PTC 2023

Matthew Klein, M.D., COO
JP Morgan Healthcare Conference

January 9, 2023

PKU Patient

PTC THERAPEUTICS®

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 preliminary unaudited 2022 financial information with respect to 2022 total net revenue and 2022 DMD franchise net product revenue, statements with respect to guidance relating to 2023 total net product revenue, 2023 DMD franchise net product revenue and 2023 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions, commercialization and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any 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; 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.

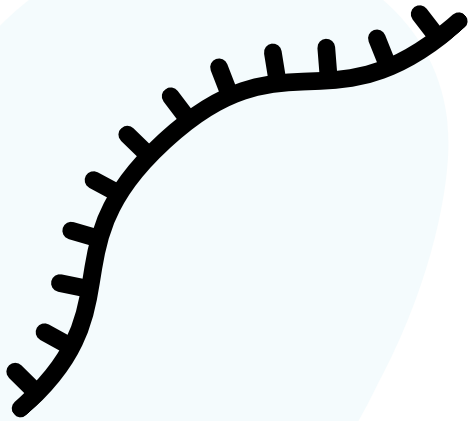
PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, 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; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; 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 future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to utilize results from 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, to support a marketing approval for Translarna for the treatment of nmDMD in the United States and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the results of PTC's clinical trial for emvododstat for COVID-19; 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 its lease agreements, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; 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 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.

Treating Rare Diseases by Modulating Gene and Protein Expression

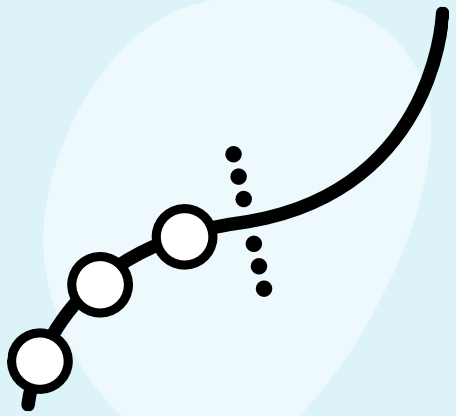
DNA



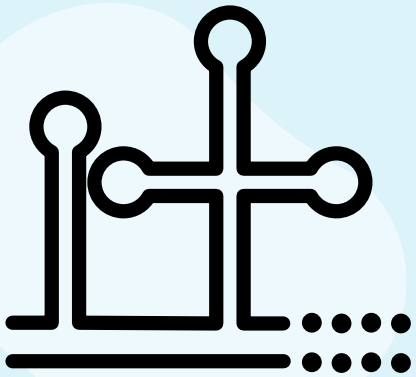
RNA

Protein

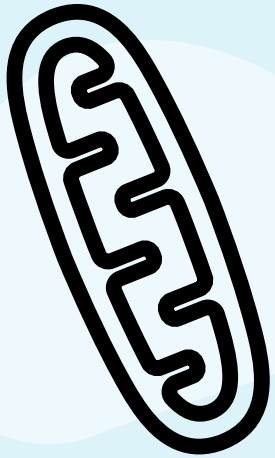
Treating Rare Diseases by Modulating Gene and Protein Expression



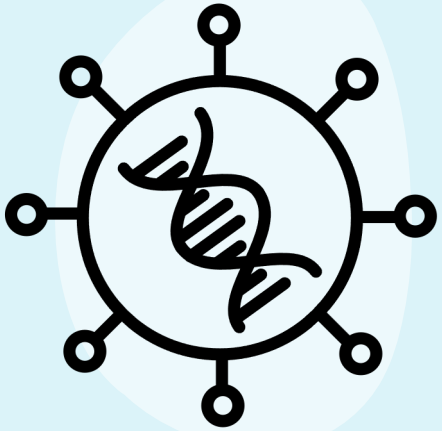
Nonsense Suppression



Splicing

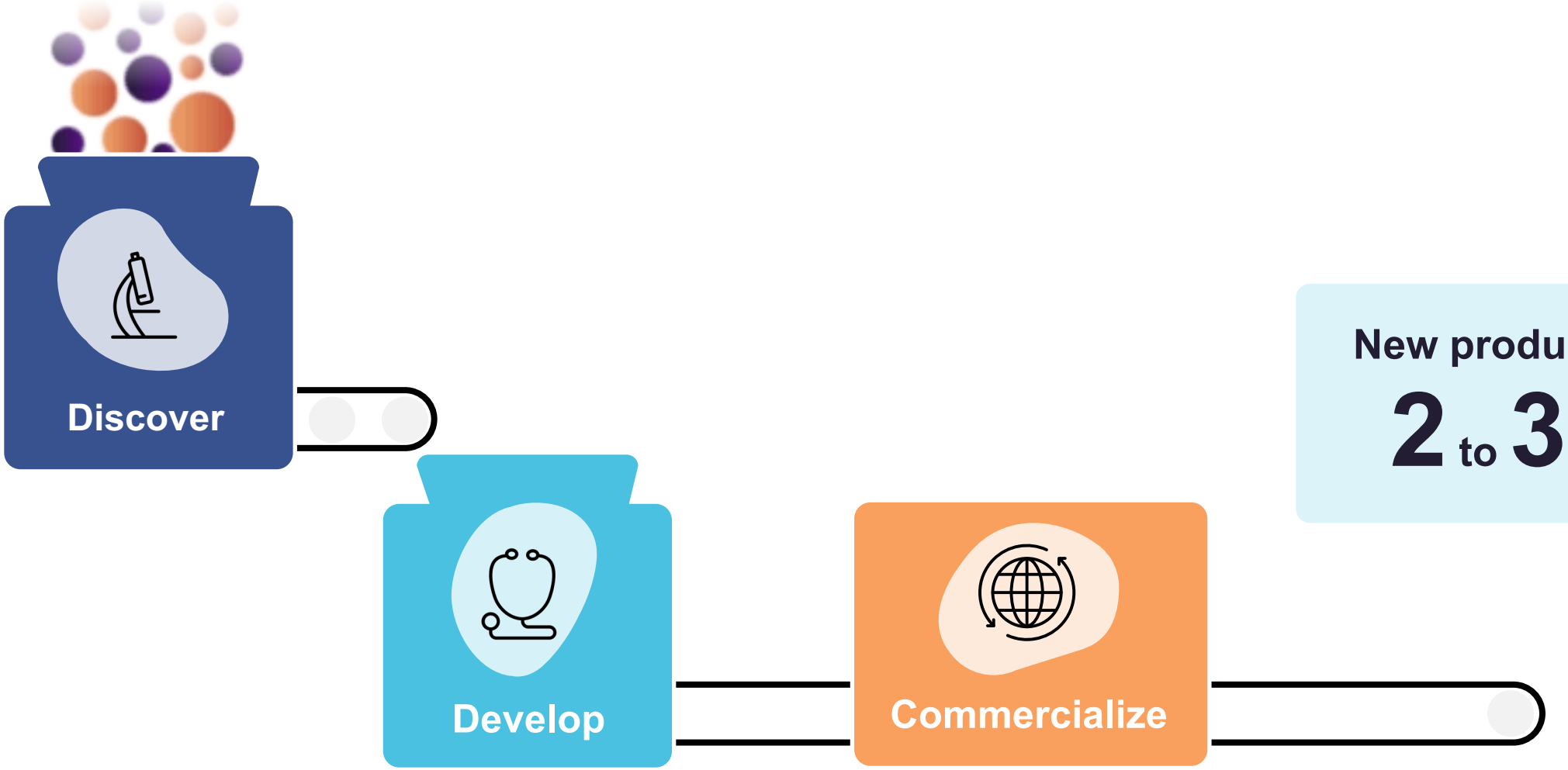


Bio-e



Gene Therapy

Building a Pipeline to Produce a Therapy Every Two to Three Years



New product every

2 to **3** years

A Diverse and Robust Portfolio To Continually Create Value

Commercial

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

Small Molecule

Gene Therapy

Neurology

Metabolism

Oncology

Research

Neurology
- SCA-3
- GT-FA
- MAP-Tau
- GT-AS
- 13 Undisclosed

Metabolism
- 2 Undisclosed

Oncology
- 3 Undisclosed

Development

Neurology
- PTC518 HD
- Vatiquinone MDAS
- Utreloxastat ALS
- Vatiquinone FA

Metabolism
- Sepiapterin PKU

Oncology
- Unesbulin LMS
- Unesbulin DIPG

DIPG, diffuse intrinsic pontine glioma; FA, Friedreich ataxia; ALS, amyotrophic lateral sclerosis; HD, Huntington's disease; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

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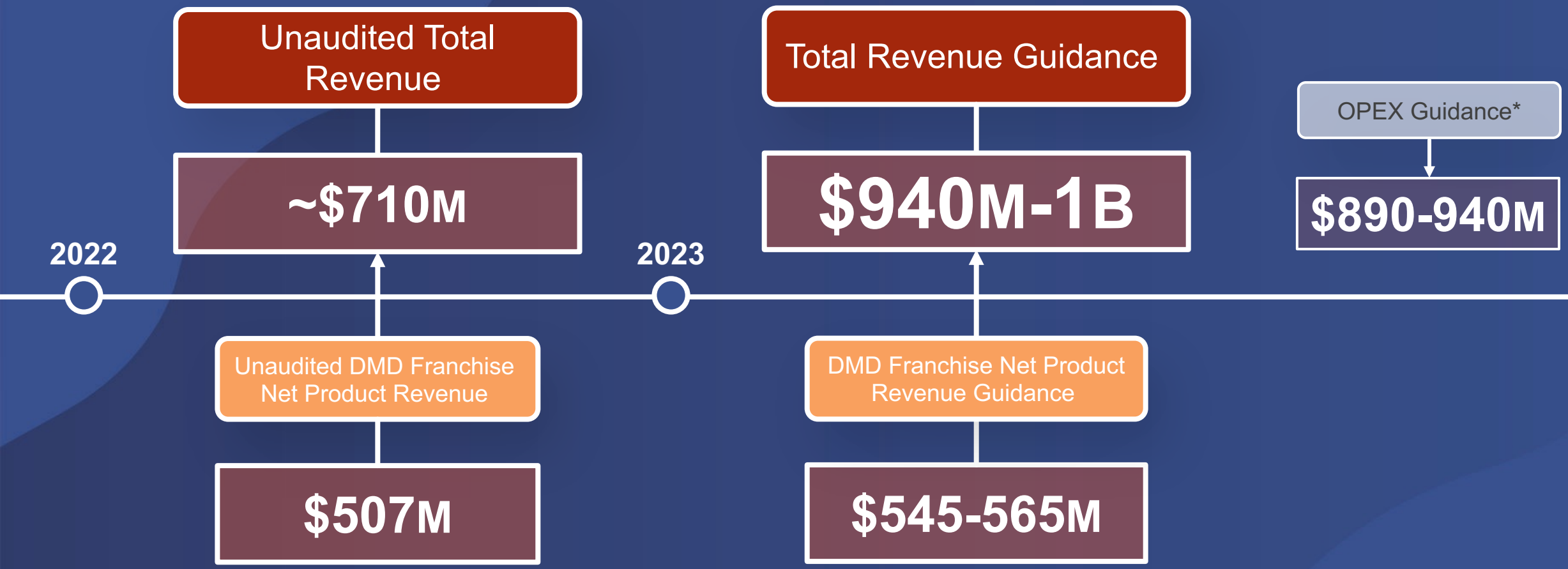
Continuing Strong Financial Performance Expected in 2023



*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $120 million. GAAP R&D and SG&A expense for the full year 2023 is anticipated to be between $1,010 and $1,060 million. The Company anticipates up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions.

Continued Success Across Our Commercial Portfolio

translarna™ ataluren

Distributed in 50+ countries with continued growth from new patients and geographic expansion

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

First and only corticosteroid for all US DMD patients with growth from new patient starts and favorable access

Evrysdi® risdiplam

Established market leadership in all major markets with continued growth expected

Upstaza™ (eladocagene exuparvovec)

First EMA approved disease-modifying treatment for AADC deficiency for patients 18 months and older

Tegsedi® (inotersen) injection 284 mg/1.5 mL

For treatment of hATTR with LATAM patients benefiting through early-access programs

waylivra® (volanesorsen) injection 285 mg/1.5 mL

For treatment of FCS and FPL with LATAM patients benefiting through early-access programs

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Building Foundation for Upstaza Success

Upstaza™
(eladocagene exuparvovec)

2022

Early Access

Commercial Access

Cross Border

PTC THERAPEUTICS

Substantial Growth in 2023 and Beyond with Additional Global Registrations

Upstaza™ (eladocagene exuparvovec)

2022

Early Access

Commercial Access

Cross Border

2023 and Beyond

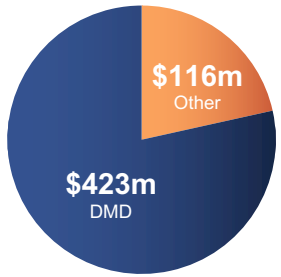
PTC THERAPEUTICS

Substantial Revenue Growth from 2021 to 2023

Commercial

2021

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

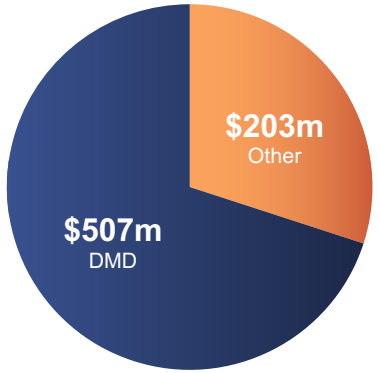


$539M

Total revenue

2022

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

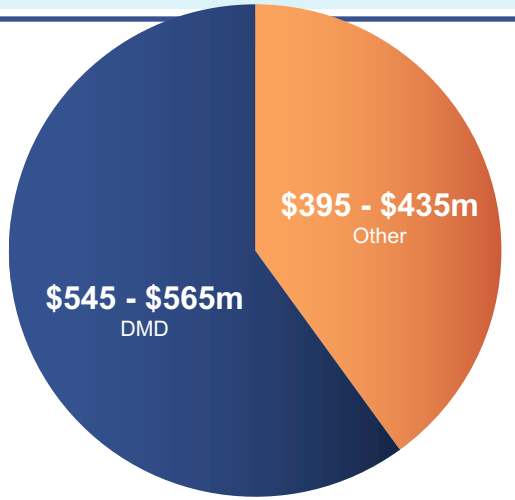


~$710M

Unaudited total revenue

2023

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



$940M-$1bn

Potential future revenues

Development

Sepiapterin | Vatiquinone | Unesbulin | PTC518 | Utreloxastat |

PTC THERAPEUTICS

Strong R&D Execution and Value Creation in 2022

Clinical Achievements

- Initiated CardinALS study for utreloxastat
- Initiated PIVOT-HD study for PTC518
- Initiated SunriseLMS trial for unesbulin
- Completed Study 041 for Translarna
- Completed FITE-19 study for emvododstat
- Completed enrollment for MIT-E

Regulatory Achievements

- Upstaza EU and UK approval
- Waylivra approved in Brazil for treatment of FPL
- Filed type II variation for MAA for Translarna in EU and additional global approvals

Substantial Pipeline Progress Planned in 2023

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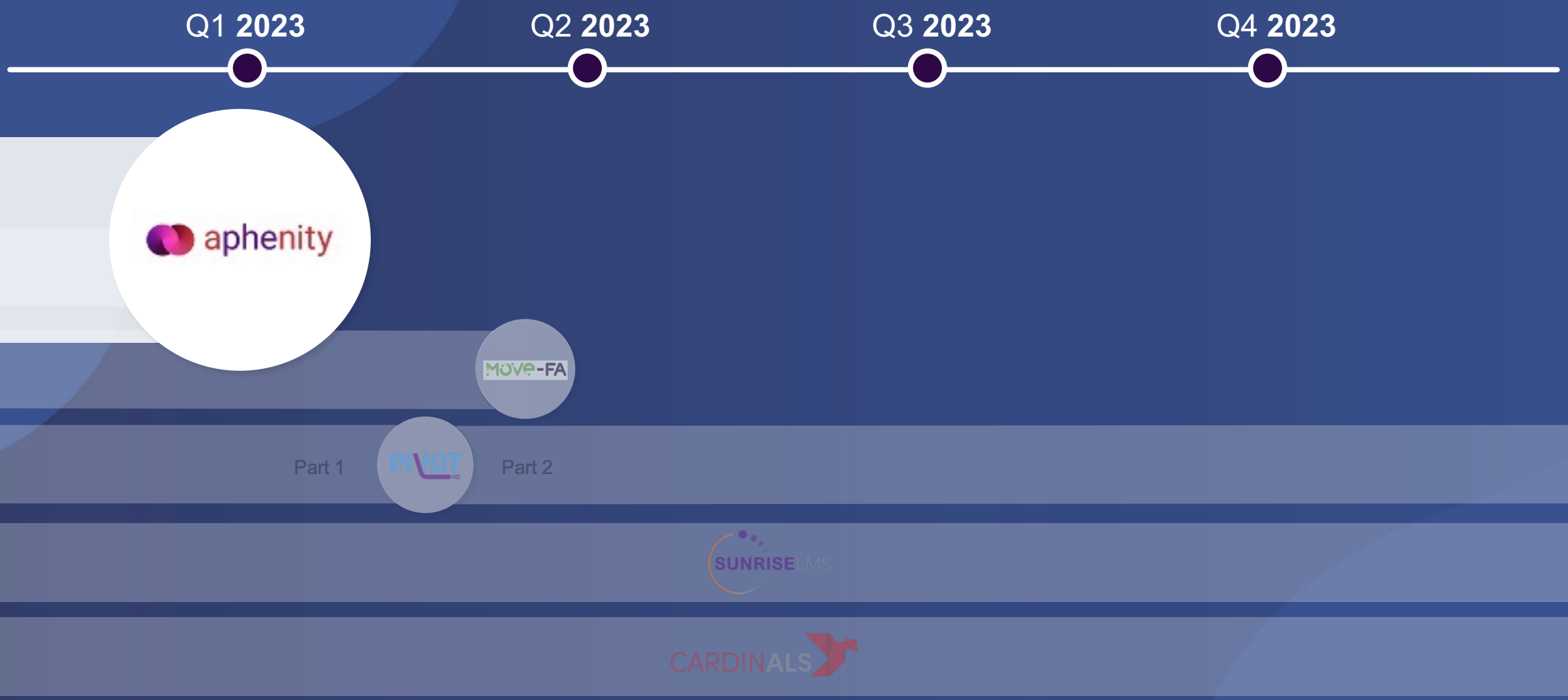
Substantial Pipeline Progress Planned in 2023



Sepiapterin Can Potentially Treat Broad PKU Population Including Classical PKU

aphenity

Disease

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

Mechanism of Action

Sepiapterin is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat a broader range of PKU patients

Current Treatments

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

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Large Unmet Need Remains in PKU

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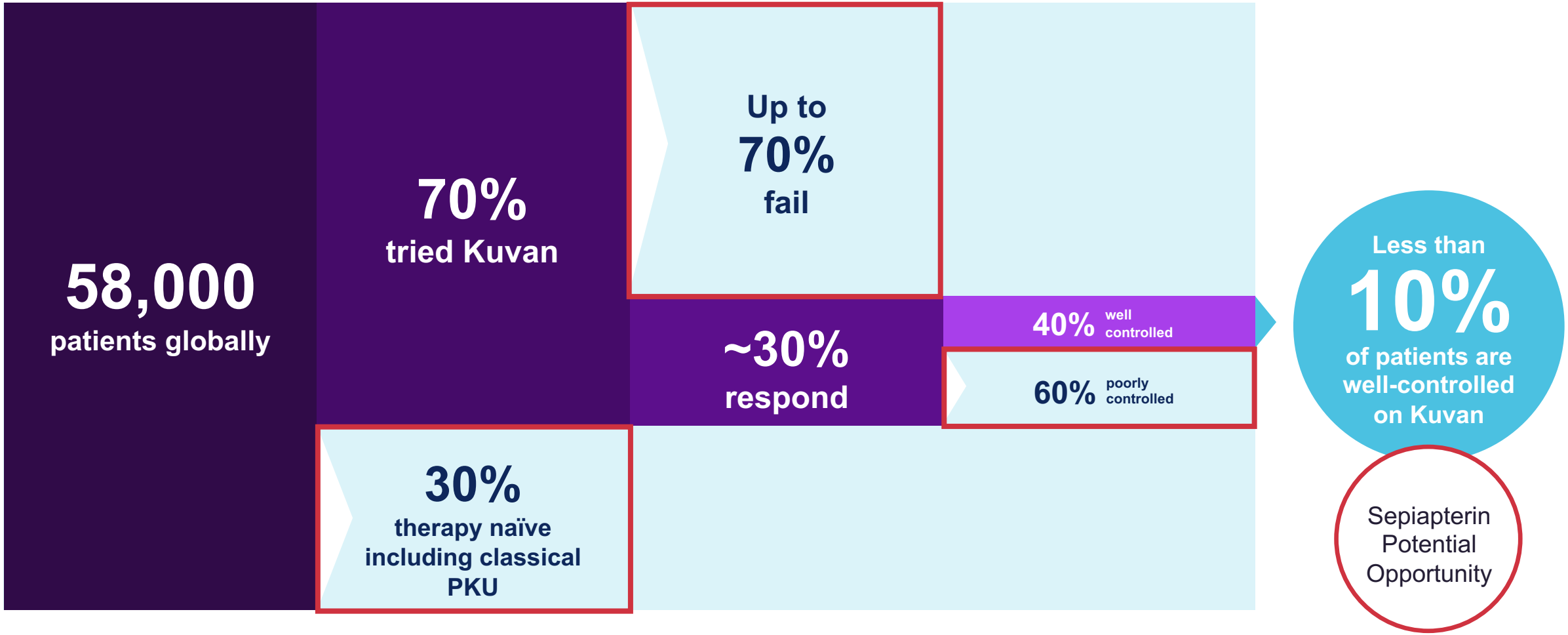

1. BioMarin first quarter 2021 presentation 1Q 2021. 2. Third party reports prepared for PTC

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Established Pillars for Commercial Success

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- Newborn screening with ~58,000 patients worldwide[1,2,3]
- Well-known metabolic centers of excellence across the world
- Disease pathology well understood and documented
- Connected and coordinated patient advocacy community

1. NPKUA; https://www.npkua.org/, 2. Shoraka et. Al, Clin Exp Pediatr. 2020; 63(2): 34–43., 3. Borrajo G., Acta Ped Mex. 2012; 33(6): 279-287.

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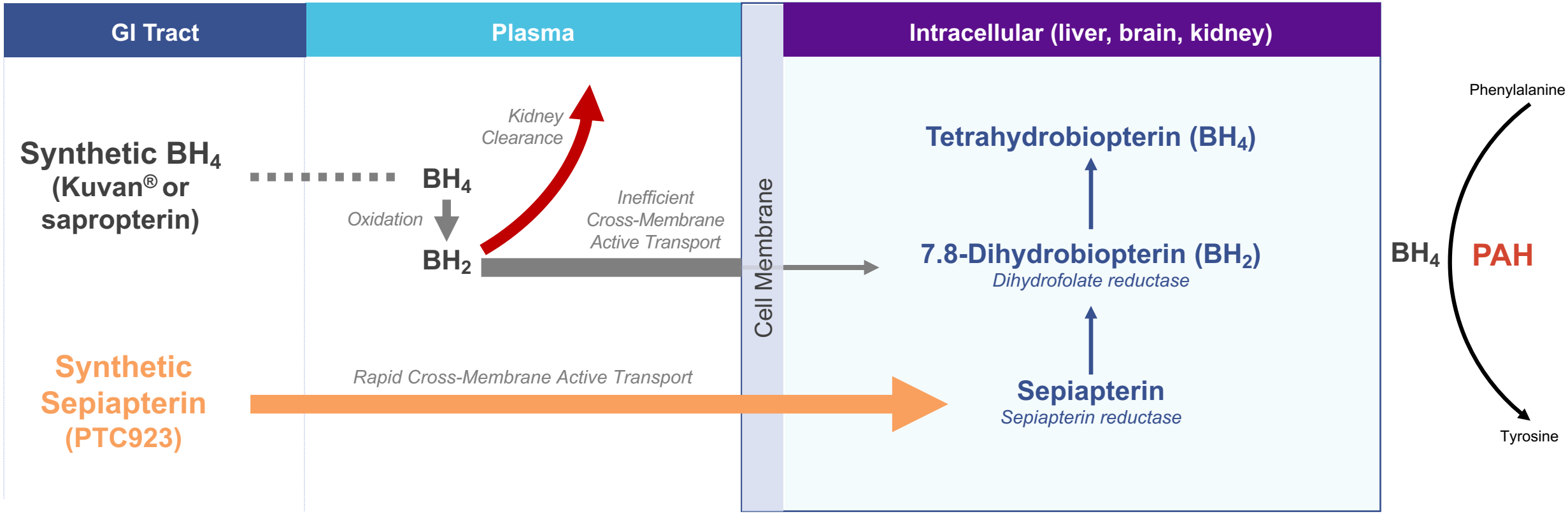
Mechanistic Advantages of Sepiapterin Over Sapropterin: Increased Bioavailability

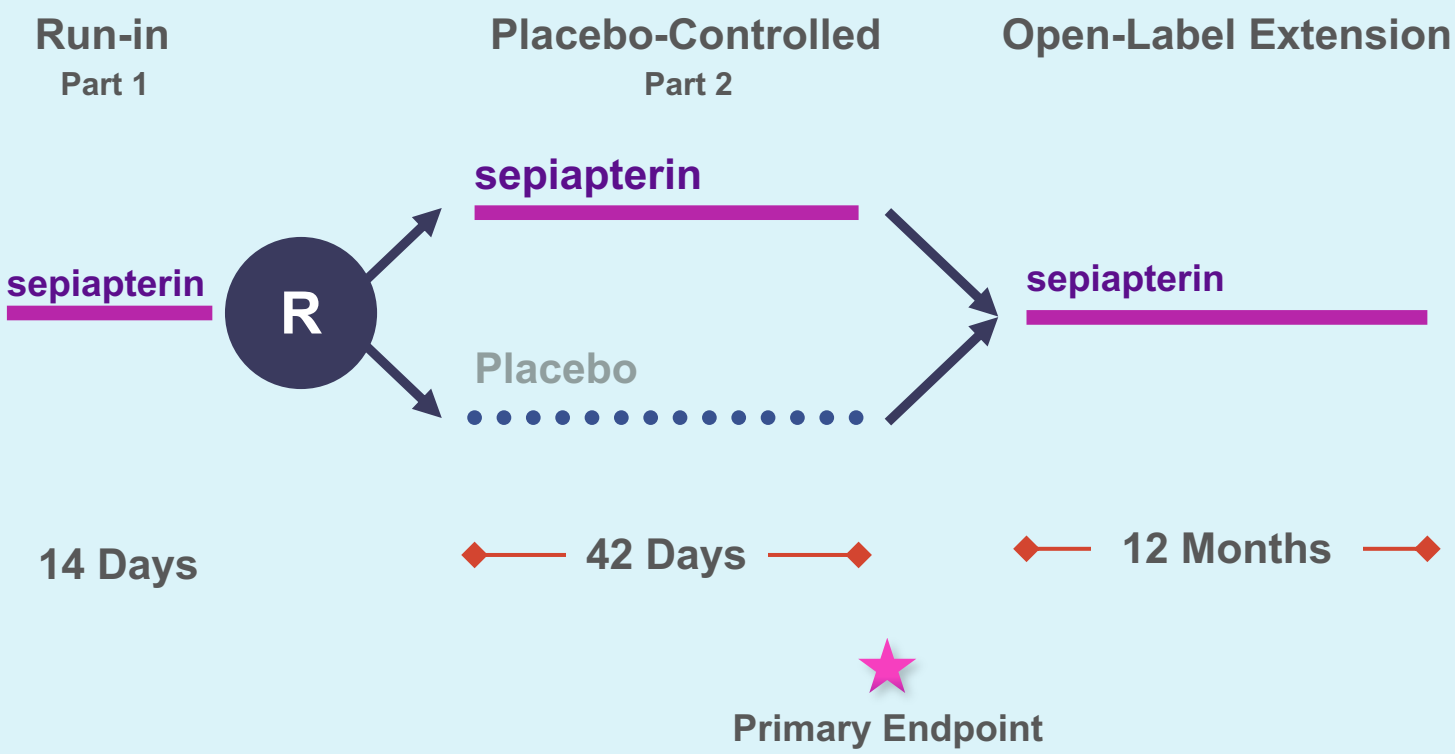
aphenity



PTC THERAPEUTICS

APHENITY is a Global Registration-Directed Trial of Sepiapterin for PKU

aphenity



Primary Endpoint

Reduction in blood phenylalanine levels

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APHENITY Part 1 Preliminary Data (n=104)

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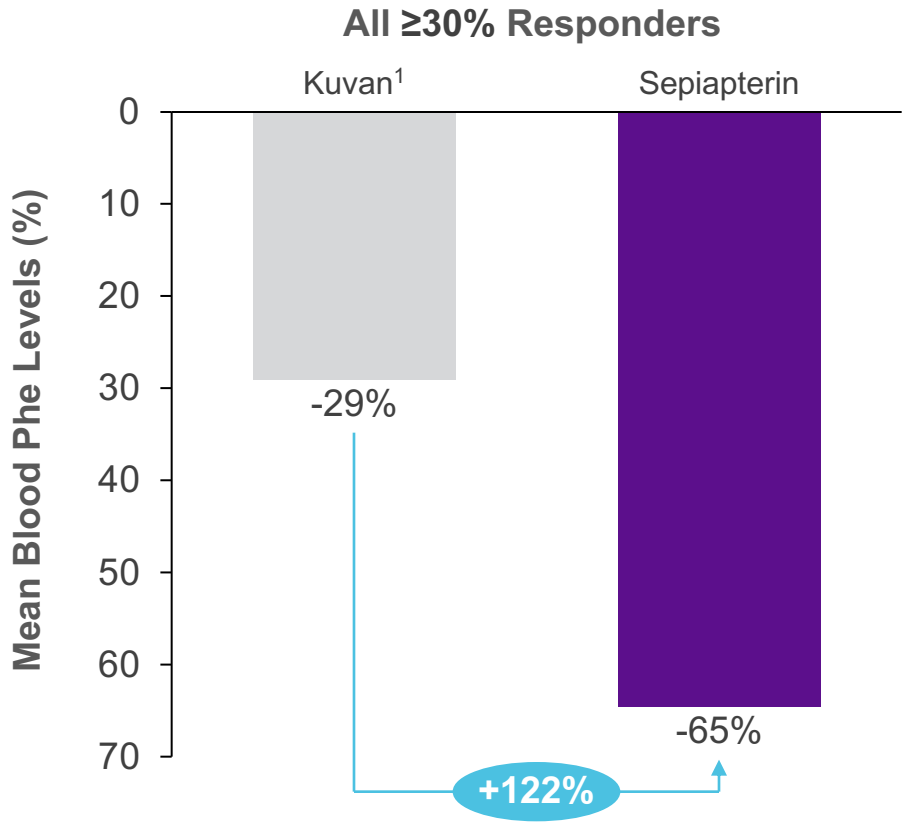
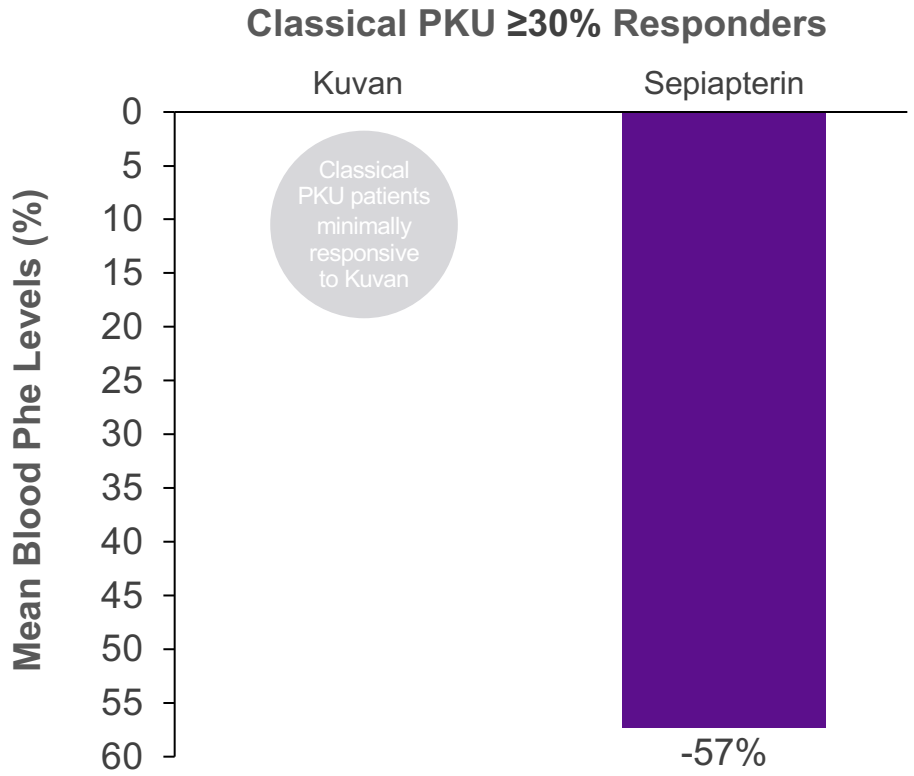
Part 1 is an open-label run-in phase to identify subjects to be randomized

The primary analysis population is those who have a ≥30% Phe reduction





1. KUVAN label (sapropterin dihydrochloride) - Accessdata.fda.gov

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Substantial Pipeline Progress Planned in 2023

Q1 **2023** | Q2 **2023** | Q3 **2023** | Q4 **2023**

MIT-E epilepsy

MOVE-FA

Part 1 PIVOT HD Part 2

SUNRISE LMS

CARDINALS

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Vatiquinone Has the Potential to Show Clinically Differentiated Improvement for MDAS Patients

Disease

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

Mechanism of Action

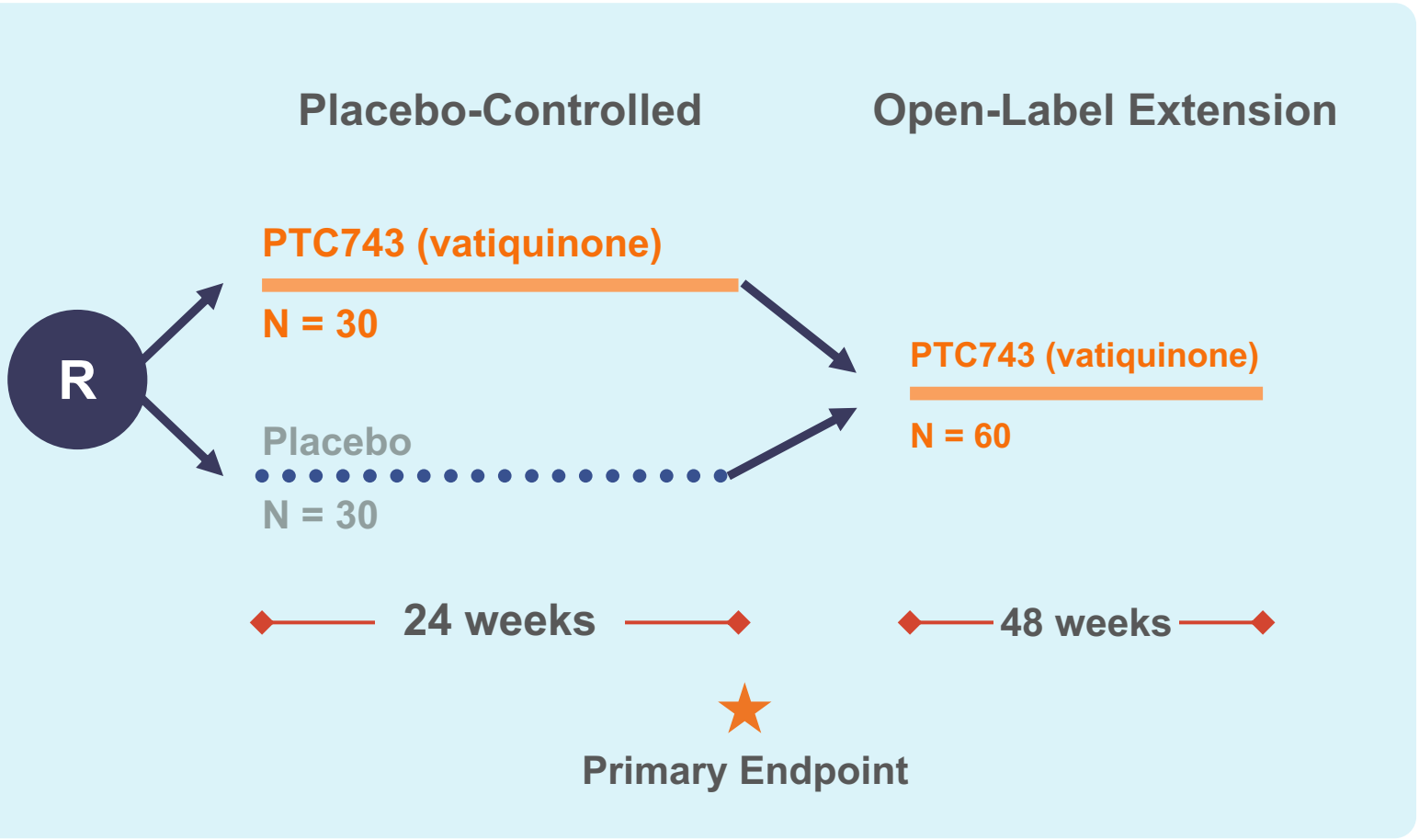
Vatiquinone targets 15-lipoxygenase, a regulator of the key energetic and oxidative stress pathways that underpin seizures in these patients

Current Treatments

No approved disease modifying treatments

~20,000 Global Prevalence

MIT-E is a Global Registration-Directed Trial of Vatiquinone for MDAS



Primary Endpoint

Change from baseline in frequency of observable motor seizures

Trial Status

- Enrollment completed
- Data expected 1Q 2023

Substantial Pipeline Progress Planned in 2023

Q1 **2023** | Q2 **2023** | Q3 **2023** | Q4 **2023**

aphenity

MOVE-FA

Part 1 | Part 2

SUNRISE LMS

CARDINALS

Vatiquinone Has the Potential to Provide Improvement in Neurological Function

MOVE-FA

Disease

Friedreich ataxia (FA)
is a rare, inherited, progressive disease resulting from mitochondrial dysfunction

Mechanism of Action

Vatiquinone targets 15-lipoxygenase, a regulator of key energetic and oxidative stress pathways that are disrupted in FA

Current Treatments

No approved disease modifying treatments

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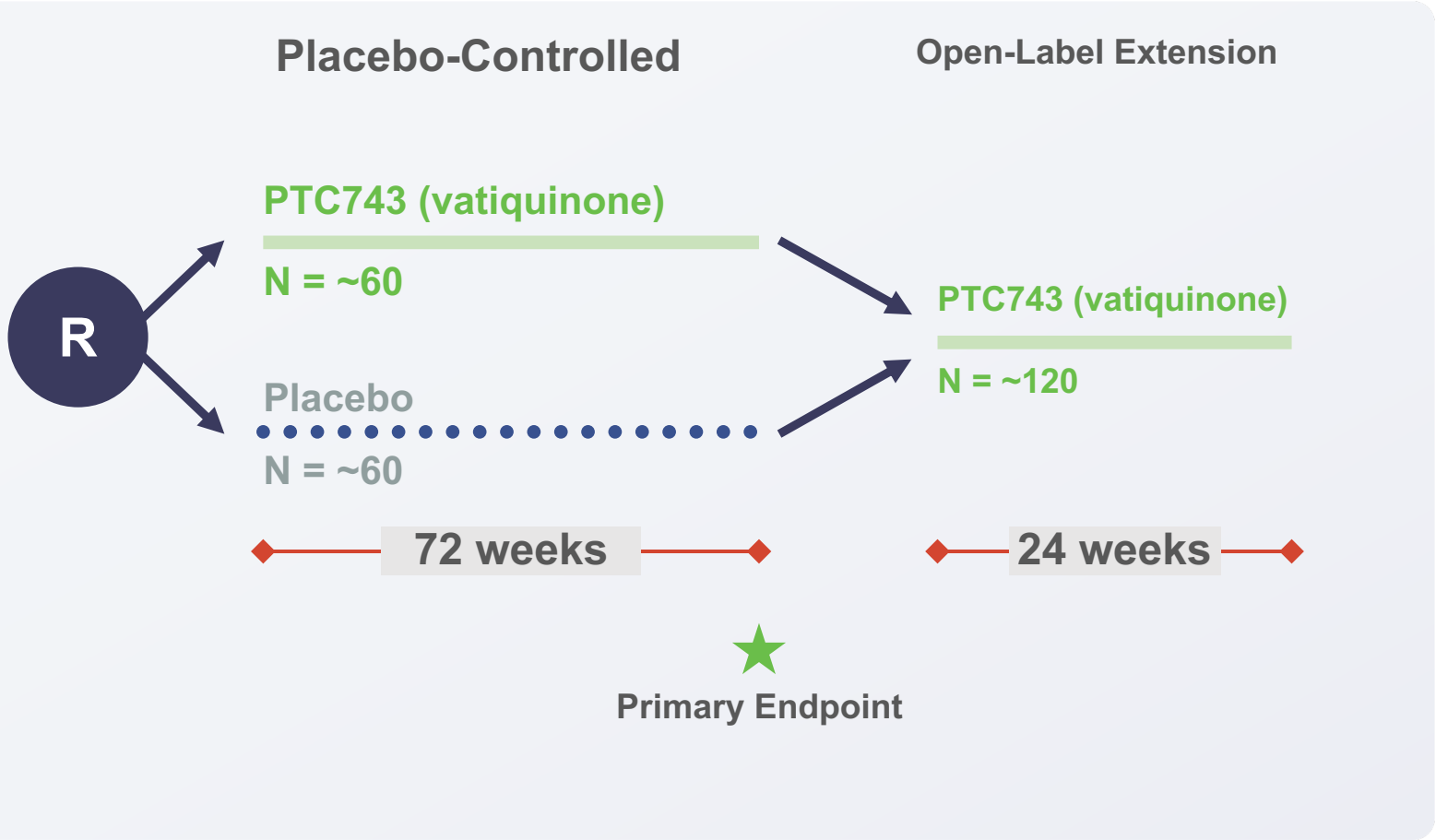
MOVE-FA is a Global Registration-Directed Trial of Vatiquinone for FA



Primary Endpoint

Change in mFARS

Key Secondary Endpoint

Change in FA-ADL

Trial Status

- ✓ Enrollment completed
- Data expected in 2Q 2023

Substantial Pipeline Progress Planned in 2023

Q1 **2023** | Q2 **2023** | Q3 **2023** | Q4 **2023**

aphenity

MIT-E

Part 1

PIVOT HD

Part 2

SUNRISELMS

CARDINALS

PTC518 Reduces HTT mRNA and Protein to Target the Underlying Cause of HD

Disease

Huntington's disease (HD) is a progressive brain disorder that causes uncontrolled movements and cognitive loss

Mechanism of Action

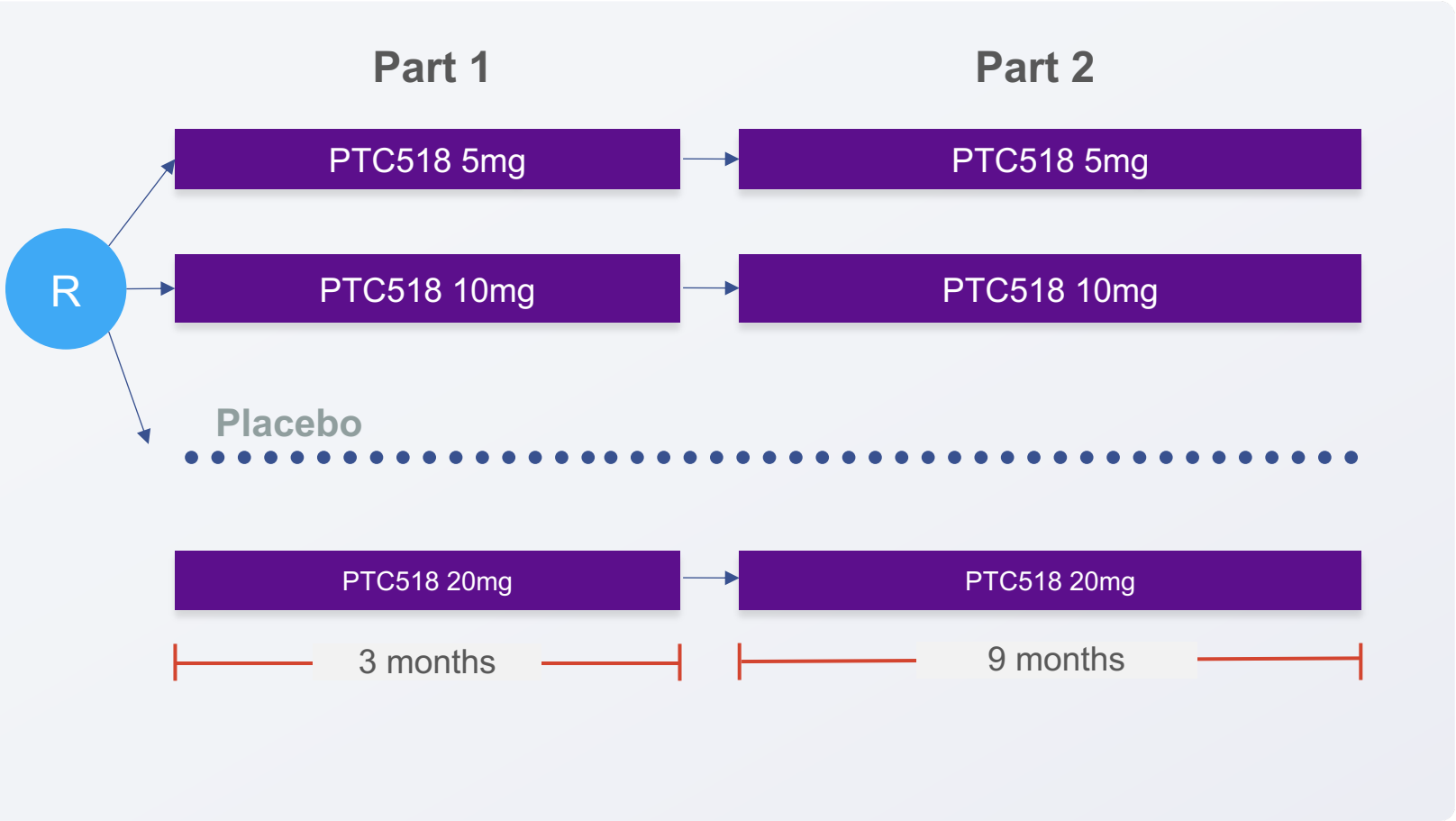
PTC518 modulates splicing to induce degradation of HTT mRNA, reducing expression of the toxic HTT protein

Current Treatments

No approved disease modifying treatments

~135,000 Global Prevalence

PIVOT-HD is a Global Phase 2 Trial of PTC518 for HD



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

PIVOT-HD Expanding Trial Target Population

Inclusion Criteria

Stage 2

- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 40-50 inclusive
- Motor and Cognitive Function:
  - UHDRS-IS score of **100**
  - UHDRS TFC score of **13**
- PIN_{HD} score **0.18 - 4.93**
  - Multivariate calculation including SDMT, TMS, age, CAG

Inclusion Criteria

Early Stage 3

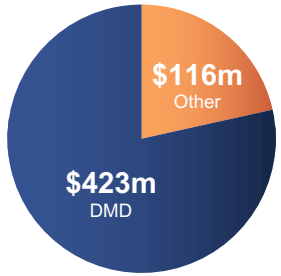
- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 40-50 inclusive
- Motor and Cognitive Function:
  - UHDRS-IS score of **less than 100**
  - UHDRS TFC score of **11 or 12**
- PIN_{HD} score **0.18 - 4.93**
  - Multivariate calculation including SDMT, TMS, age, CAG

Transformational Commercial Revenue in 2023

Commercial

2021

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

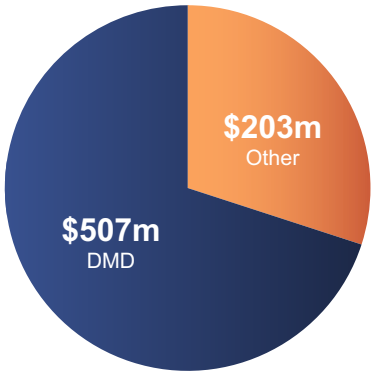


$539M

Total revenue

2022

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

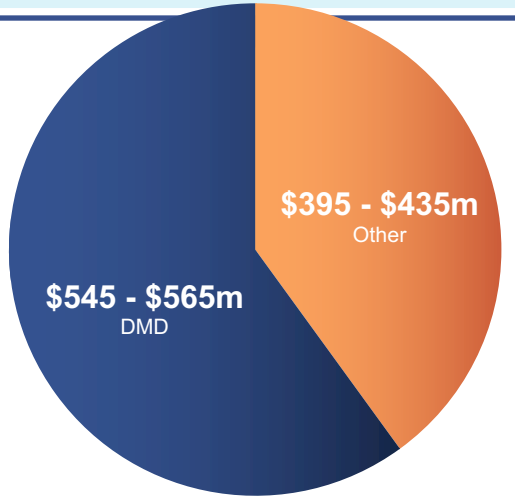


~$710M

Unaudited total revenue

2023

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



$940M-$1bn

Potential future revenues

Transformational Development Milestones in 2023

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Transformational Development Milestones in 2023

Q1 **2023** | Q2 **2023** | Q3 **2023** | Q4 **2023**

aphenity

MIT-E

MOVE-FA

Part 1 PIVOT HD Part 2

SUNRISELMS

CARDINALS

Upstaza BLA Submission

DIPG

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Matthew Klein, M.D., COO
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