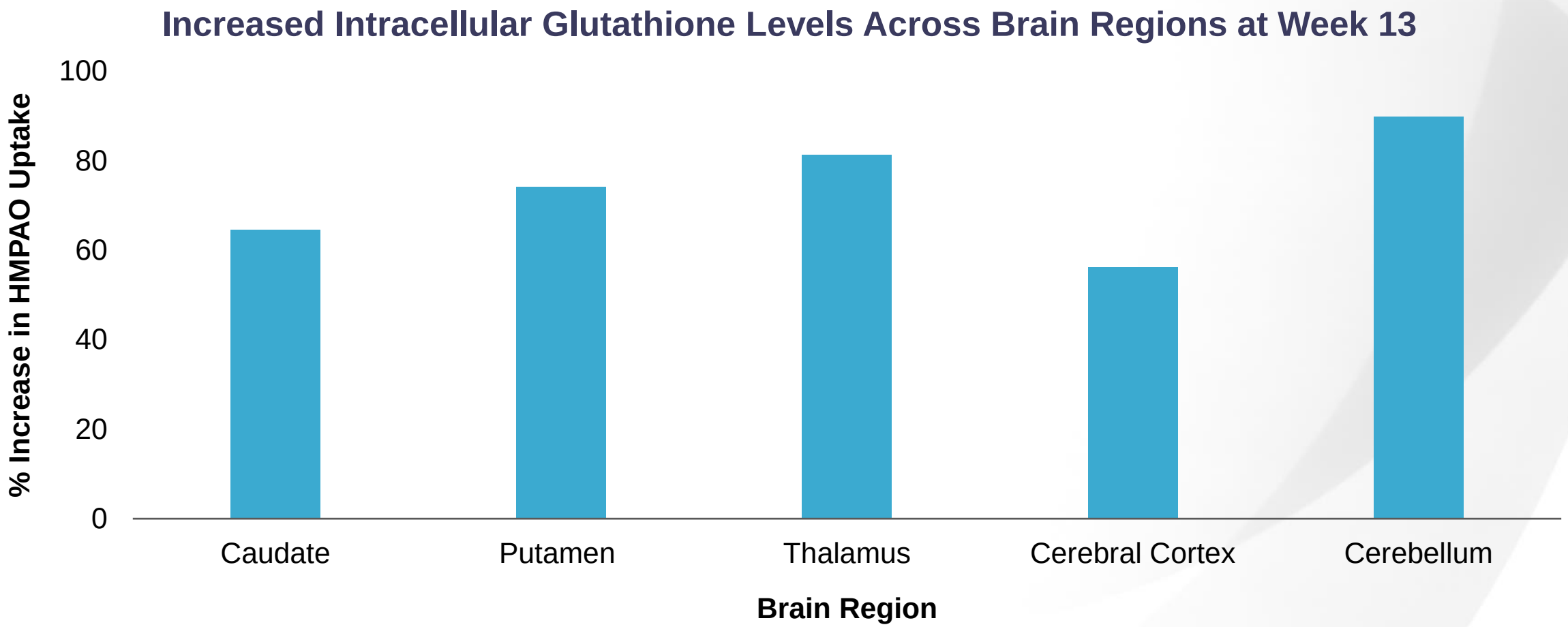
Brain HMPAO-SPECT Measurements



HMPAO

- Technetium-99m-hexamethylpropyleneamineoxime
- FDA-approved commercial reagent
- Binds to intracellular reduced glutathione
- Used as surrogate for blood flow/glutathione

PTC743 Increases Brain Glutathione in Mitochondrial Disease Patients



Key Learnings from Expanded Access Protocol

- Safe and well-tolerated
- Signals of clinical effect across multiple disease subtypes
  - “Common disease pathway”
  - Mortality effect
- CNS biomarker data consistent with clinical effect
- Data sets that could inform subsequent drug development

PTC743: Refractory Mitochondrial Epilepsy

Description:

40-50% of all patients with mitochondrial disease have associated epilepsy which is refractory to traditional anti-epileptic medications

Rationale (Scientific):

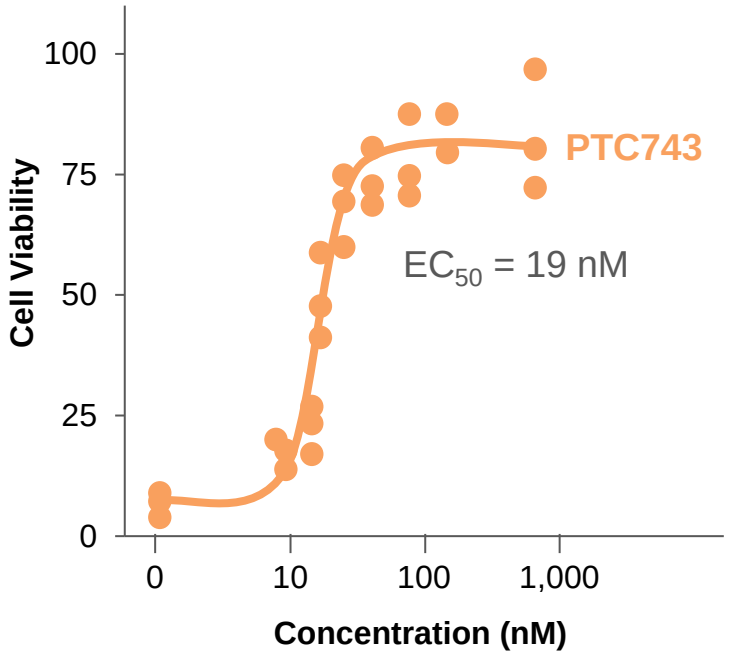
PTC743 target pathway has been linked to refractory epilepsy syndromes in both *in vitro* and *in vivo* models

Rationale (Clinical):

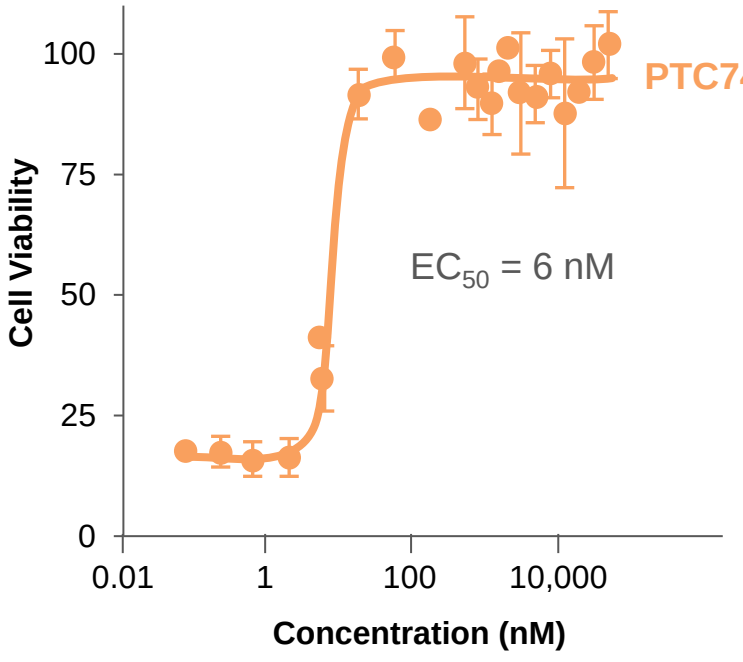
In clinical studies, PTC743 has disrupted refractory status epilepticus, decreased seizure frequency, and decreased seizure-related morbidity in patients with PCH6 and other mitochondrial disease subtypes

PTC743 Rescues Refractory Mitochondrial Epilepsy Patient Fibroblasts from Lipid Oxidation-Mediated Cell Death

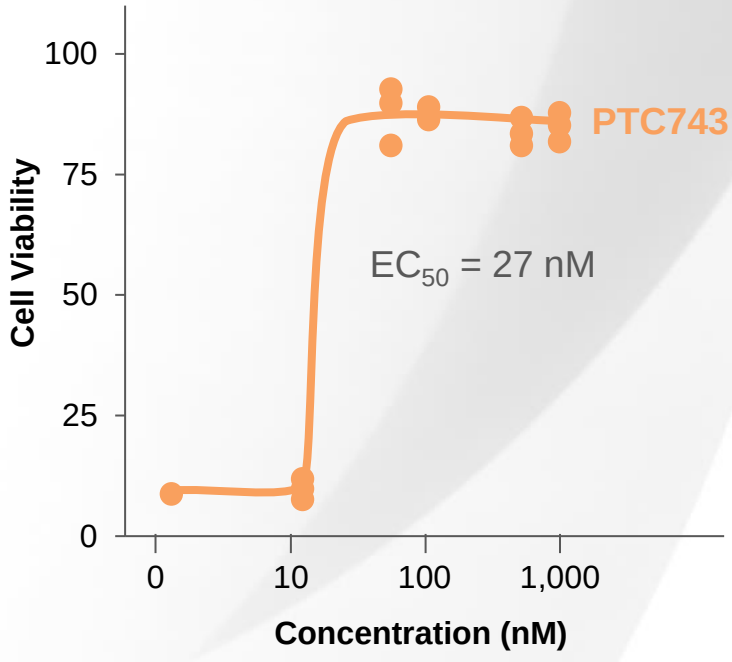
Alpers-Huttenlocker^{POLG}



PCH6



Leigh Syndrome^{SURF1}



PCH6 patient fibroblasts treated with Fe++/BSO to induce glutathione depletion, lipid oxidation and cell death. Cell viability was assessed 24 h after Fe++/BSO treatment using CellTiter-Glo. Mean ± SEM (N=6 replicates per patient culture).

PTC743: Mitochondrial Epilepsy (PCH6 Subtype)

Disease Description

- Pontocerebellar hypoplasia type 6 (PCH6) is an ultra-rare fatal pediatric mitochondrial epilepsy subtype
- Characterized by drug-resistant seizures
- Rapidly progressive with death in early childhood

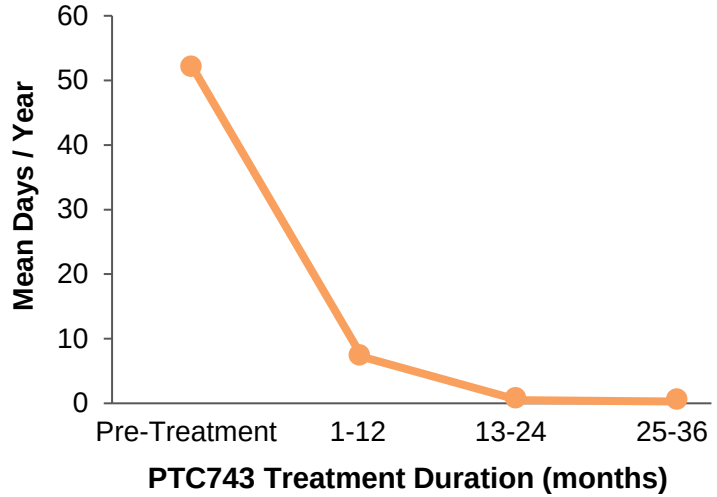
Clinical Study

- Single site, compassionate use study
- N=5 subjects; duration: 36 months

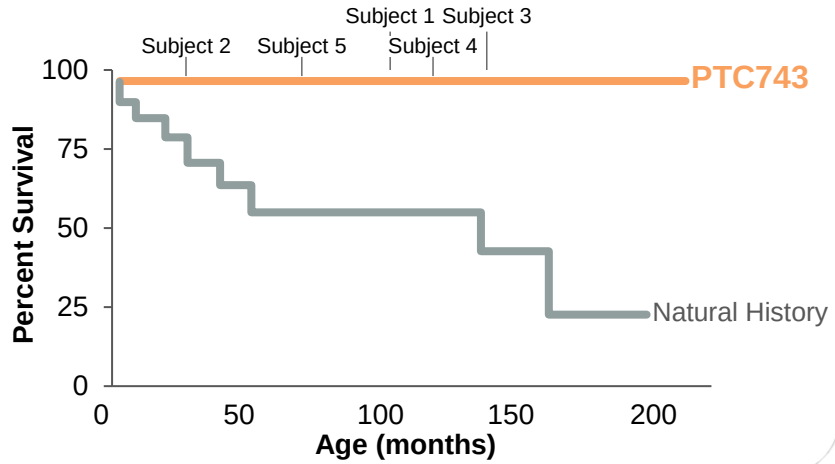
Key Outcomes:

- Reduced seizure frequency
- Reduced status epilepticus
- Reduced hospitalizations
- Reduced mortality risk
- Safe and well-tolerated

Decrease in disease-related hospitalizations



Survival



PTC743 Mitochondrial Epilepsy Next Steps: Phase 2/3 Trial

- Regulatory-approved protocol
- Randomized, placebo-controlled trial
- Target enrollment: 60 subjects
- Duration: 7 months
- Primary endpoint: Reduction in observed motor seizure frequency
- Secondary endpoints: Occurrence of status epilepticus, hospitalizations, rescue medications, caregiver burden
- Planned initiation in Q3 2020

PTC743: Friedreich Ataxia

Description:

Highly morbid, life-shortening neurological and neuromuscular disorder

Rationale (Scientific):

PTC743 target and mechanism-of-action, 15-lipoxygenase and response pathway, have been linked to FA disease pathology in *in vitro*, *in vivo*, and human biomarker studies

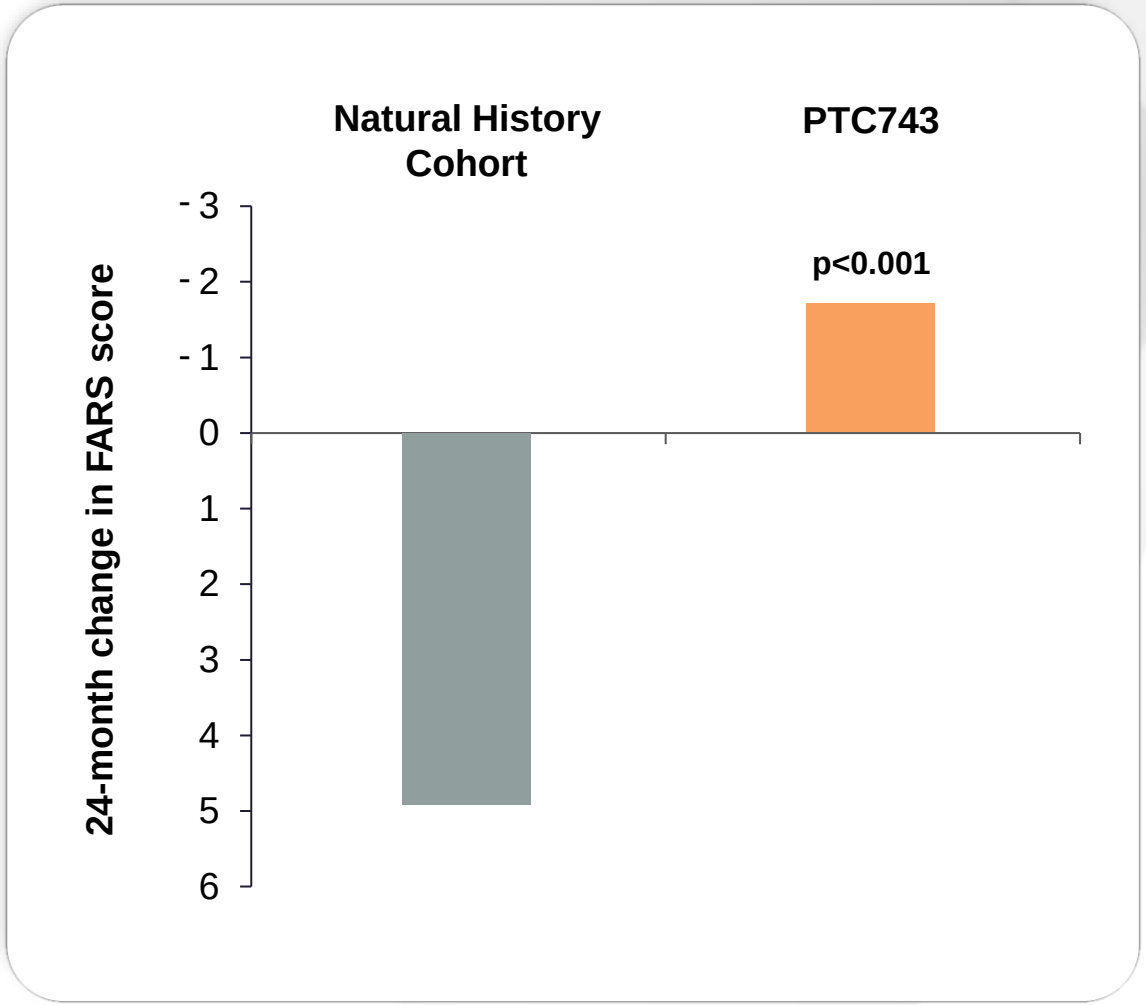
Rationale (Clinical):

In a phase 2 trial of Friedreich ataxia patients, PTC743 treatment was associated with a significant improvement in long-term disease progression relative to an age-, stage- and sex-matched natural history cohort

Phase 2 FA Trial Demonstrated Significant Improvement in Long-term Disease Severity & Neurological Function

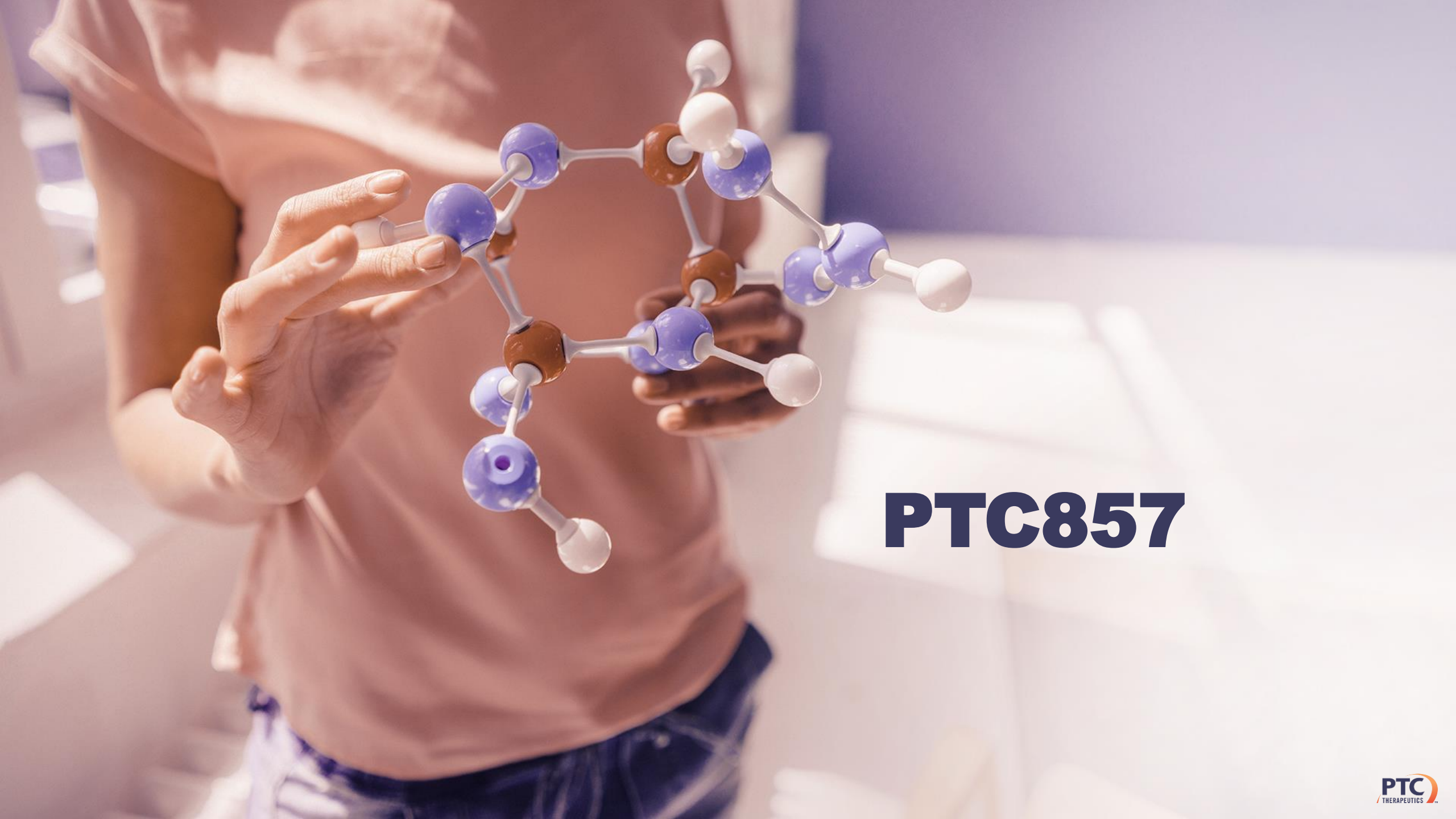
Clinical Study Summary

- Double-blind, placebo-controlled with delayed start
- Three US clinical sites; 63 subjects
- Outcome Measurements, Disease progression, Neuromuscular function, Visual acuity, Safety
- Initial analysis after 6 months; long-term analysis after 24 months
- Note: At 12 months, there was a 3.6 point improvement in the FARS score



PTC743 Friedreich's Ataxia Next Steps: Phase 3 Trial

- Study design - Randomized, placebo-controlled trial
- Target enrollment: ~100 subjects
- Key inclusion criteria: Age 7 to 21; homozygous GAA repeat; ambulatory
- Duration:12 months
- Primary endpoint: Disease progression as assessed by FARS validated disease rating scale
- Secondary endpoints: ADL scale, speech, timed walk test, fatigue scale
- Planned initiation in Q4 2020

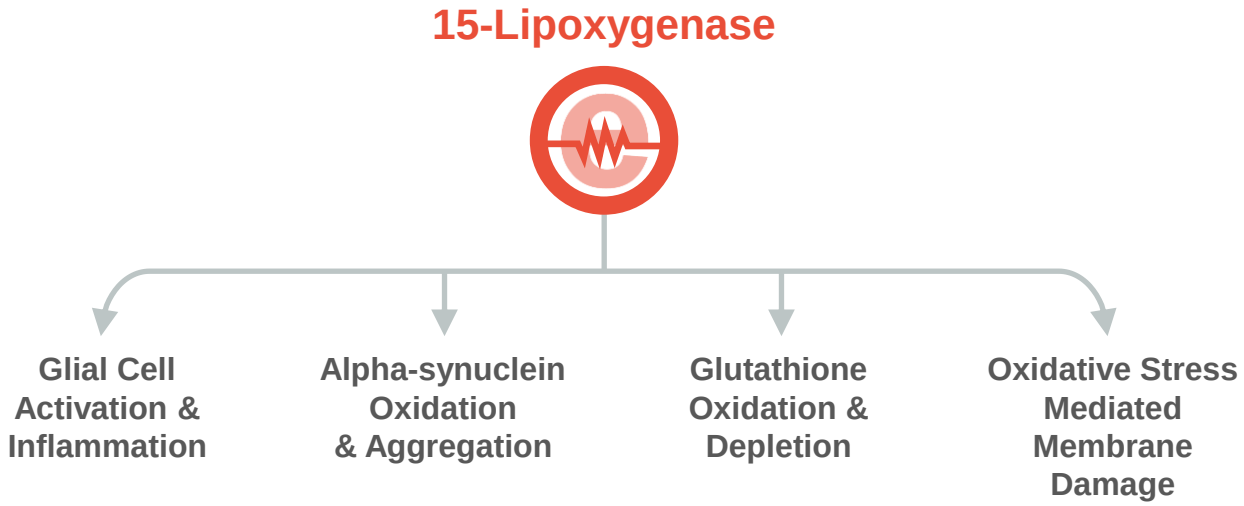


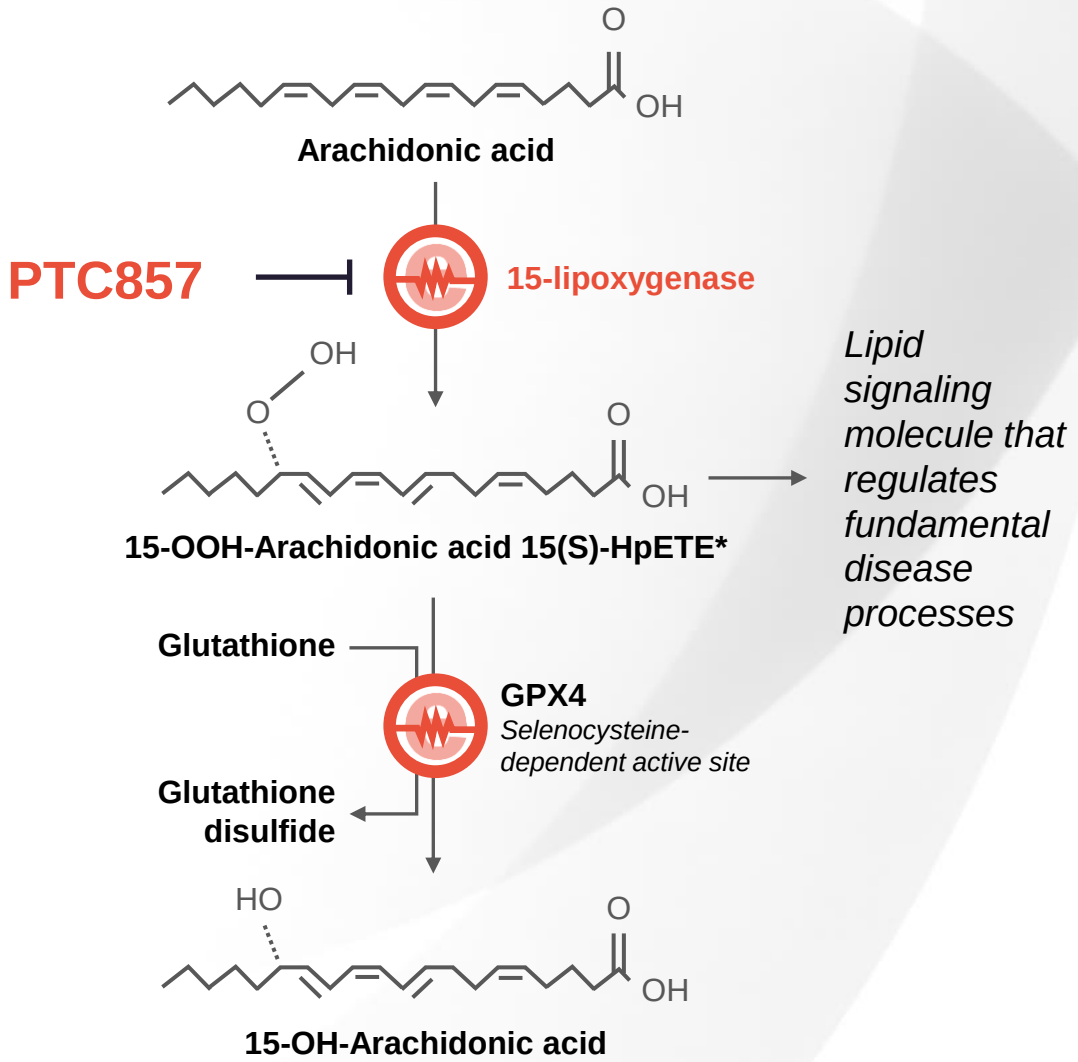
PTC857

PTC857 Overview

- Novel small molecule for neurodegenerative diseases of oxidative stress and inflammation
- Target: 15-Lipoxygenase
- Mechanism of action: Inhibit 15-lipoxygenase to reduce oxidative stress, lipid-based inflammation and cell death
- Initial Indication: Parkinson’s disease (GBA subtype)
- Status: Planned initiation of Phase 1 healthy volunteer in Q3 2020

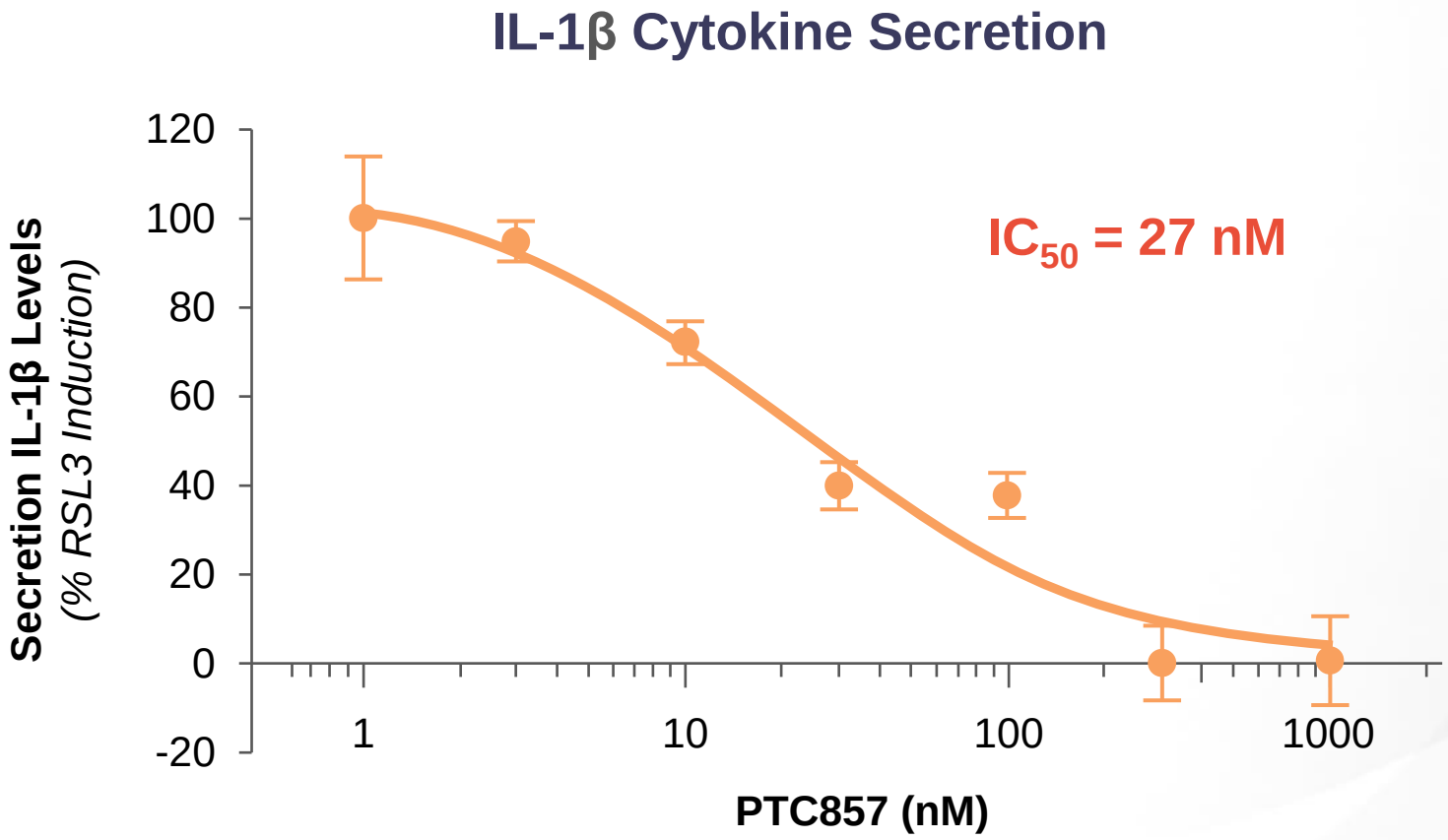
PTC857 2nd Generation 15-LO Inhibitor for Adult CNS Disorders





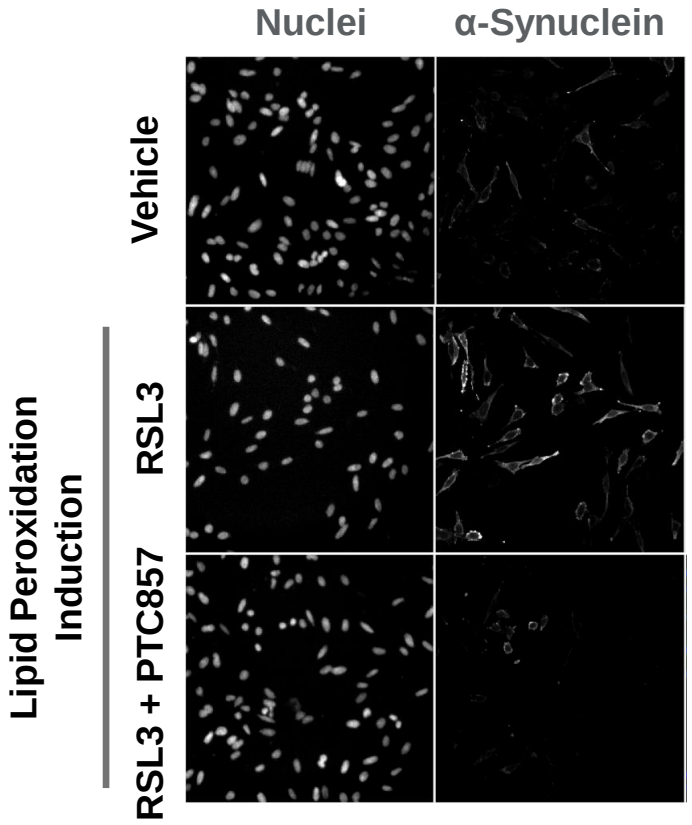
PTC857 *in vitro* Target Validation

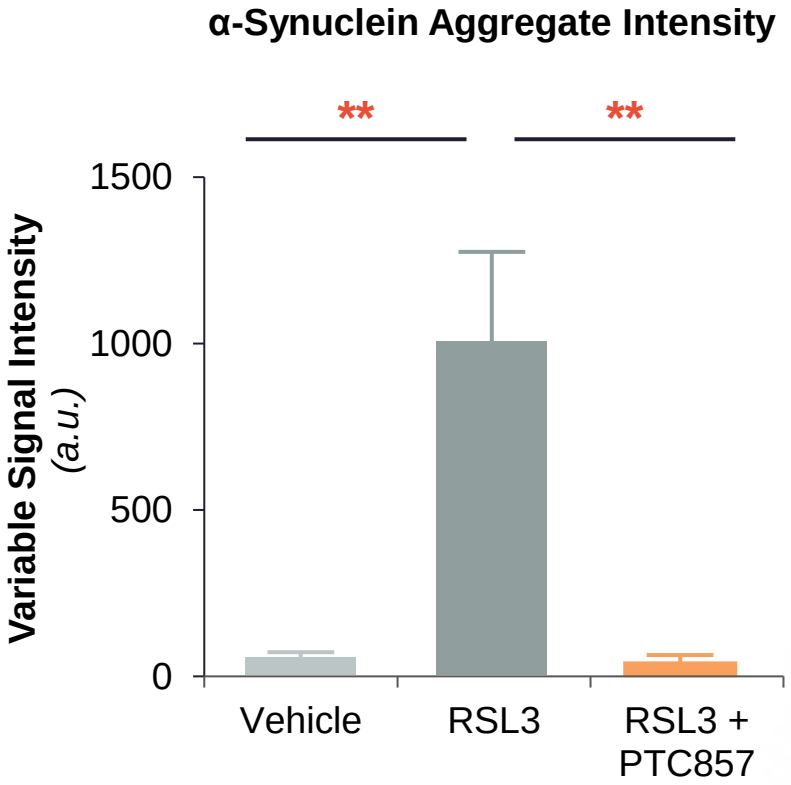
PTC857 dose-dependently reduces pro-inflammatory IL-1β cytokine production by microglial cells



Cytokine release by primary rat microglia exposed to RSL3-challenged Q7 striatal cells +/- PTC857. IL-1β cytokine levels in conditioned media were quantified by MSD ELISA. Mean ± SEM (n=3-6 / condition).

PTC857 Prevents α-synuclein Aggregation (Lewy Body) Associated with Lipid Peroxidation





N27 Cells α-synuclein Assay

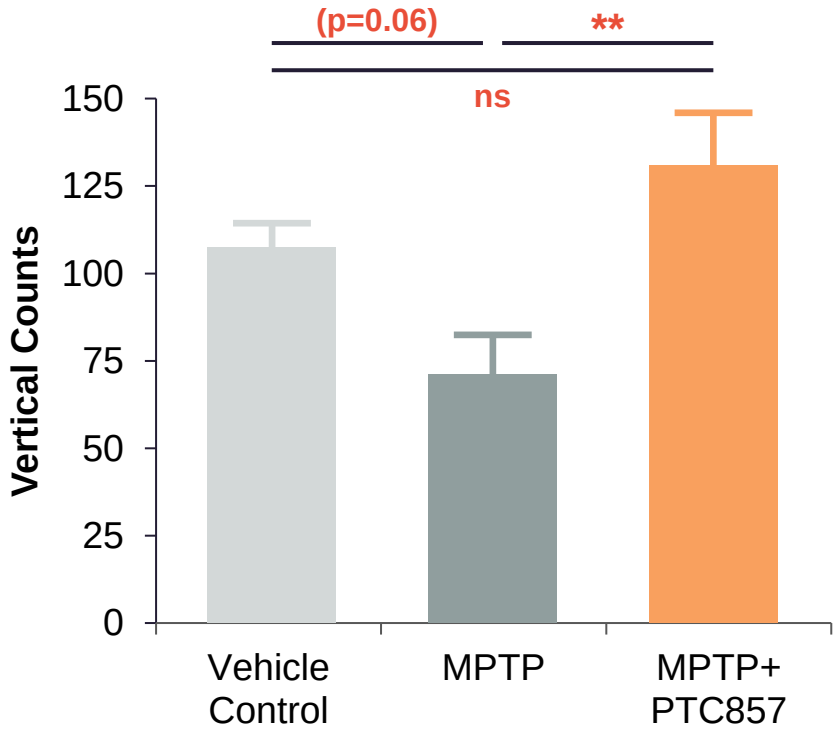
- Lipid peroxidation→ increase in α-synuclein aggregation
- PTC857 potently prevents increase in α-synuclein aggregation (IC_{50} = 28 nM)

N27 stable cell line over-expressing human α-synuclein (A2 colony). Cells were treated with 40nM RSL3 +/- 1µM PTC857. α-Synuclein protein aggregation levels were detected by ICC (1% Triton-X, 48 h) and quantified on the ArrayScan XTI platform. Data represented as mean + sem, n=4-6 wells/condition. One-way ANOVA with post-hoc Tukey's t-test: ** p<0.01.

PTC857 Protects Dopamine-dependent Motor Function in MPTP Mouse Model

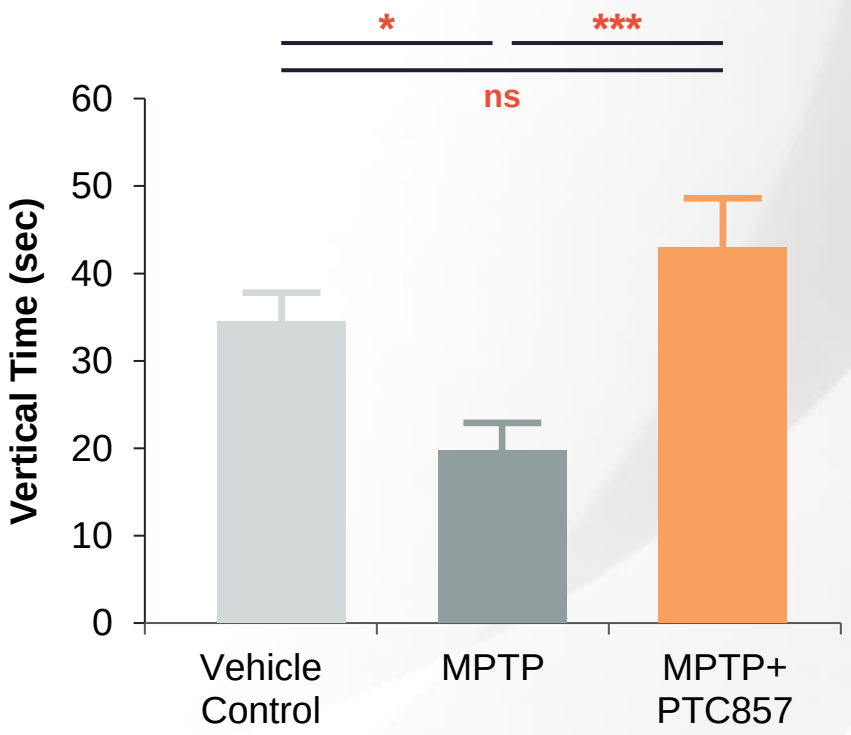
Dopamine-dependent Vertical Counts Open Field Locomotor Assay

(Mean+SEM, N = 12-14 / group)



Dopamine-dependent Vertical Time Open Field Locomotor Assay

(Mean+SEM, N = 12-14 / group)



Male C57Bl/6 mice administered three 20 mg/kg doses of MPTP on day 1. PTC857 dosed orally at 300 mg/kg for seven consecutive days following MPTP dose. Locomotor measurements taken on day 7.

PTC857 Next Steps: Healthy Volunteer Study

- Single ascending and multiple ascending dose studies
- Demonstrate safety and pharmacology
- Inform dosing for Phase 2 study
- Planned initiation in Q3 2020

Bio-e Platform Overview

Platform capabilities

Based on a family of oxidoreductase enzyme targets critical to generation and regulation of energy key to disease pathology

Novel approach

Intersection of electron-transfer chemistry and biology

Validated target and mechanism of action

Lead compound PTC743 targets the enzyme 15-lipoxygenase – a key enzyme hub that regulates inflammation and oxidative stress

Extensive pediatric safety and exposure history

PTC743 has been evaluated in over 500 patients – mostly children – with duration of exposure up to 10 years, and has been safe and well-tolerated

Pipeline potential

Large number of oxidoreductase targets with known biological significance (> 100), and diverse redox small molecule library