PTC 2021

Corporate Deck

Updated Nov 30, 2021

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 release, other than statements of historic fact, are forward-looking statements, including statements with respect to 2021 net product revenue guidance, statements with respect to the 2021 operating expenditure 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 and other matters; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; the timing with respect to orders for PTC's products; 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; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; the enrollment, conduct, and results of ongoing studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any potential regulatory submissions and potential commercialization with respect to Evrysdi; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA 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 is a specific obligation to continued marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra™; the enrollment, conduct and results of PTC's emvododstat clinical trial for COVID-19; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 the lease agreement 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, Evrysdi, Tegsedi, Waylivra or PTC-AADC.

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.

Translating Science to Transform Lives

Significant Execution and Value Creation in 2020

Clinical

- ✓ Initiated two potential registrational trials with vatiquinone in Mitochondrial Disease Associated Seizures & Friedreich ataxia
- ✓ Initiated Phase 1 trial of PTC518 in healthy volunteers for Huntington disease program
- ✓ Initiated one potential registrational trial with emvododstat in COVID-19

Regulatory

- ✓ Evrysdi™ approval in US and multiple additional countries
- ✓ Submitted MAA to EMA for gene therapy to treat AADC deficiency
- ✓ Translarna label modification related to non-ambulatory patients

Commercial

- ✓ Broader patient access and continued geographic growth of Translarna
- ✓ Strong Emflaza growth; 38% YoY
- ✓ Evrysdi strong commercial launch

Financial

- ✓ Strengthened balance sheet; over $1B cash position
- ✓ 2020 Net Revenue: $333M Net Product Revenue; $331M DMD Franchise Net Product Revenue; $42.5M Roche Collaboration Revenue associated with Evrysdi regulatory and sales milestones

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology	Virology
Commercial	Emflaza® (deflazacort) 6 mg | 18 mg | 30 mg | 36 mg tablets 22.75 mg/mL oral suspension	Tegsedi™ (inotersen) waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren	Evrysdi® risdiplam					
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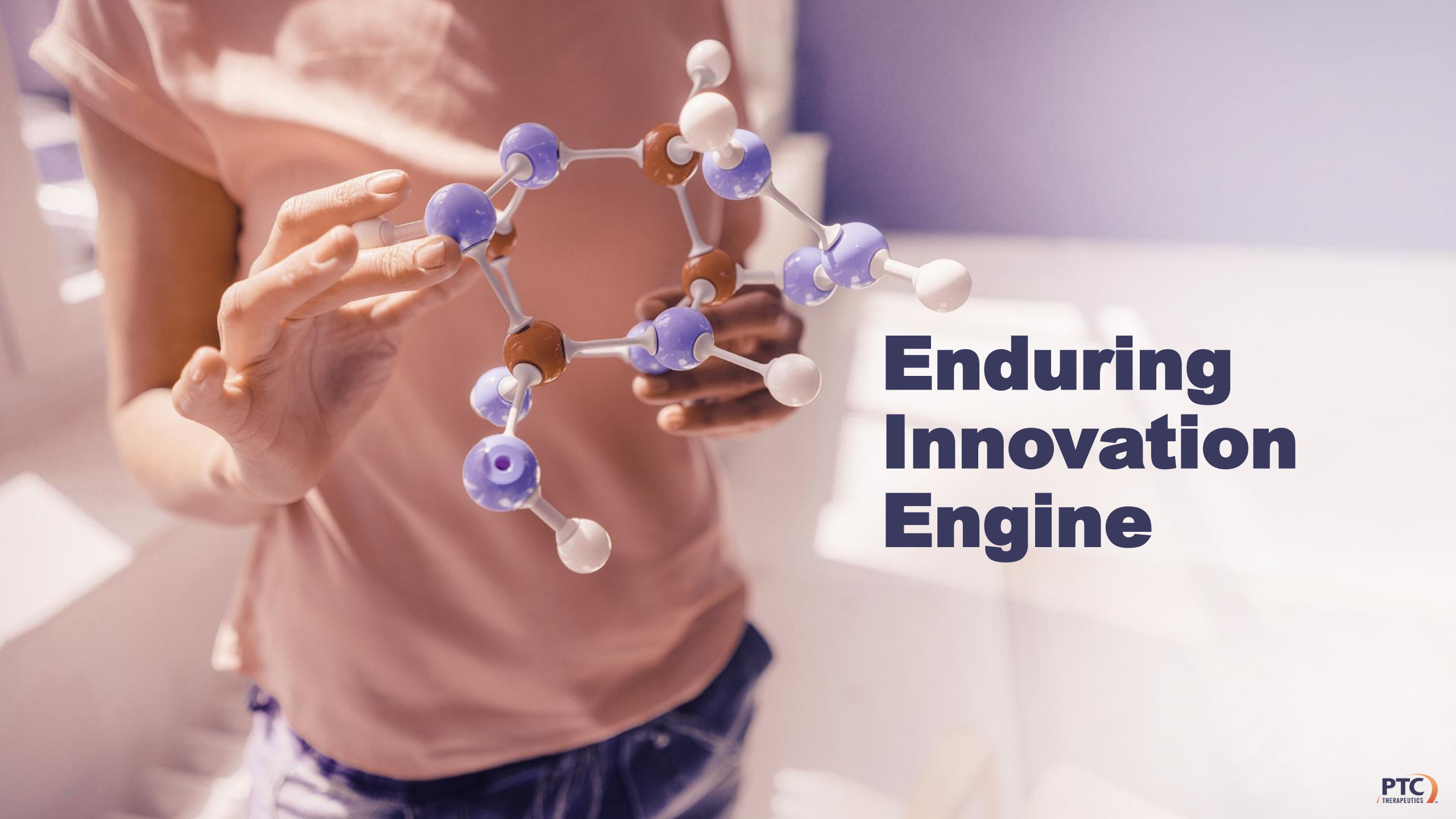
Research	Potential registrational studies			SCA-3 MAP-Tau	FA Angelman IRDs Cog Disorders	Undisclosed			

- AADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich's ataxia; GBA, glucocerebrosidase; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; ME, Mitochondrial Epilepsy; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.



Enduring Innovation Engine

Diversified Platform Drives Strong Portfolio

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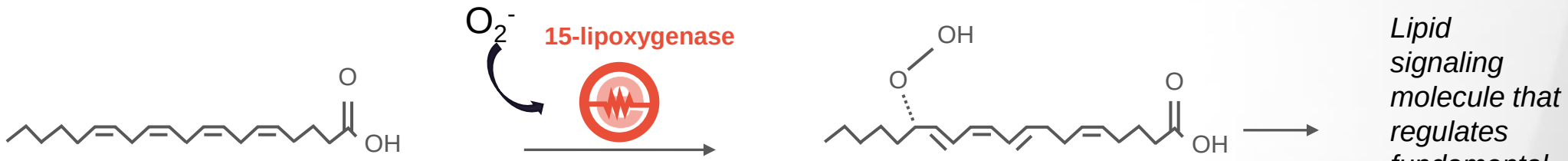
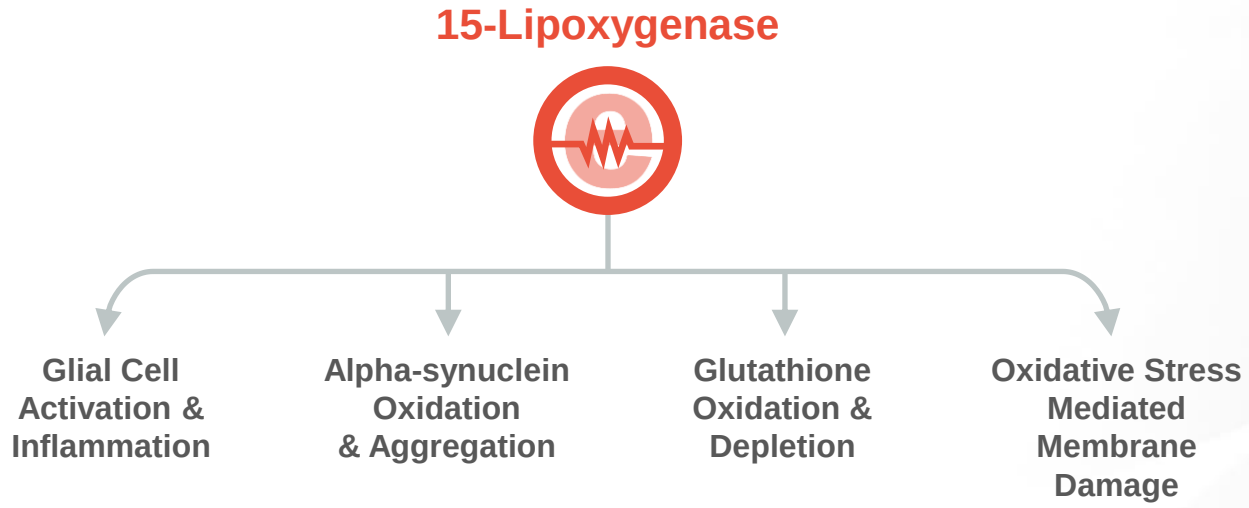
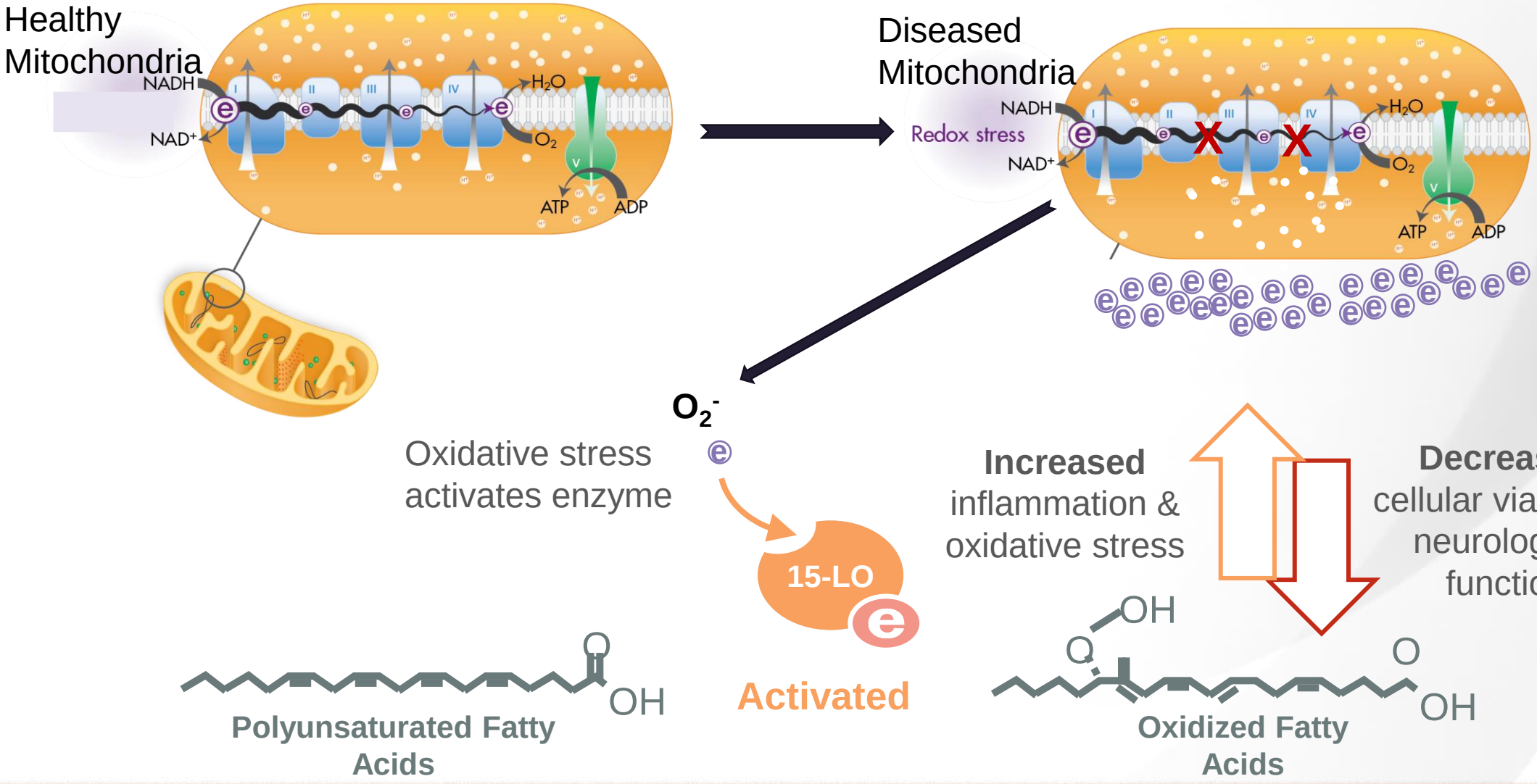
15-Lipoxygenase is a Key Regulator of Inflammation and Oxidative Stress Pathways in CNS Diseases



Lipid signaling molecule that regulates fundamental disease processes

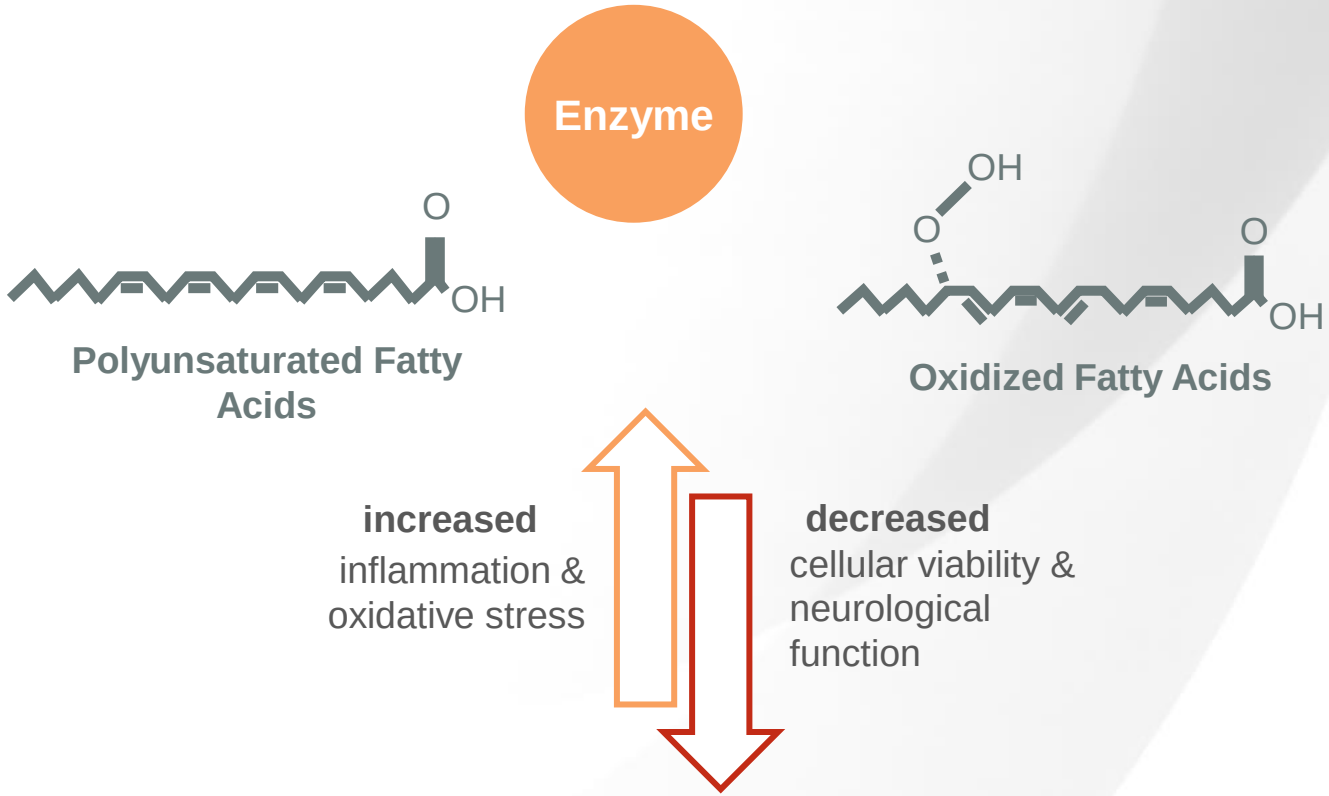


15-Lipoxygenase is a Key Regulator of Inflammation and Oxidative Stress Pathways in Refractory Epilepsies



Bio-e Platform is a Novel Approach to Treating Rare Disorders

- Bio-e platform targets a family of oxidoreductase enzymes critical to generation and regulation of energy key to disease pathology
- Dysregulation of this pathway results in several CNS disease pathologies including epilepsy
- 15-lipoxygenase is a well-known regulator key to CNS and other diseases



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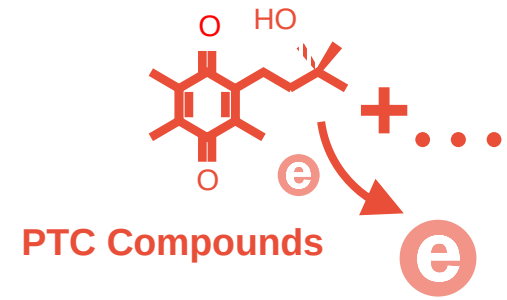
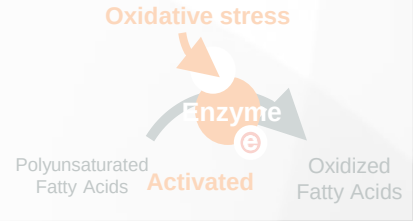
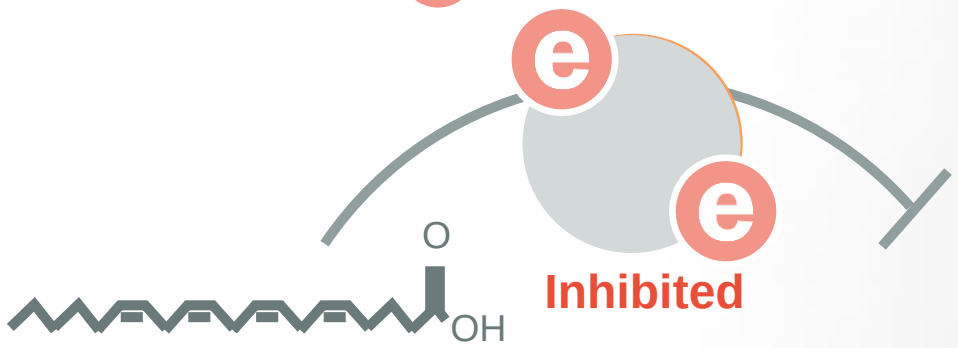






decreased inflammation & oxidative stress

increased cellular viability & neurological function

MIT-E: Registrational trial of vatiquinone for **Mitochondrial Disease Associated Seizures**

Mitochondrial Disease Associated Seizures is a Highly Morbid Disorder with High Unmet Medical Need

Disease

- Mitochondrial disease associated seizures is the highly morbid condition of refractory seizures in patients with inherited mitochondrial disease

Current Treatments

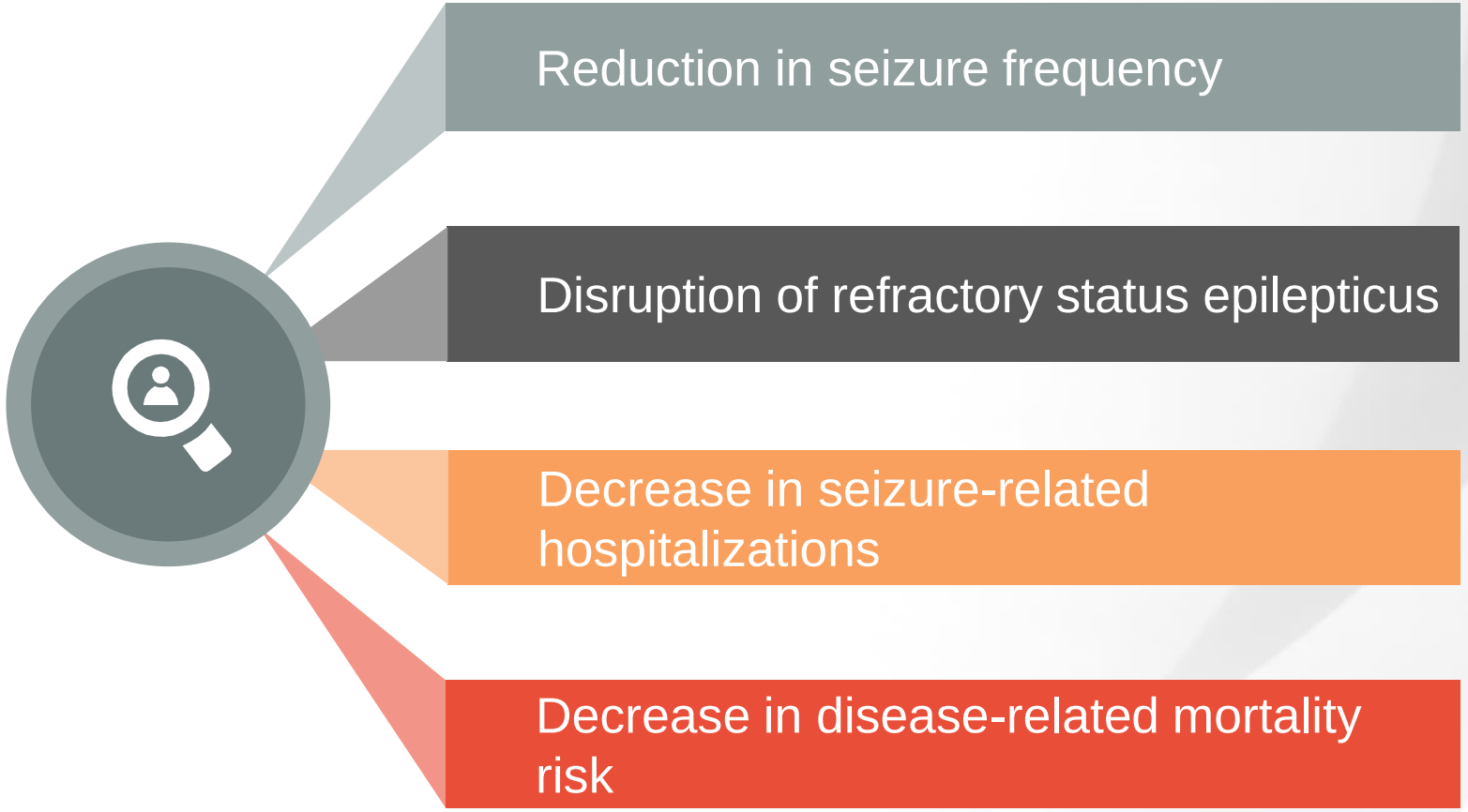
- No approved disease modifying treatments for mitochondrial disease associated seizures

Opportunity

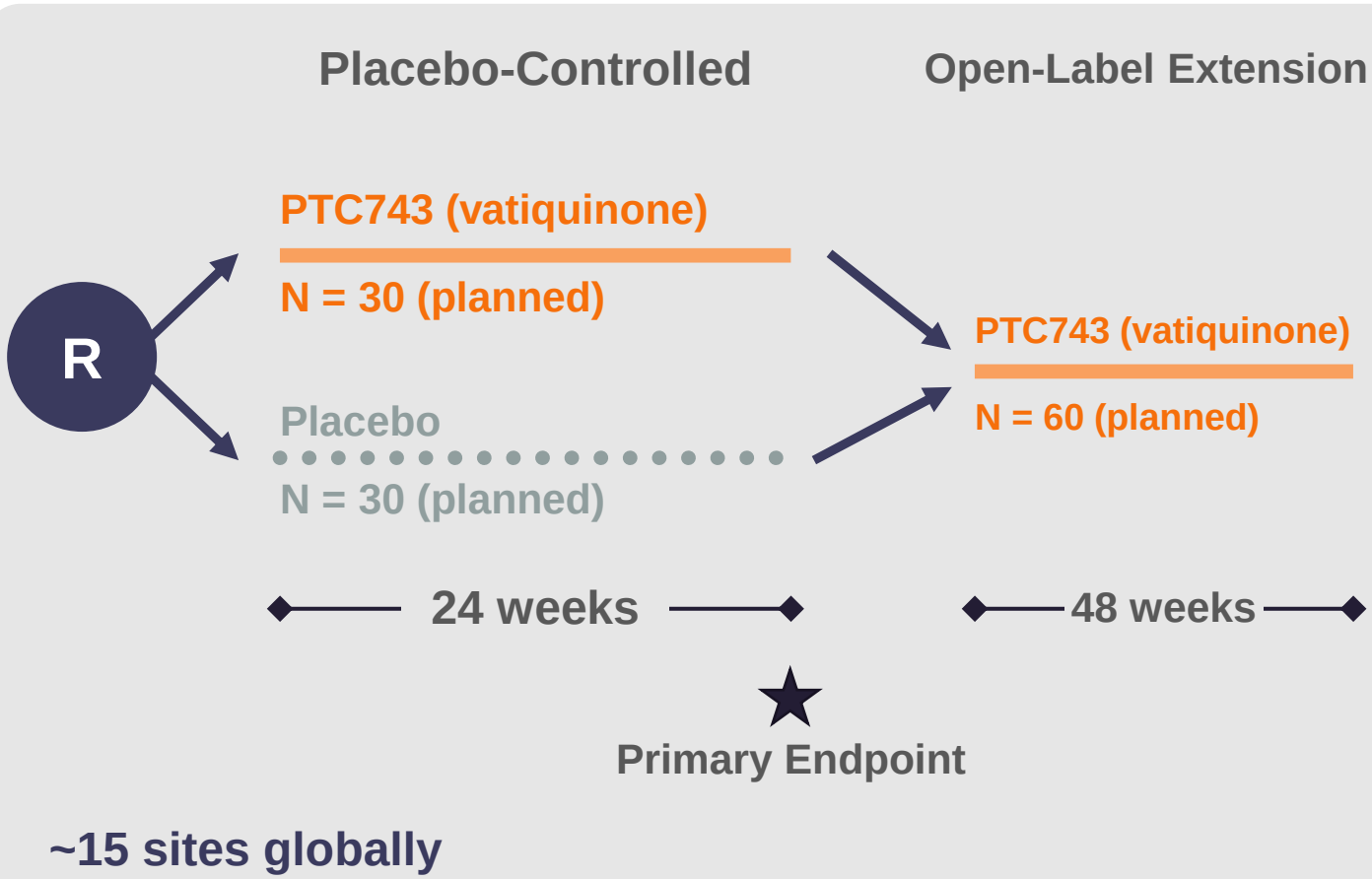
- Vatiquinone targets the energetic and oxidative stress pathways that underpin seizures in these patients

Vatiquinone Reduced Seizure Frequency and Improved Neurological Function in Mitochondrial Disease Associated Seizures Patients

Data from previous studies demonstrate a positive effect on seizures and seizure related-morbidity across multiple disease subtypes



Vatiquinone has Potential to Show Clinically Differentiated Improvement for Mitochondrial Disease Associated Seizures Patients



Primary Endpoint

Change from baseline in frequency of observable motor seizures

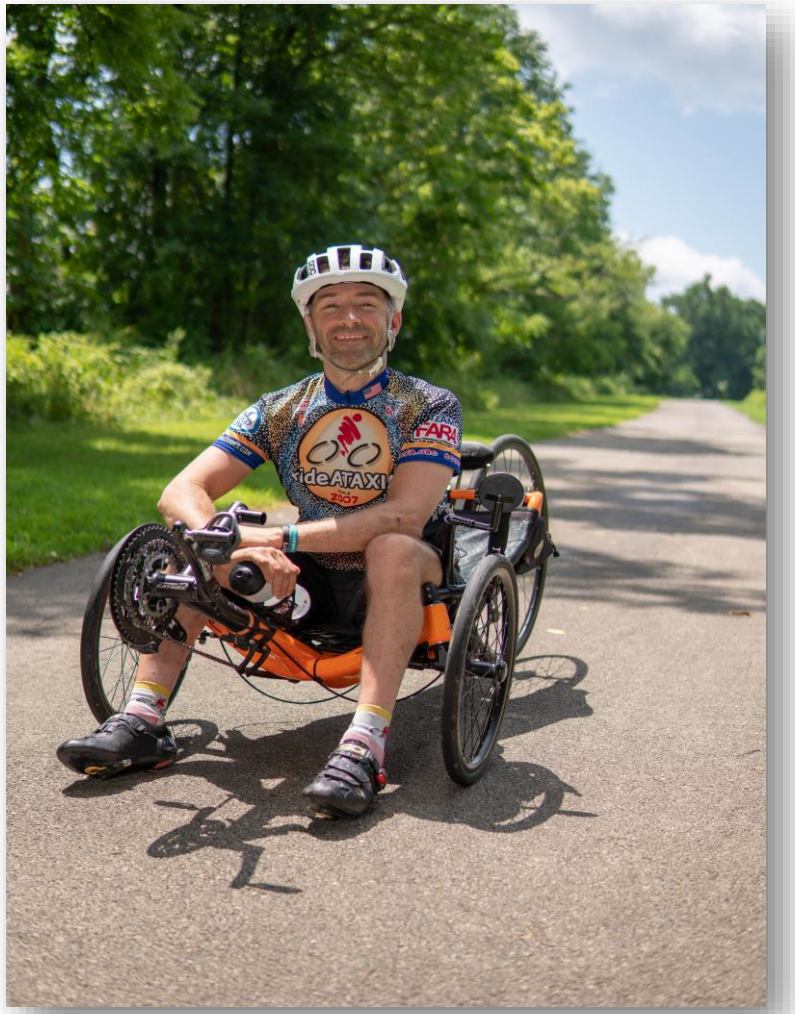
Trial Status

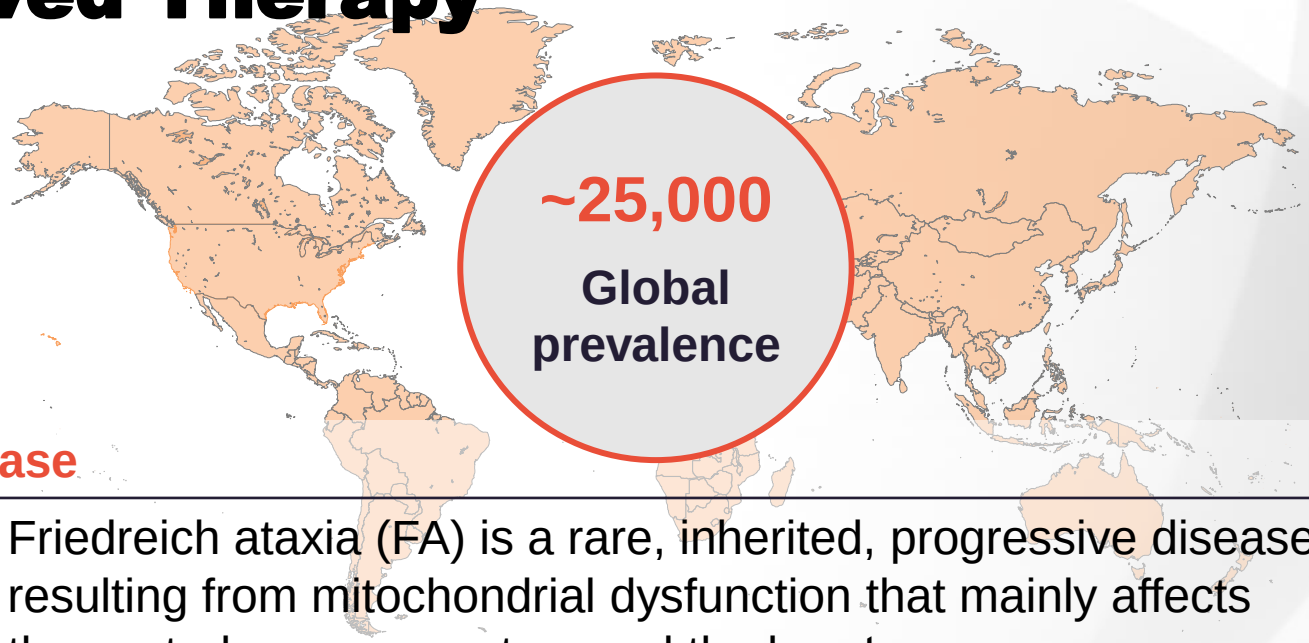
- Enrolling
- Data expected 3Q 2022

MOVE-FA

MOVE-FA: Registrational trial of vatiquinone for **Friedreich Ataxia**

Friedreich Ataxia is a Highly Morbid, Neuromuscular Disorder with no Approved Therapy





Disease

- Friedreich ataxia (FA) is a rare, inherited, progressive disease resulting from mitochondrial dysfunction that mainly affects the central nervous system and the heart

Current Treatments

- No approved disease modifying therapies

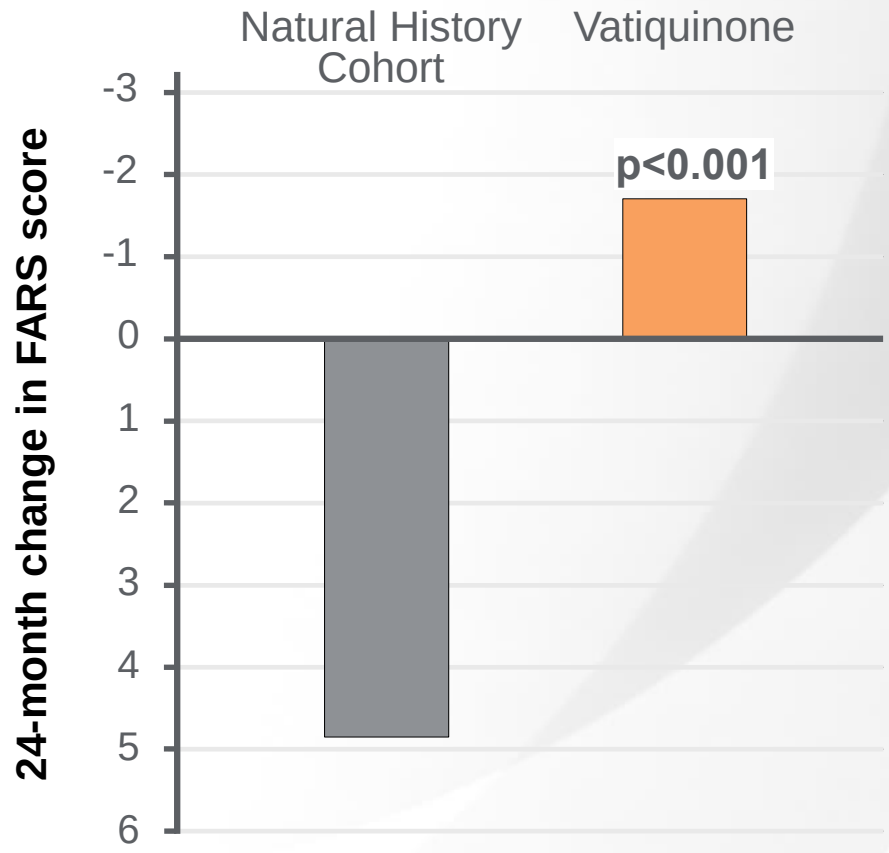
Opportunity

- Vatiquinone is a potent protector of oxidative stress-mediated cell death in FA patients

Vatiquinone Demonstrated Significant Improvement in Long-term Disease Severity & Neurological Function in Friedreich Ataxia Patients

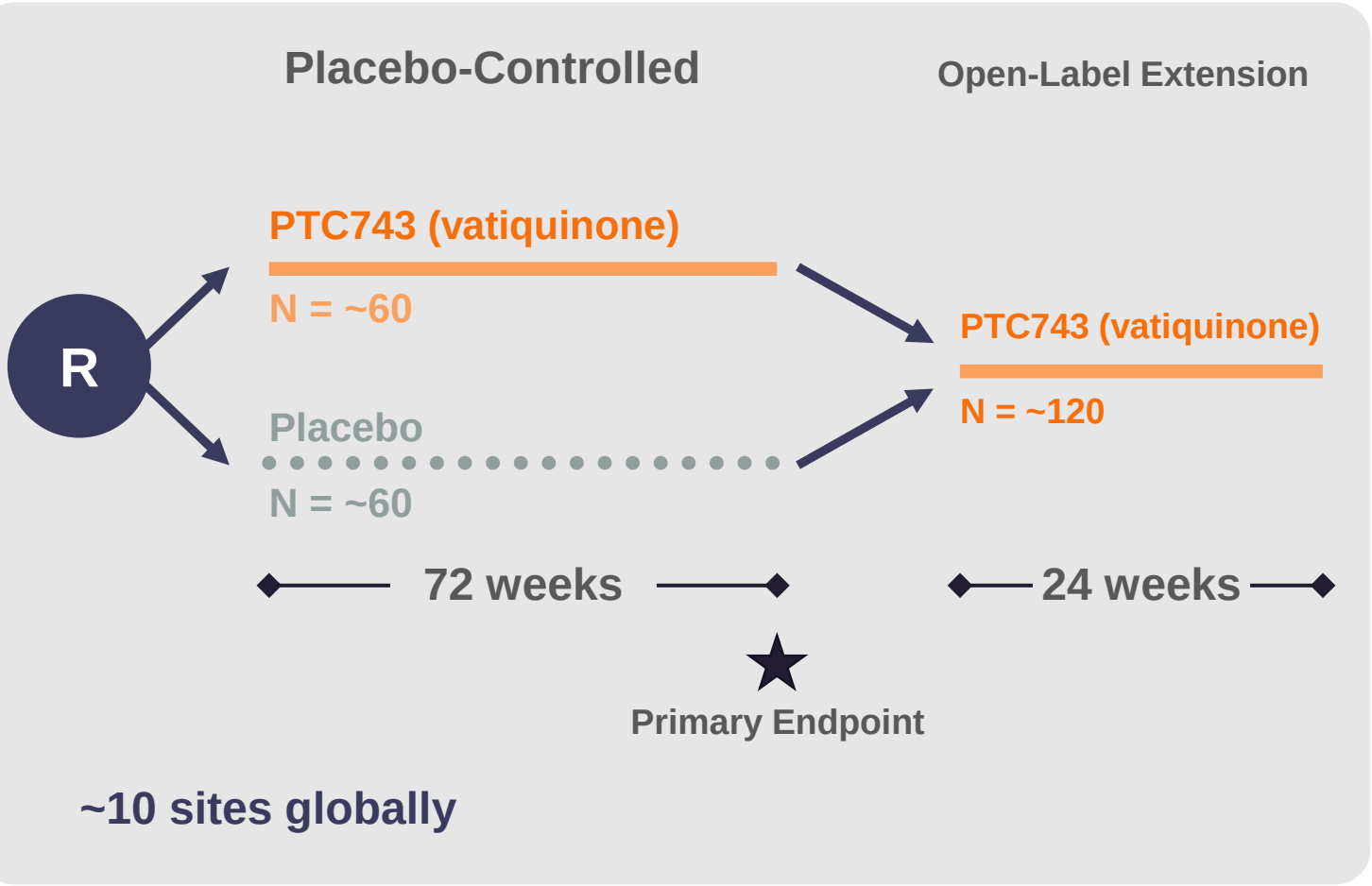
Clinical Study Summary

- Double-blind, placebo-controlled with delayed start
- N=63 subjects
- Three US clinical sites
- Key endpoint: FA disease rating scale (FARS)



Vatiquinone has the Potential to Provide Improvement in Neurological Function

MOVE-FA



Primary and Key Secondary Endpoints

Change from baseline in the Modified FA Rating Scale (mFARS) Score at Week 72

Improvement in activities of daily living (FA-ADL)

Trial Status

- Enrolling
- Data expected in 2023

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

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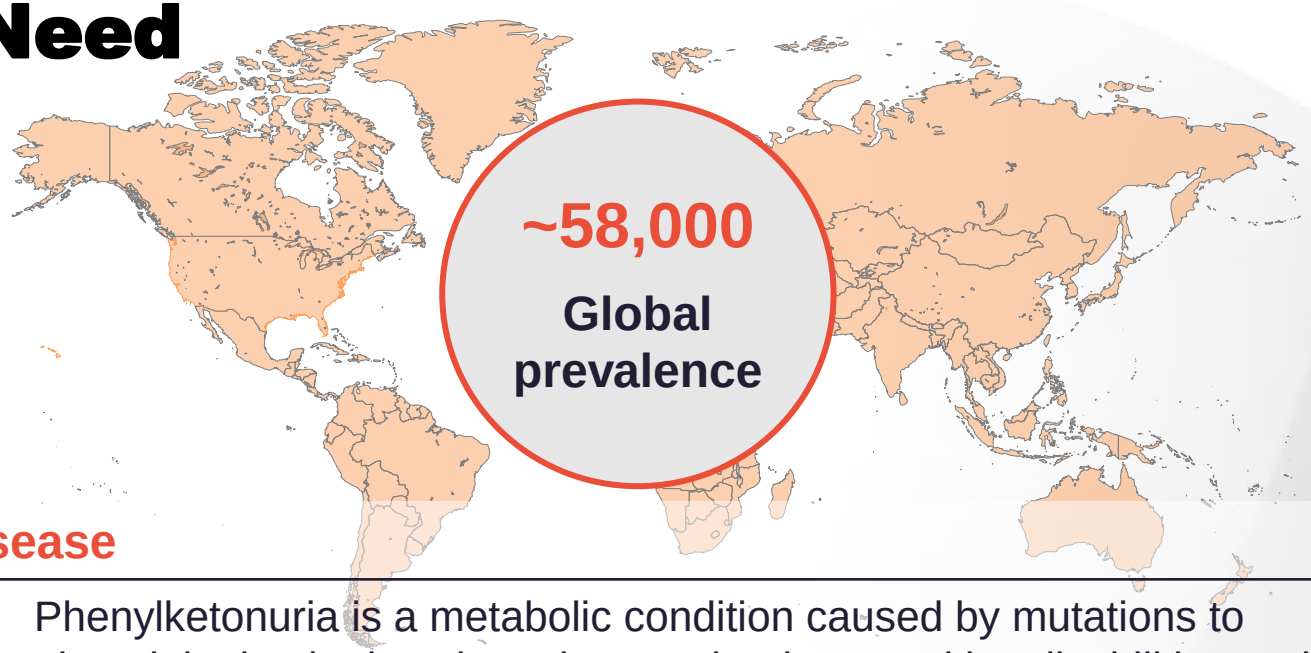
APHENITY: Registrational trial of PTC923 for **Phenylketonuria (PKU)**

Phenylketonuria is a Serious Metabolic Condition with High Unmet Medical Need



Disease

- Phenylketonuria is a metabolic condition caused by mutations to phenylalanine hydroxylase that can lead to cognitive disabilities and seizures

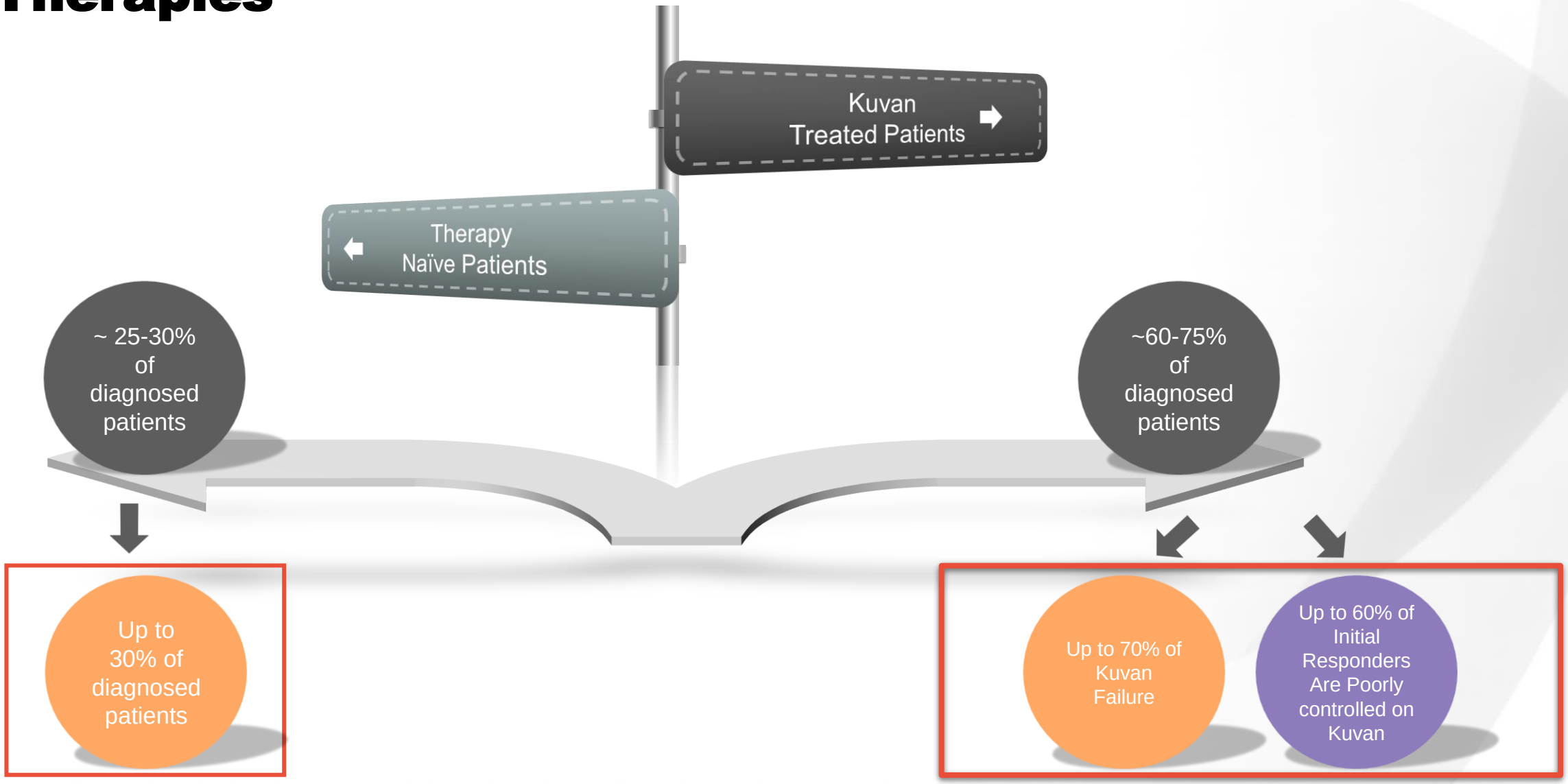
Current Treatments

- Majority of patients do not initially respond or are not well controlled by standard of care

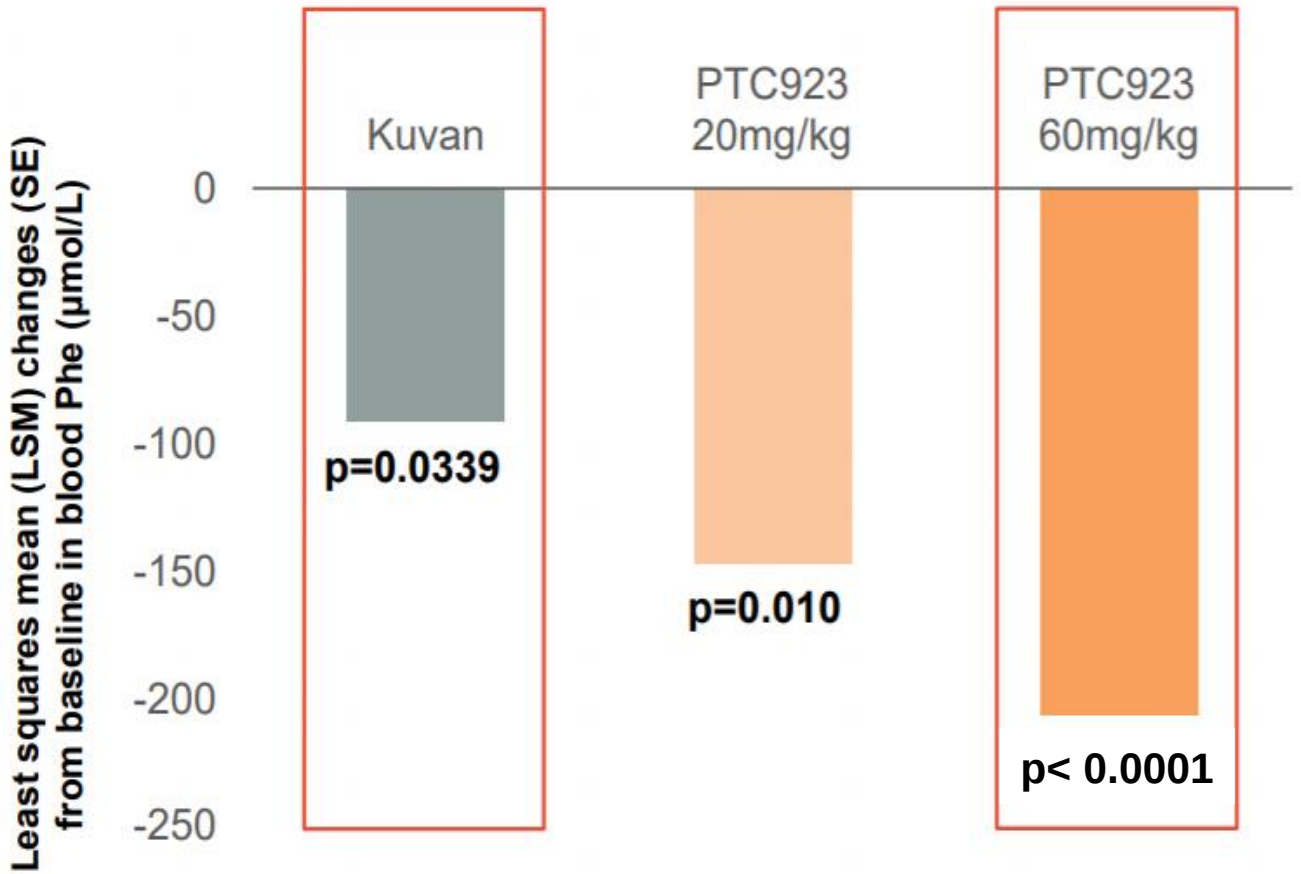
Opportunity

- PTC923 can potentially treat a broad population and is more effective than exogenously administered synthetic BH4 in increasing the intracellular levels of natural tetrahydrobiopterin

Majority of PKU Patients not Addressed by Current Therapies

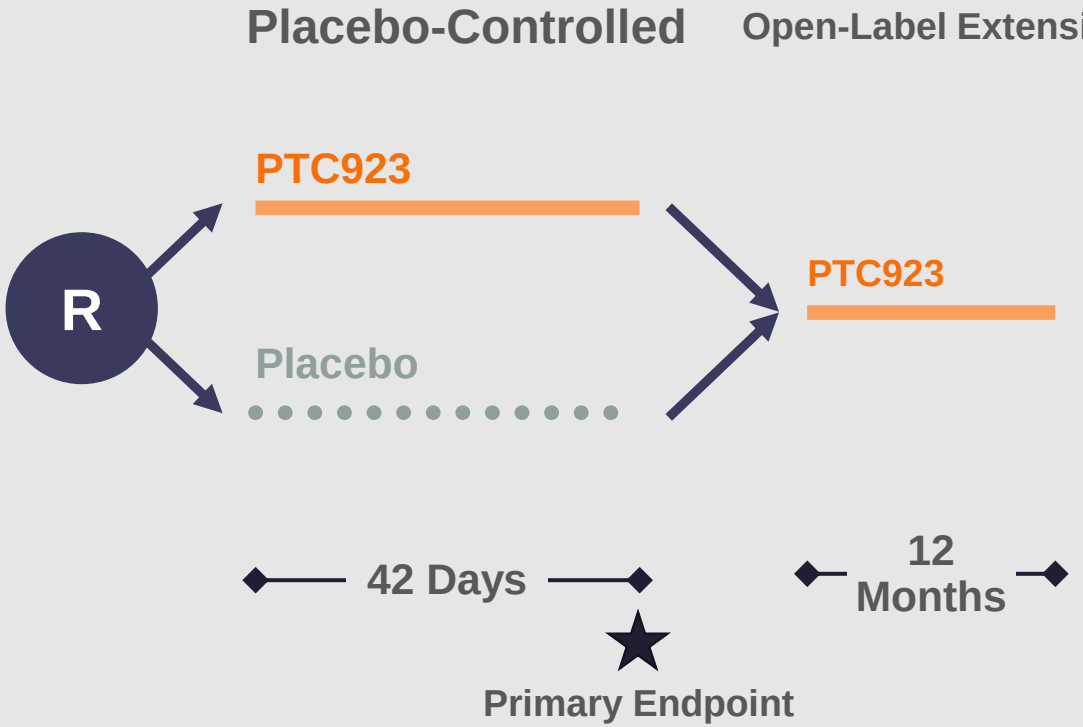


PTC923 Demonstrated Statistically Significant Differences in Reduction of Phenylalanine (Phe) Compared to Kuvan in Phase 2 Study



- 60 mg/kg/day most effective dose
- 114.9 greater µmol/L reduction of Phe with 60 mg/kg/day PTC923 relative to Kuvan; p=0.0098
- 50% increased responder rate with PTC923 as compared to Kuvan (12/19 vs. 8/19)

APHENITY is a Global Registrational Trial of PTC923 for Phenylketonuria



~12 sites in 15-19 countries

Primary Endpoint

Reduction in blood of phenylalanine levels

Trial Status

- Initiated in 3Q 2021
- Data expected YE 2022

Diversified Platform Drives Strong Portfolio

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Potential registrational studies

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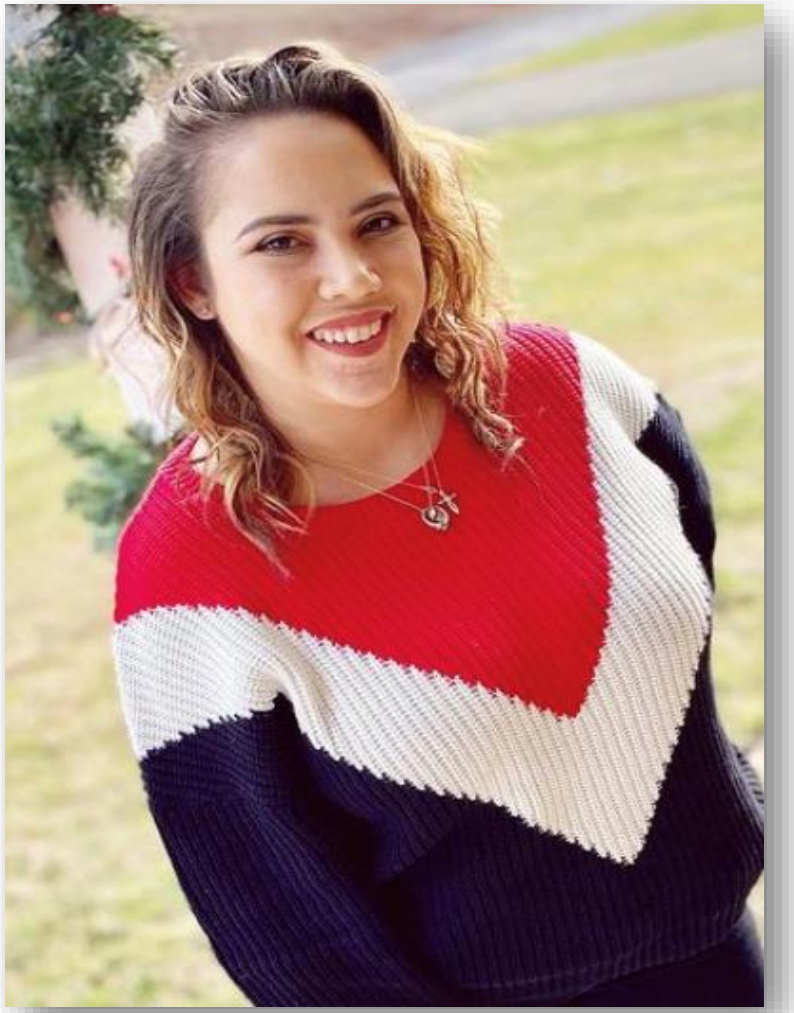
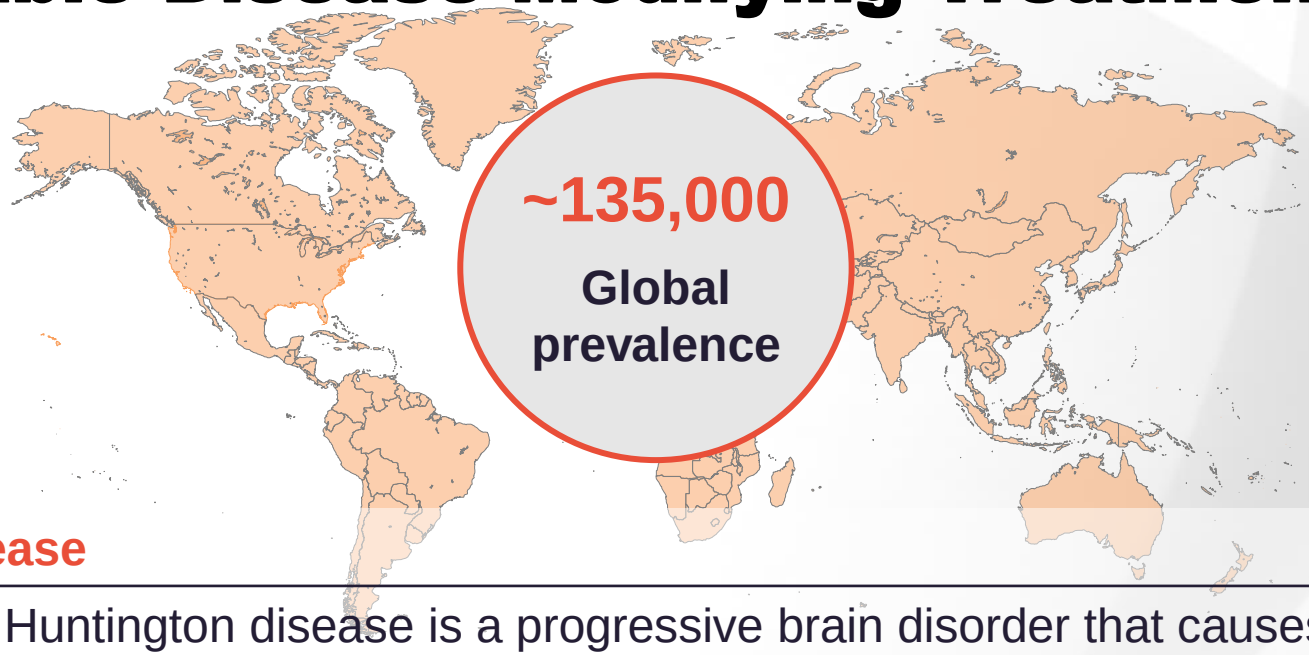
PTC518: Program for **Huntington Disease**

Huntington Disease is a Debilitating Neurological Disorder with No Available Disease Modifying Treatments





Disease

- Huntington disease is a progressive brain disorder that causes uncontrolled movements and cognitive loss

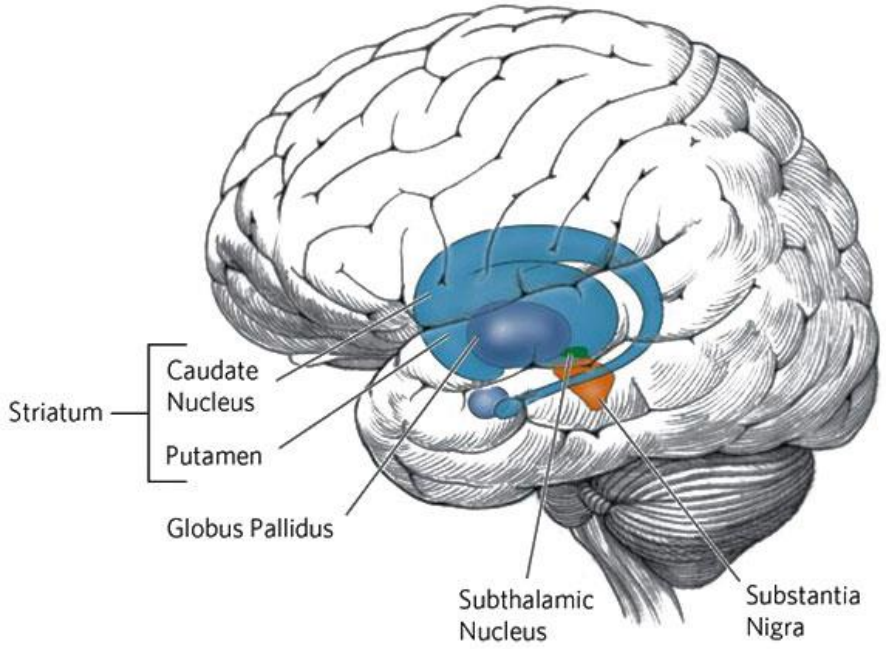
Current Treatments

- No approved disease modifying therapies

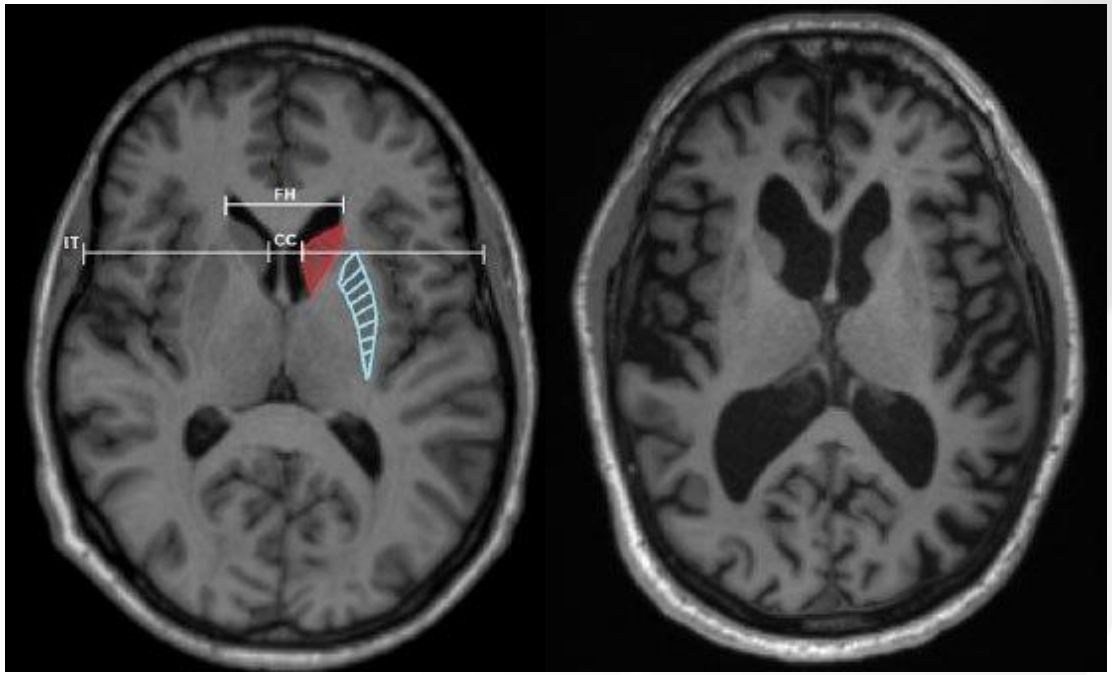
Opportunity

- PTC518 reduces HTT protein in Huntington disease

Small Molecules Have a Critical Advantage for Pan Brain Distribution

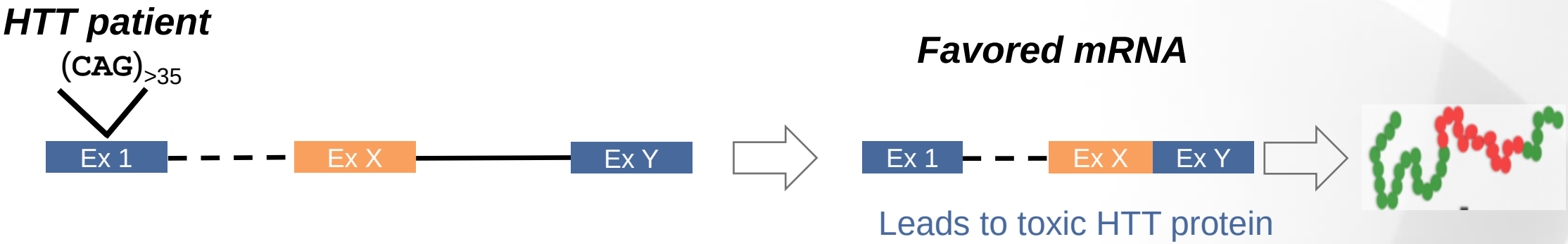


Healthy **HD**



Goh et al. *Aus Psychiatry*. 2018

Identification of a Novel Approach to Lower HTT



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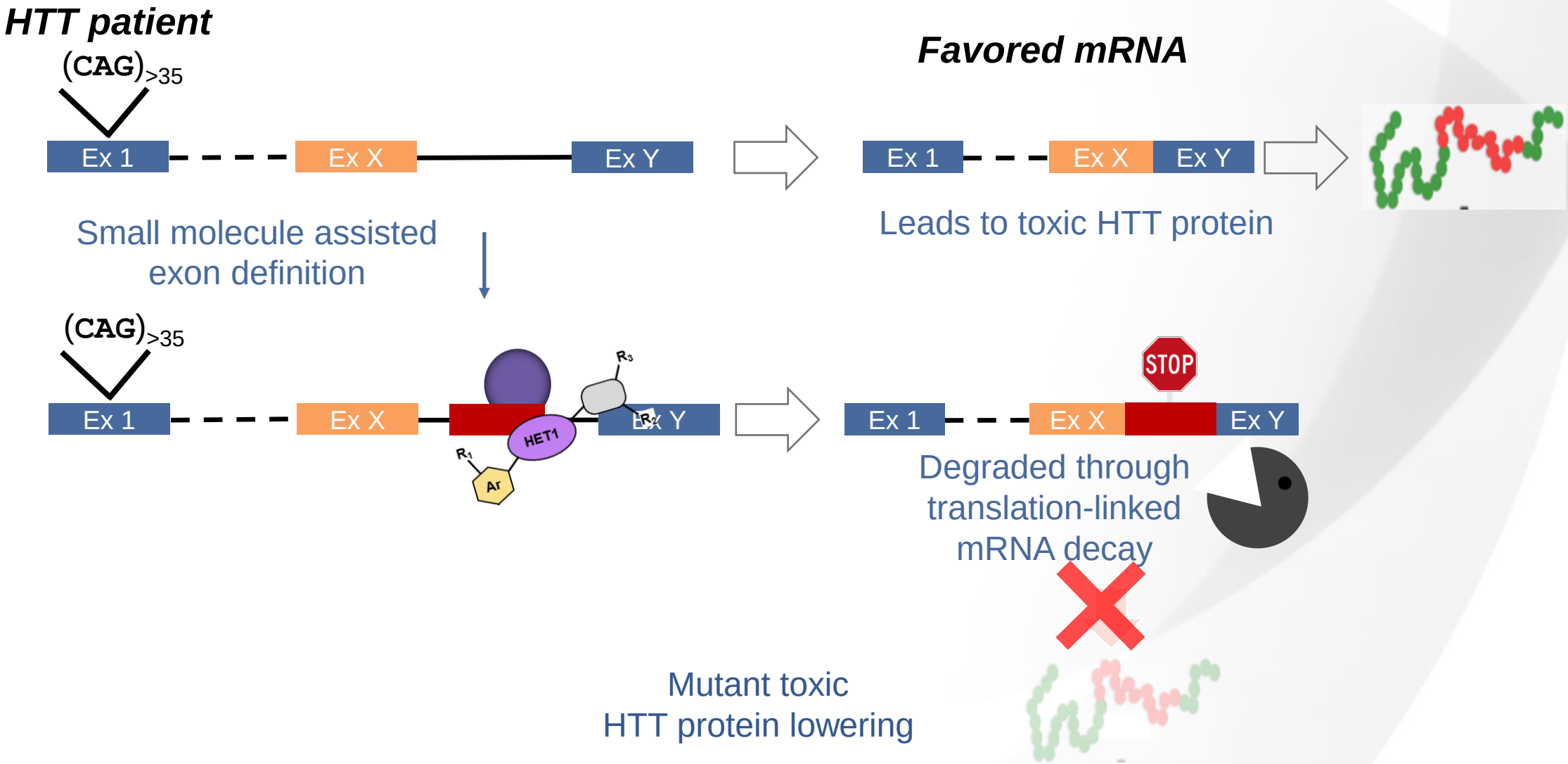
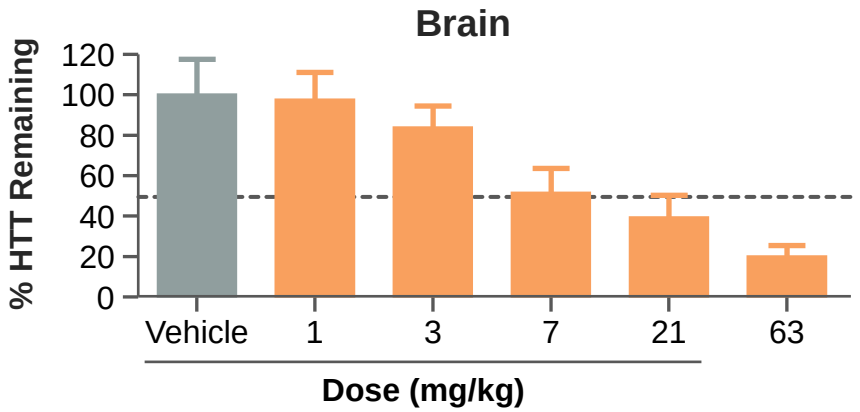
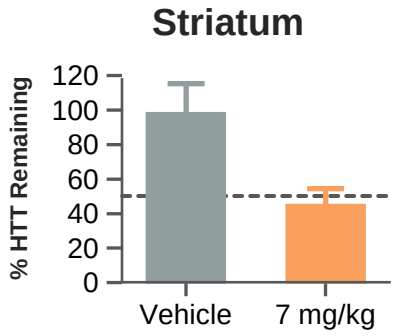


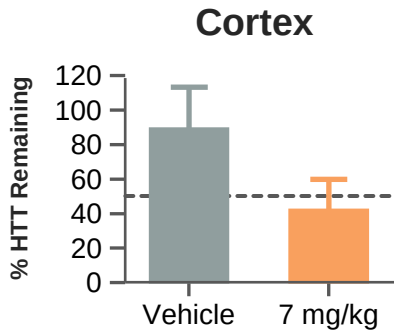
PTC518 Has Broad Tissue Distribution with Strong Correlation between Brain and Blood

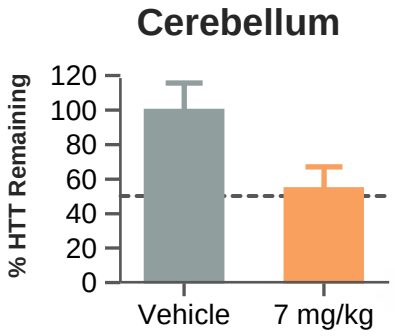
Dose dependent HTT lowering in the brain in BACHD mice

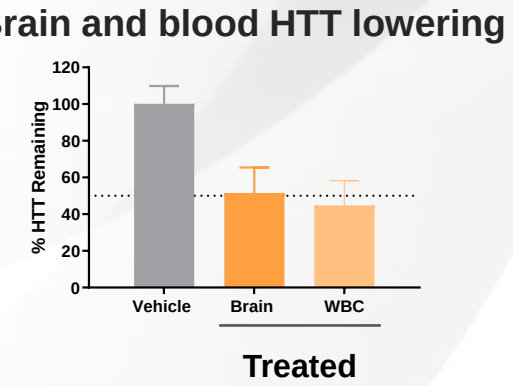


Measurements demonstrate uniform HTT lowering across brain regions with ~1:1 brain and blood concentrations*









*Data on file from multiple studies

The Phase 1 Trial is a 4-Part Study

Phase 1 trial in healthy volunteers is ongoing

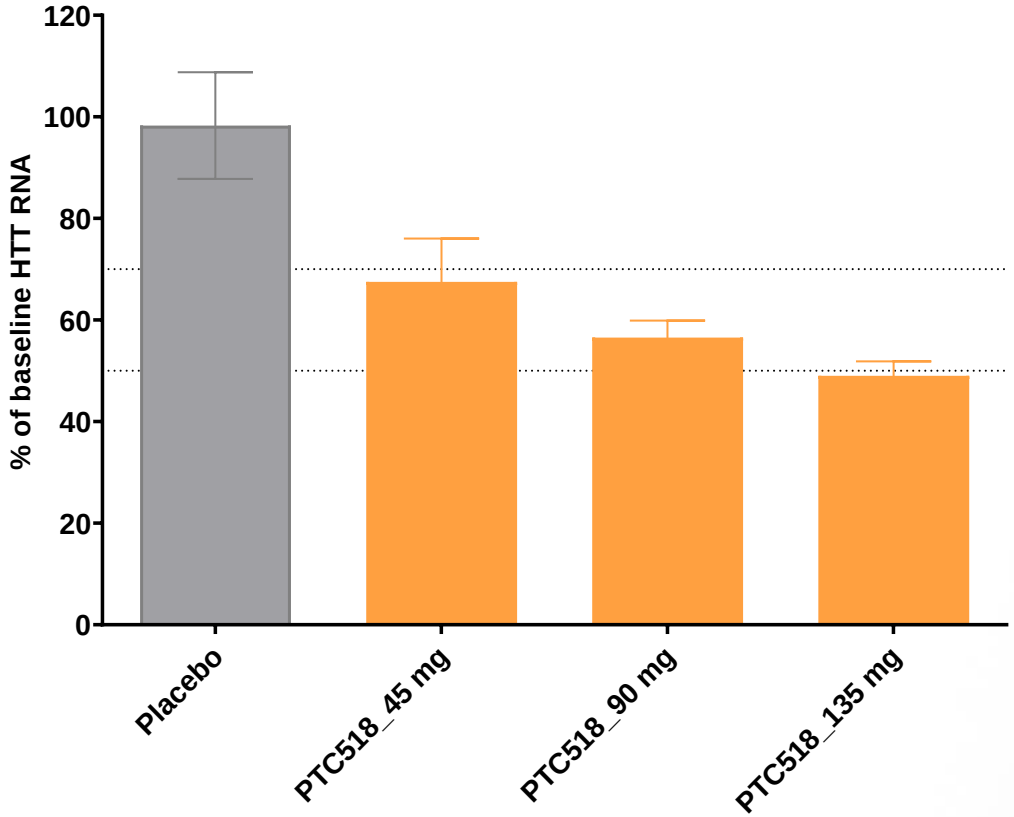
Single ascending dose
- Five cohorts of 8 healthy volunteers (6 active and 2 placebo)
- Evaluate safety & tolerability; HTT mRNA splicing

Multiple ascending dose
- Up to 5 cohorts of 8 healthy volunteers (6 active and 2 placebo)
- Evaluate safety & tolerability; HTT mRNA splicing & protein lowering

Food effect
- Evaluate the effects of food on PTC518 pharmacokinetics

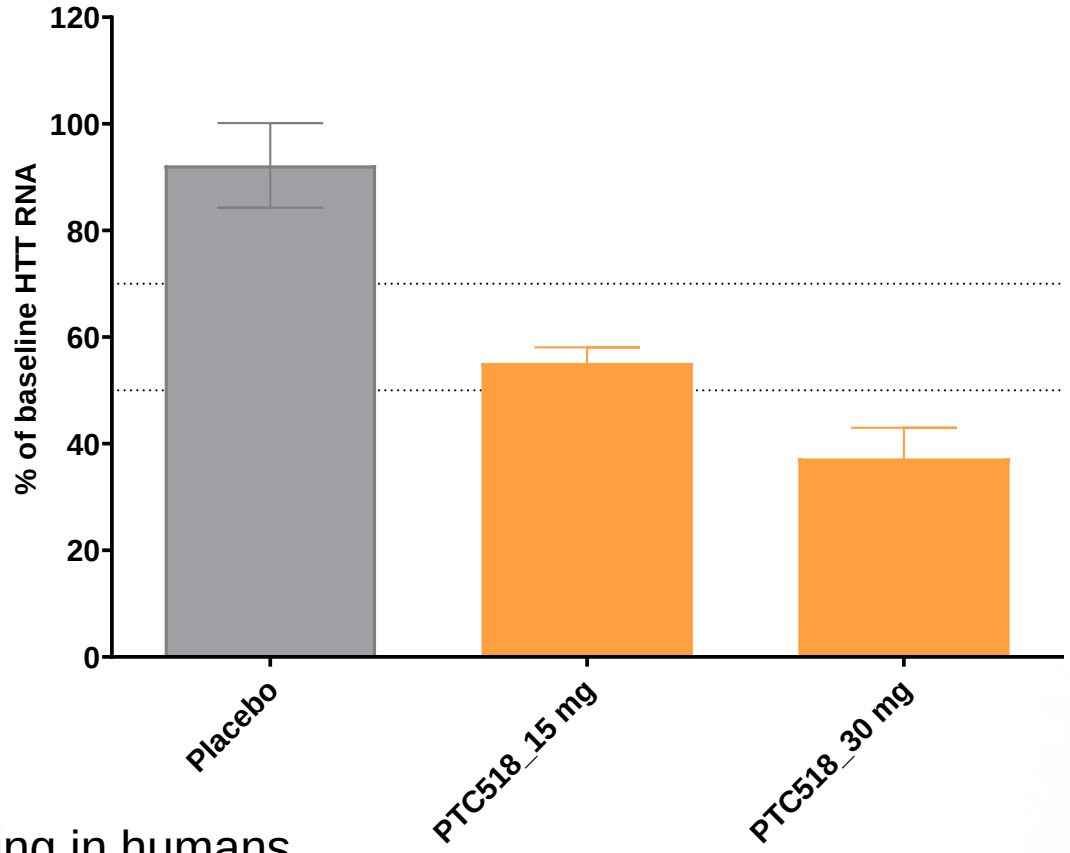
CSF sampling
- Evaluate pharmacokinetics of PTC518 in the CSF
- Compare drug levels in CSF with plasma compartment

SAD Study: Proof of Mechanism of PTC518 Demonstrated By Dose-Dependent *HTT* Splicing



- Whole blood *HTT* splicing in humans
  - Doses evaluated = 45 mg, 90 mg, and 135 mg
  - Time – one day; single dose; splicing evaluated 24h post dose

MAD Study: Proof of Mechanism of PTC518 Confirmed By Dose-Dependent *HTT* Splicing



- Whole blood *HTT* splicing in humans
  - Doses evaluated = 15 mg and 30 mg
  - Time – Day 14; multiple doses; splicing evaluated 6h post dose on day 14

Data on file PTC518-CNS-001-HD

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology	Virology
Commercial	Emflaza® (deflazacort) 6 mg | 18 mg | 30 mg | 36 mg tablets 22.75 mg/mL oral suspension	Tegsedi™ (inotersen) waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL	translarna™ ataluren	Evrysdi® risdiplam					
Clinical			US Ataluren	PTC518 HD	PTC-AADC	Vatiquinone MDAS Vatiquinone FA PTC857 GBA-PD	PTC923 PKU	Unesbulin DIPG Unesbulin LMS Emvododstat AML	Emvododstat COVID-19
Research				SCA-3 MAP-Tau	FA Angelman IRDs Cog Disorders	Undisclosed			

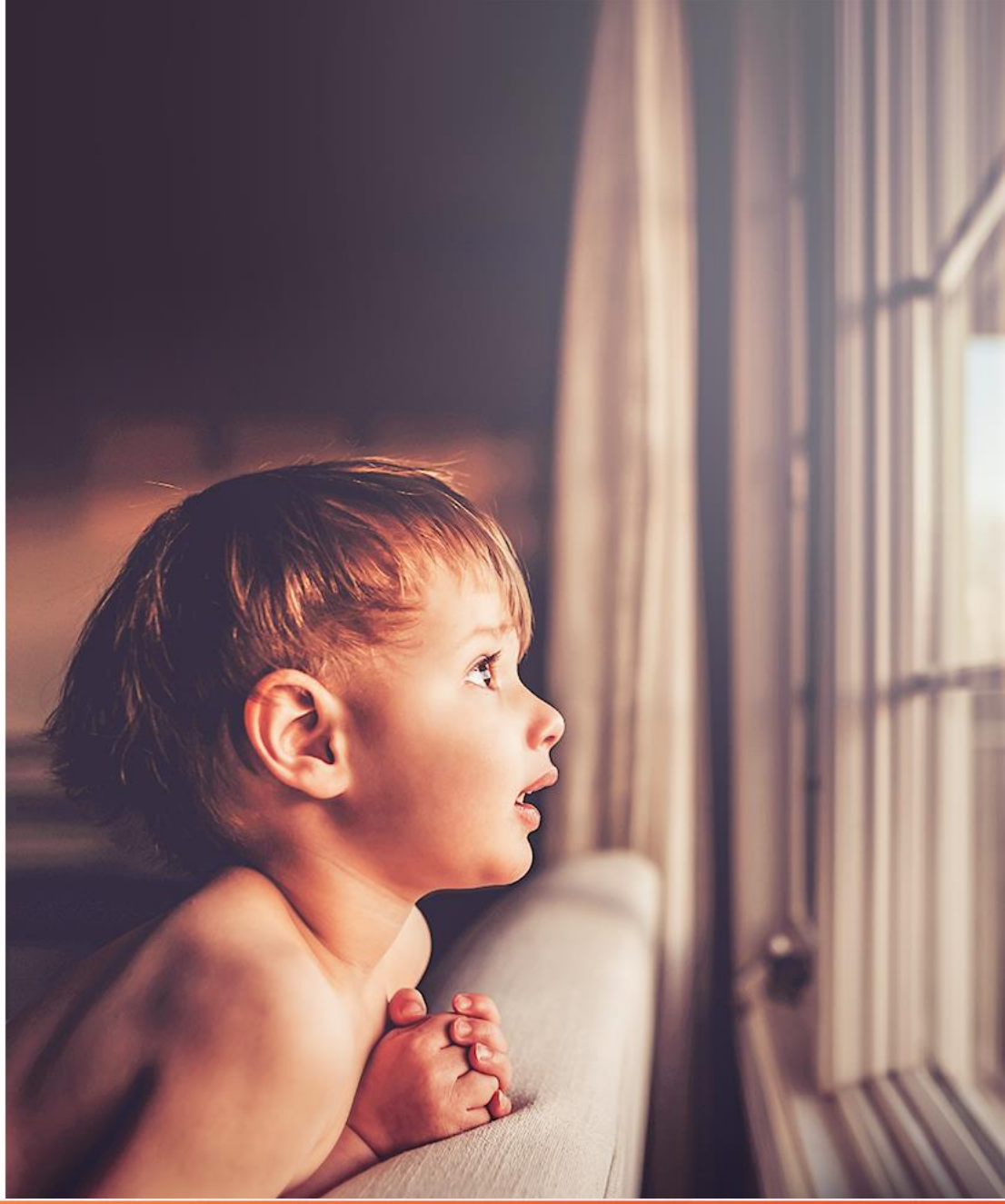
Potential registrational studies

- AADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich's ataxia; GBA, glucocerebrosidase; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; ME, Mitochondrial Epilepsy; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

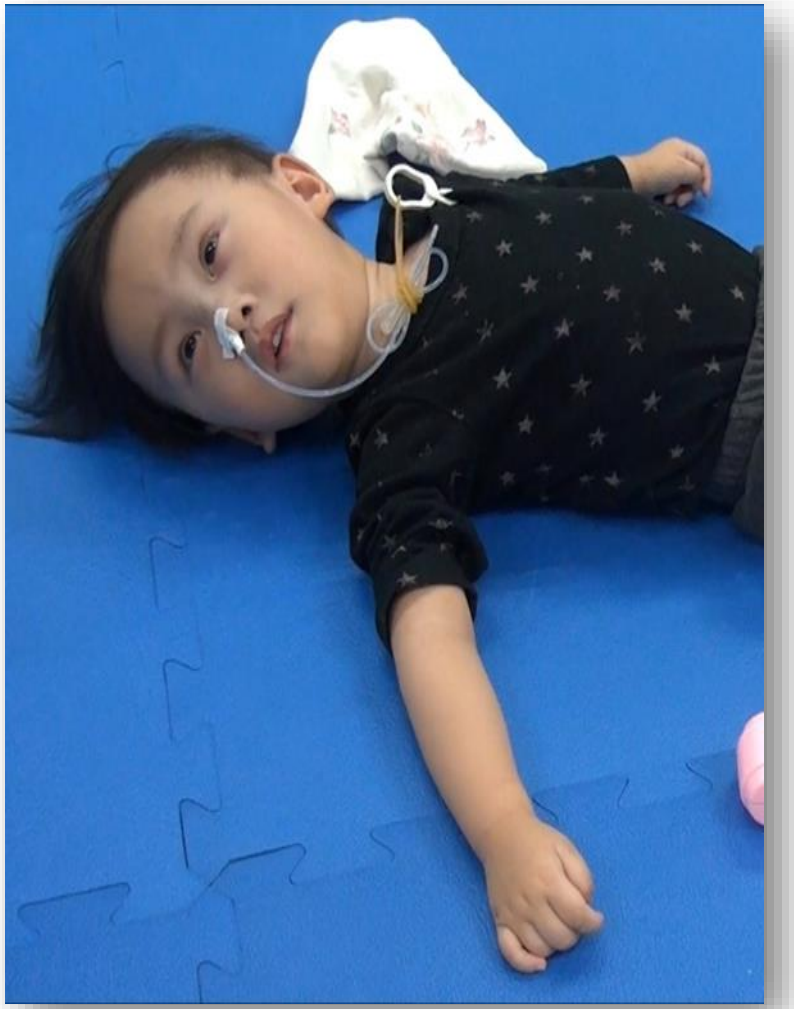
Treating Rare Monogenic Disorders with Targeted Gene Therapy

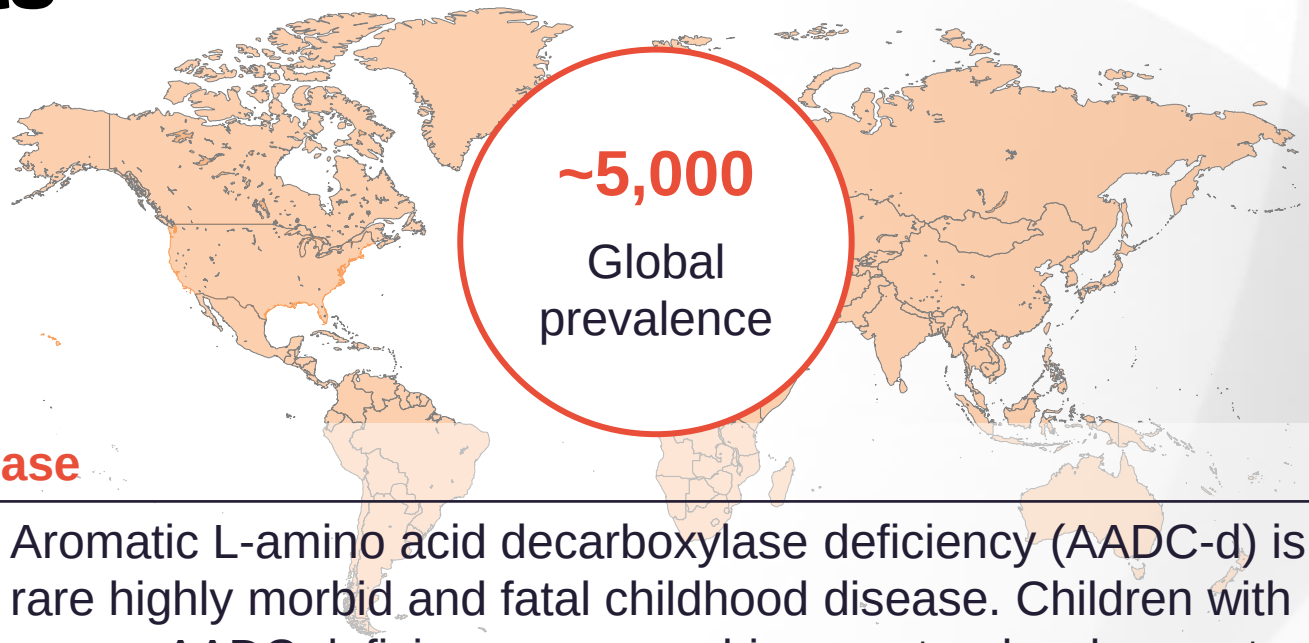
Potential advantages of targeted therapy

- Local administration lowers systemic immunogenicity and exposure
- Low turnover cells may lead to improved durability
- Leveraging stereotactic neurosurgery technologies to enable precise and accurate delivery
- Lead treatment for AADC-d, pipeline includes FA and AS



Gene Therapy Has the Potential to Provide Significant Benefit to AADC Patients





Disease

- Aromatic L-amino acid decarboxylase deficiency (AADC-d) is a rare highly morbid and fatal childhood disease. Children with severe AADC deficiency never achieve motor development milestones

Current Treatments

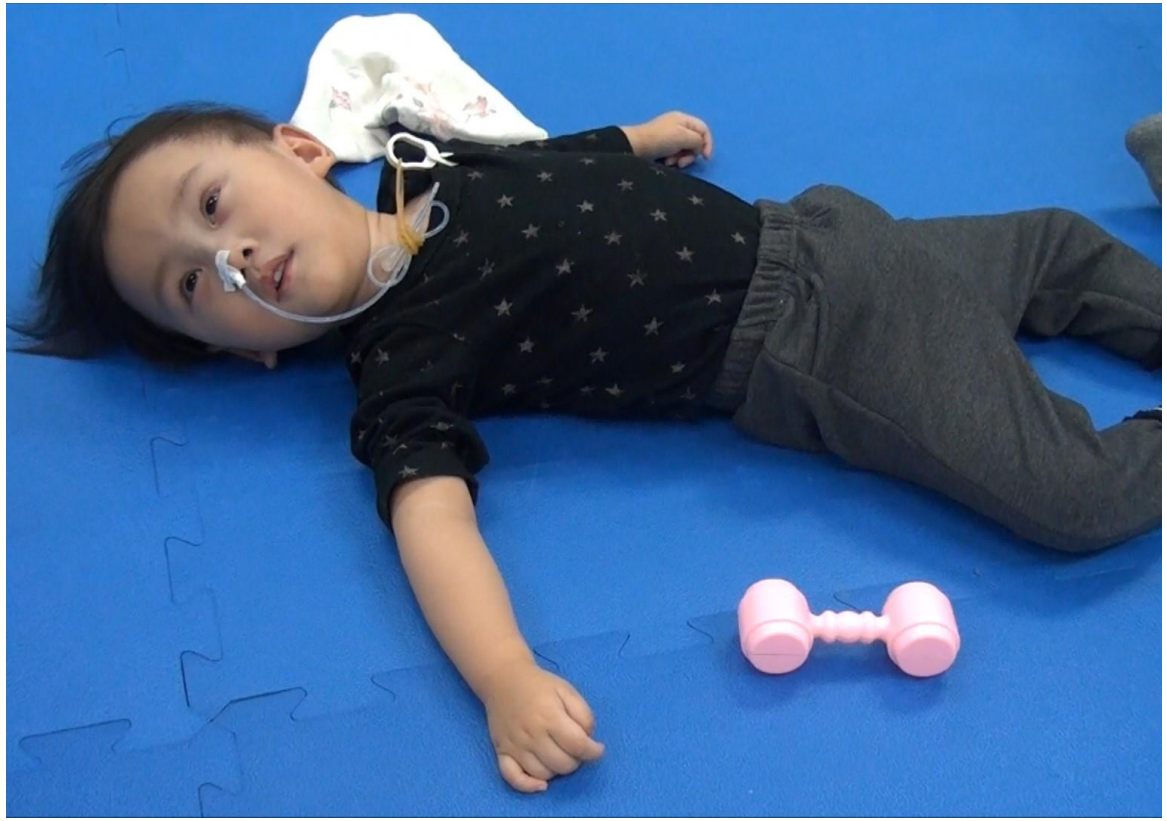
- No disease modifying therapies approved

Opportunity

- Potential for AADC gene therapy to become standard of care. Patients can achieve motor and cognitive long-term improvement

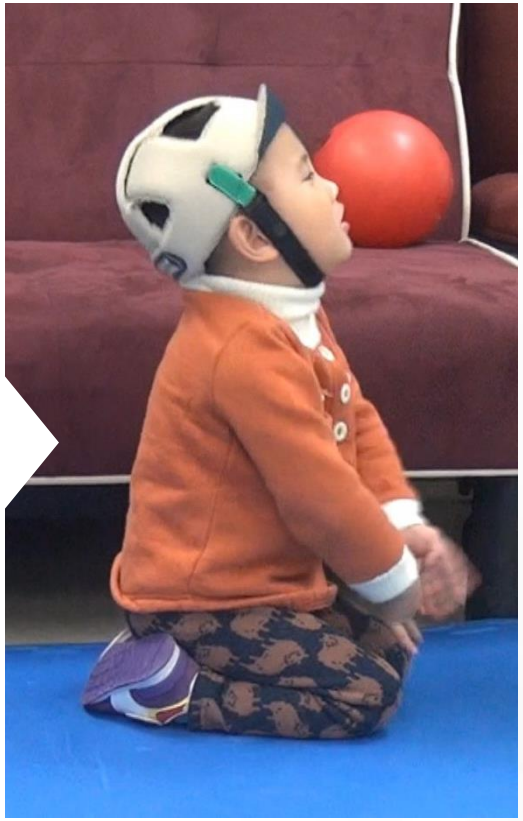
PTC-AADC Treated Patients Make Significant and Sustainable Progress

Untreated



Age 2

Post-Treatment



Age 3

Age 4.5

Preparing for PTC's First Gene Therapy Launch

EU Regulatory

The CHMP has recently asked for additional manufacturing bioanalytical data in support of the MAA. We expect to provide the additional data in the first quarter of 2022 and now expect an opinion from the CHMP shortly after that.

US Regulatory

PTC-AADC BLA submission expected in 1Q22

Treatment Centers

Identification and preparation of expert pediatric neurosurgical centers

Patient Finding

Ongoing patient finding targeting 300 patients identified at launch

Providing Patients Access to Transformative Treatments

Global Geographic Presence Supports Growing Product Portfolio

Offices in 20 countries

Footprint in >50 countries

>1,000 employees

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

Tegsedi™ (inotersen) injection 284 mg/1.5 mL

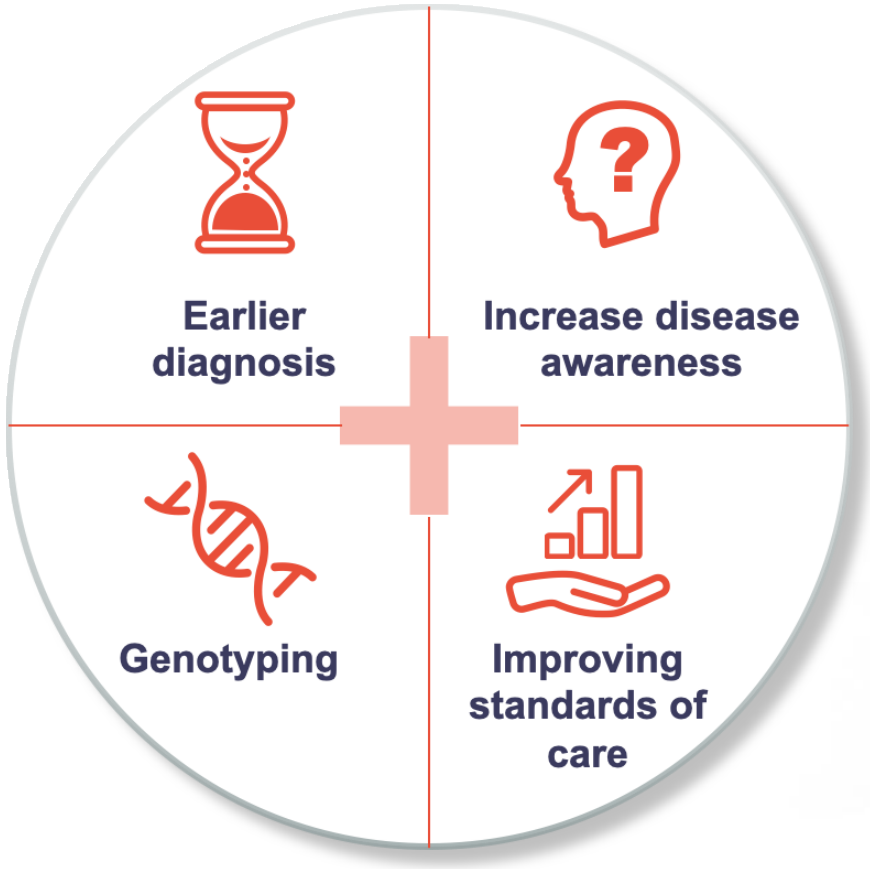
waylivra™ (volanesorsen sodium) Injection 300mg in 1.5mL

translarna™ ataluren

Evrysdi® risdiplam

PTC THERAPEUTICS

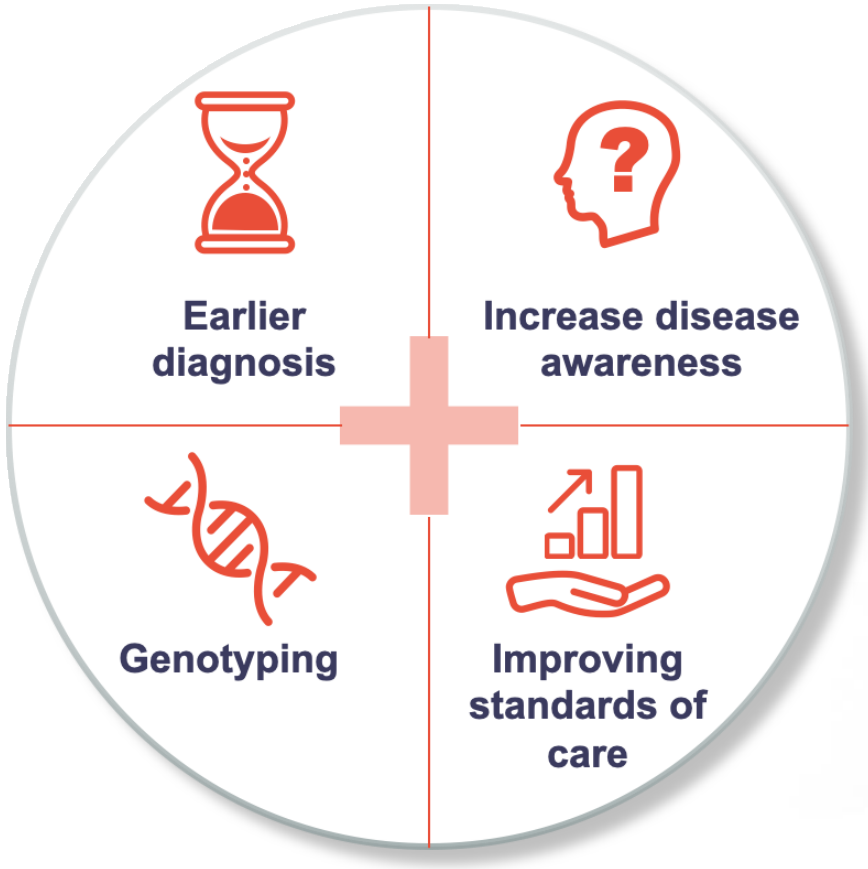
DMD Commercial Franchise – A Growing Global Business



DMD Commercial Franchise – A Growing Global Business

translarna™
ataluren

- YE 2020 net product revenue of $192M
- Treatment for nonsense mutation DMD for ages 2 and older
- Distributed in over 50 countries worldwide
- Data from Study 041 expected 3Q22



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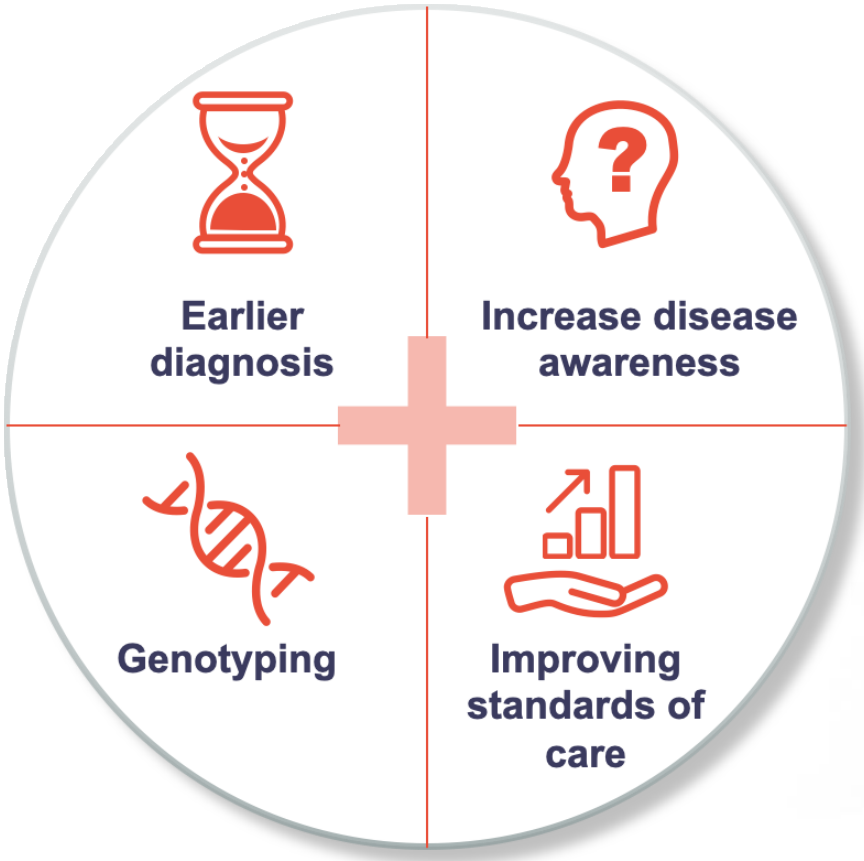
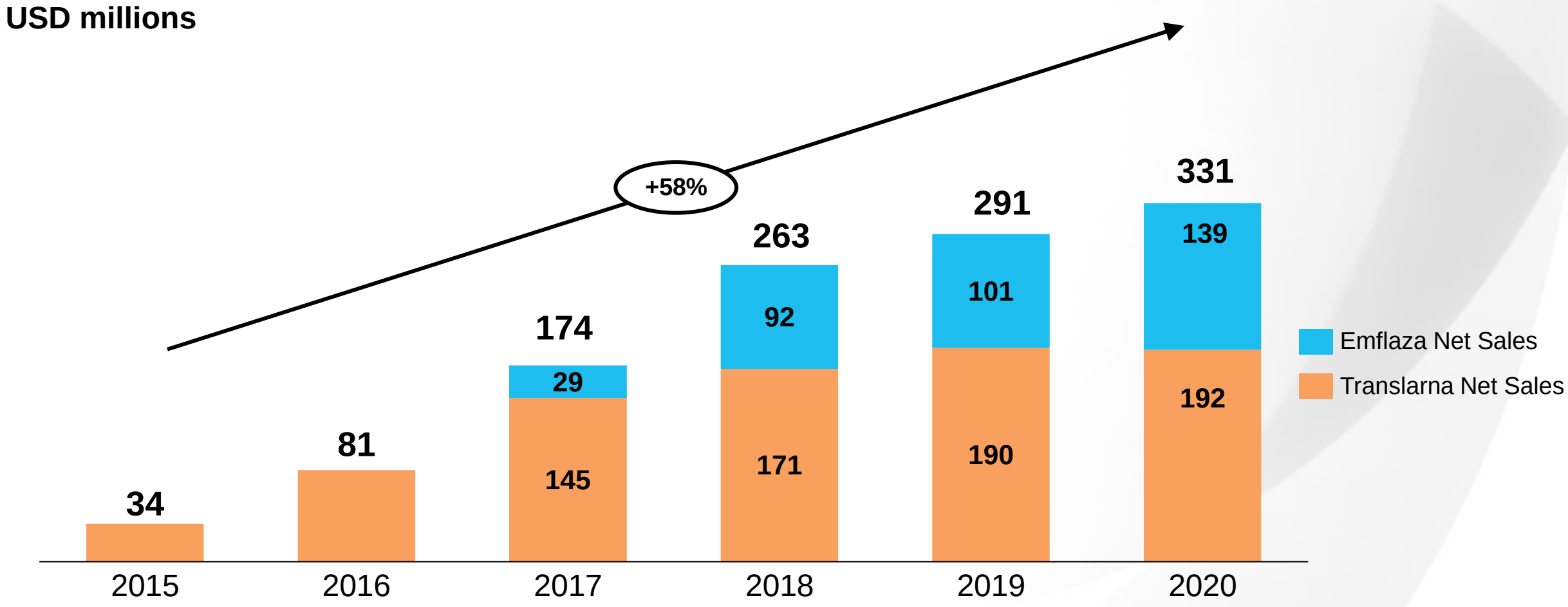


Emflaza®
(deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

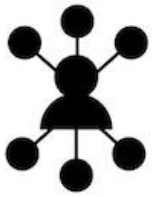
- YE 2020 net product revenue of $139M
- First and only corticosteroid approved for DMD
- Approved for all DMD patients in the US >2yrs
- Data from multiple publications demonstrate Emflaza's clinical benefit over prednisone

Continued Strong DMD Franchise Growth



Evrysdi's Strong Global Launch Brings Therapy to SMA Patients with High Unmet Need

Patients treated across all SMA types



Patients are treatment-naïve or switching from both Spinraza and Zolgensma

Broadest range of age treated

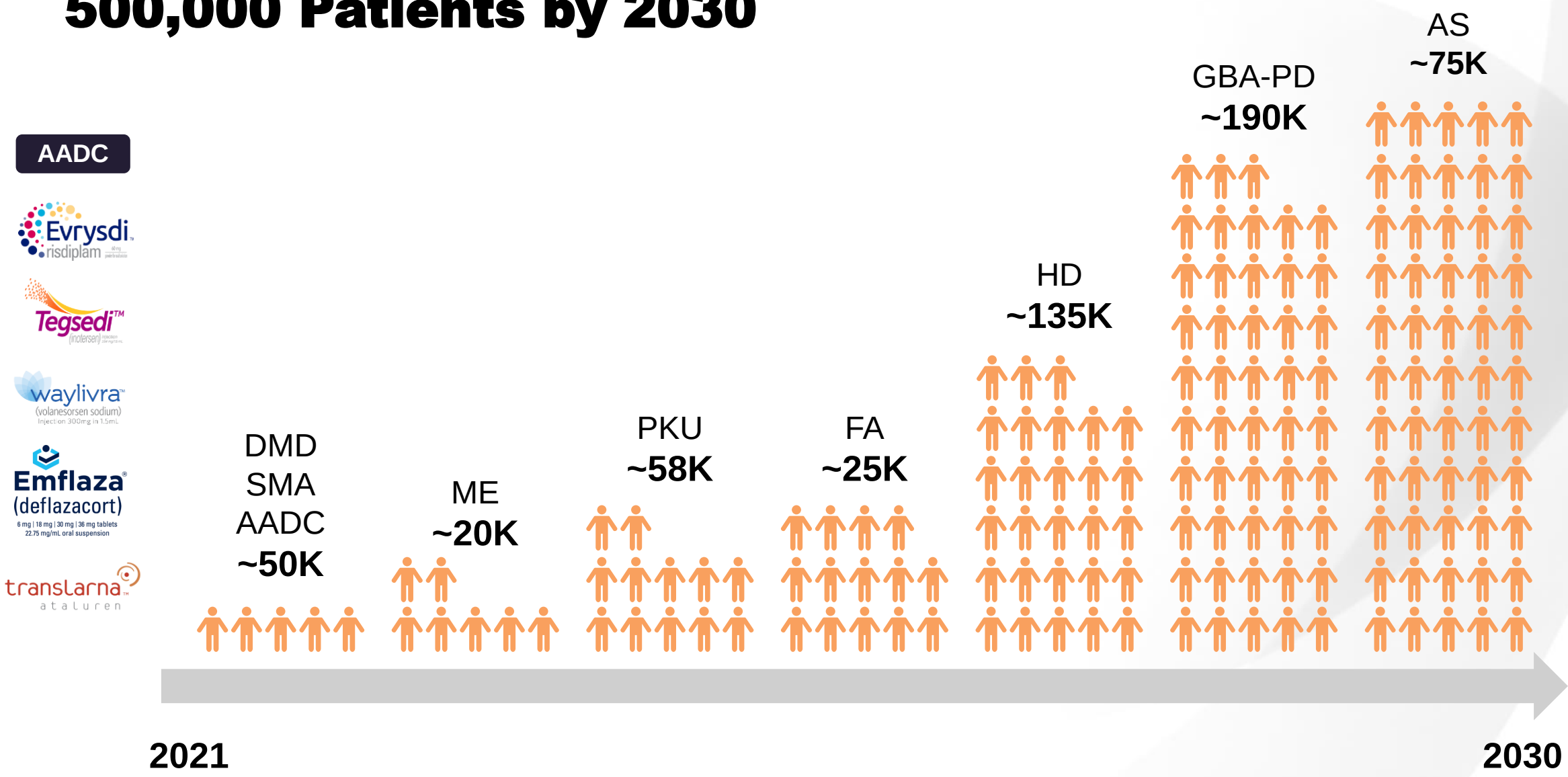
Differentiated product for SMA patients

- The first at-home, oral treatment for SMA
- Global approvals and regulatory submissions
- EMA approval in 1Q21
- Japanese approval in 2Q21

Significant milestones ahead

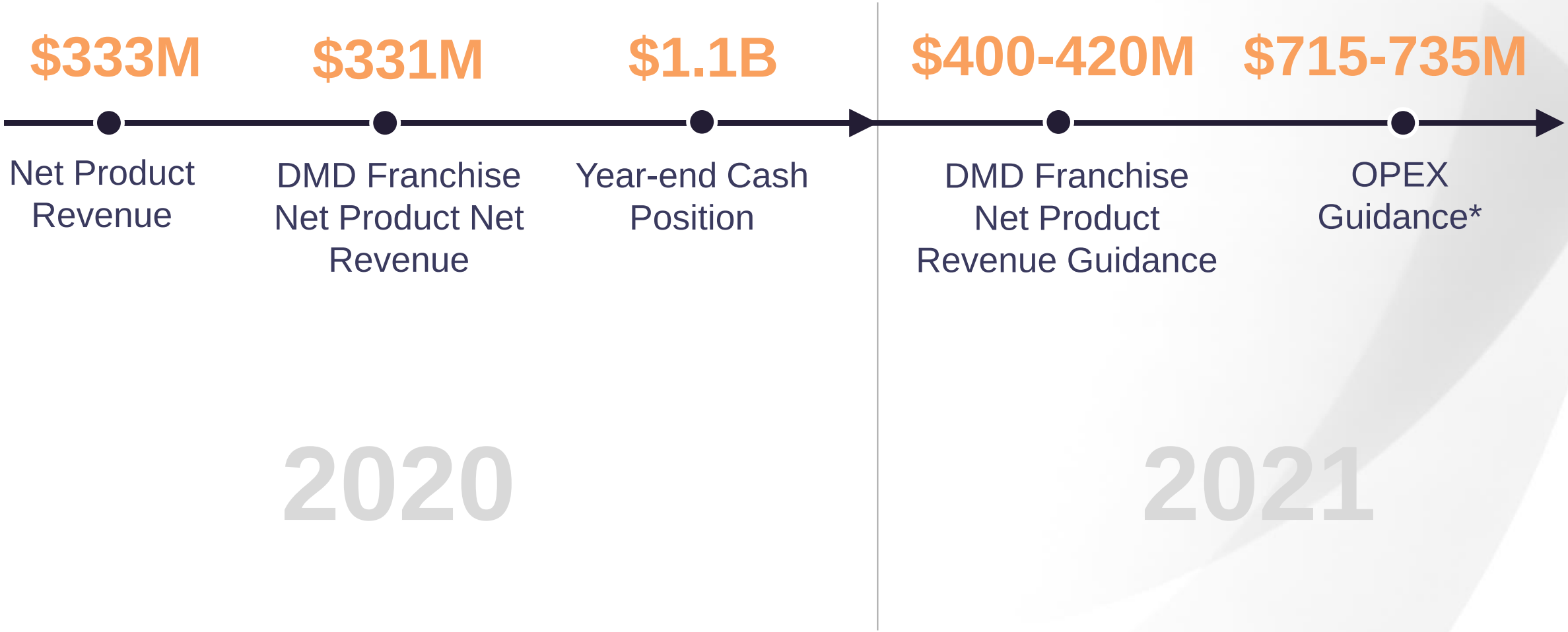
- Potential $300 million in sales milestones
- ~15% tiered royalty on global sales

Multiple Platforms Provide Opportunity to Treat Over 500,000 Patients by 2030



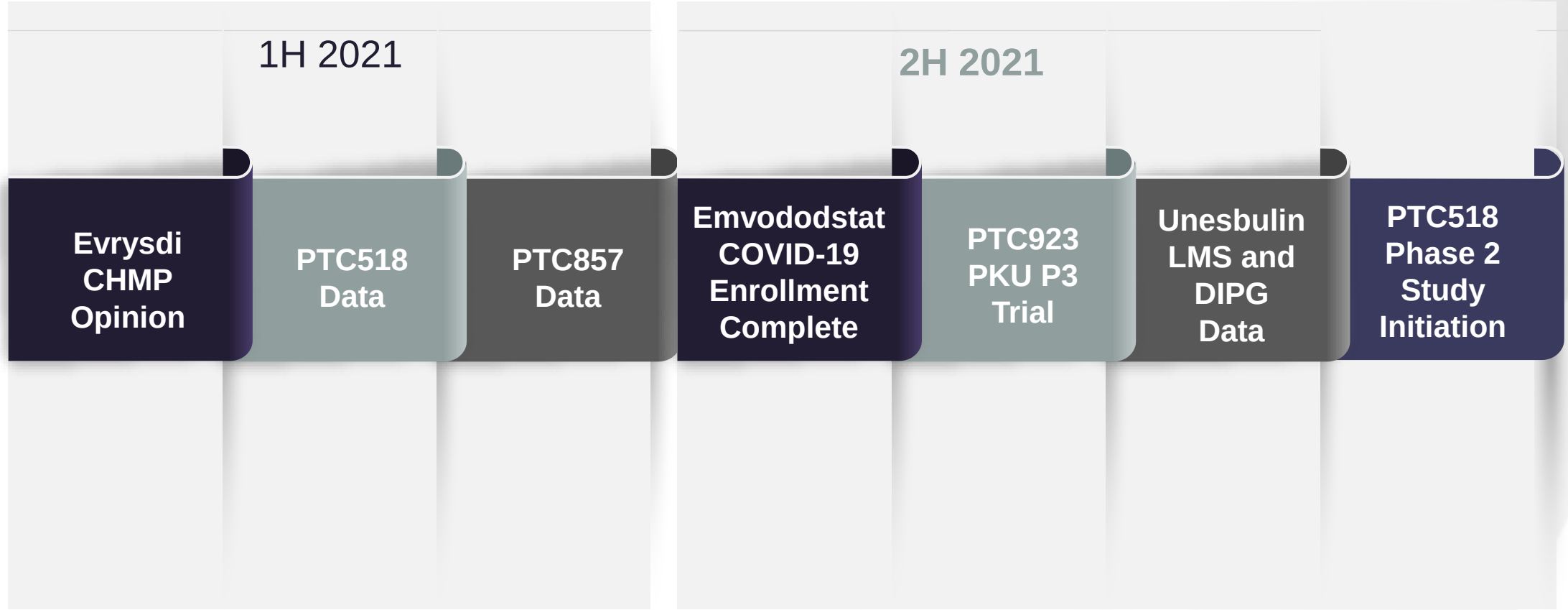
Strong Financial Performance Supports Future Growth

Strong Financial Performance Supports Future Growth

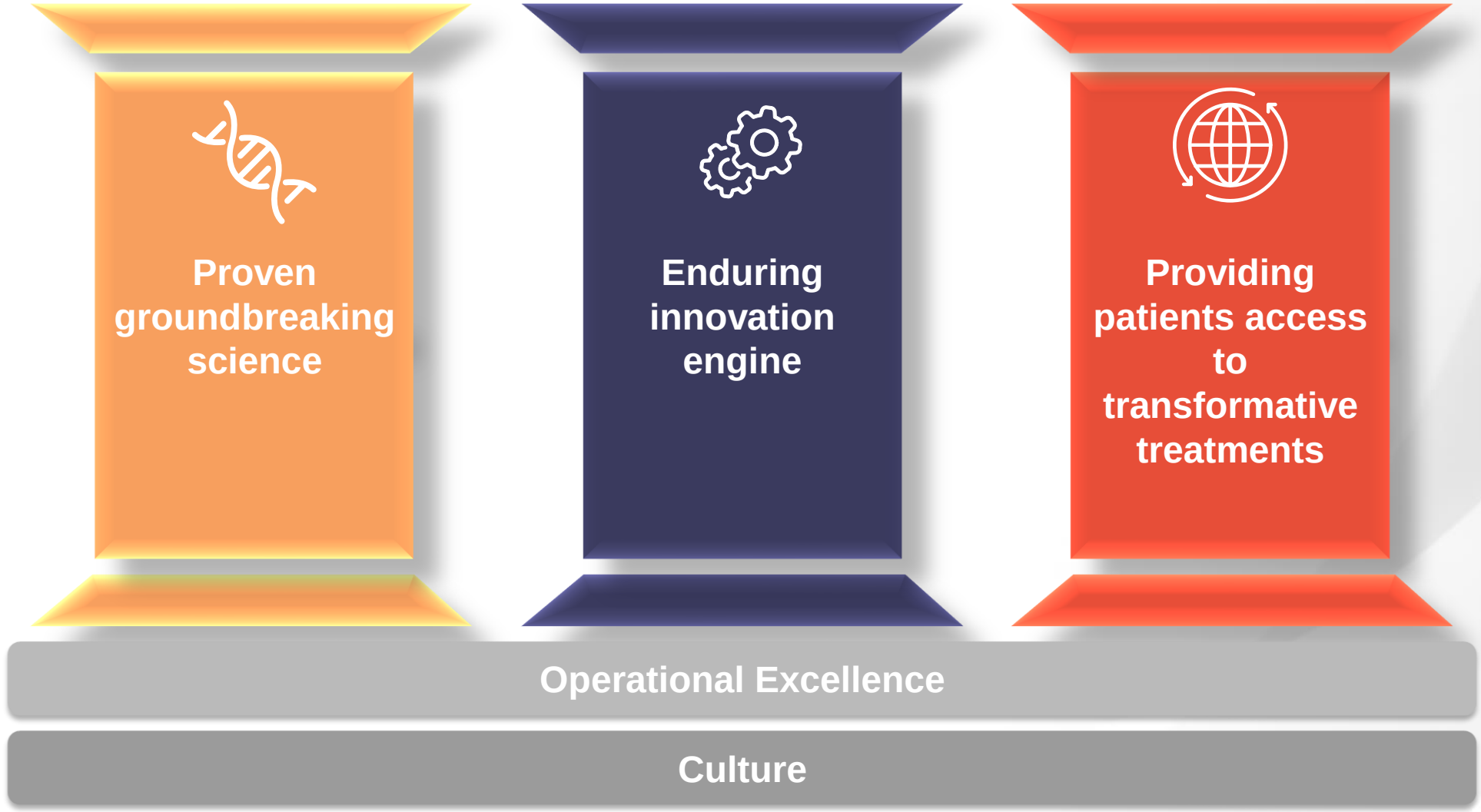


*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $100 million. GAAP R&D and SG&A expense for the full year 2021 is anticipated to be between $815 and $835 million.

2021 Potential Milestones to Generate Value



PTC has Built a Strong, Sustainable Company to Execute on our Mission



Translating Science to Transform Lives