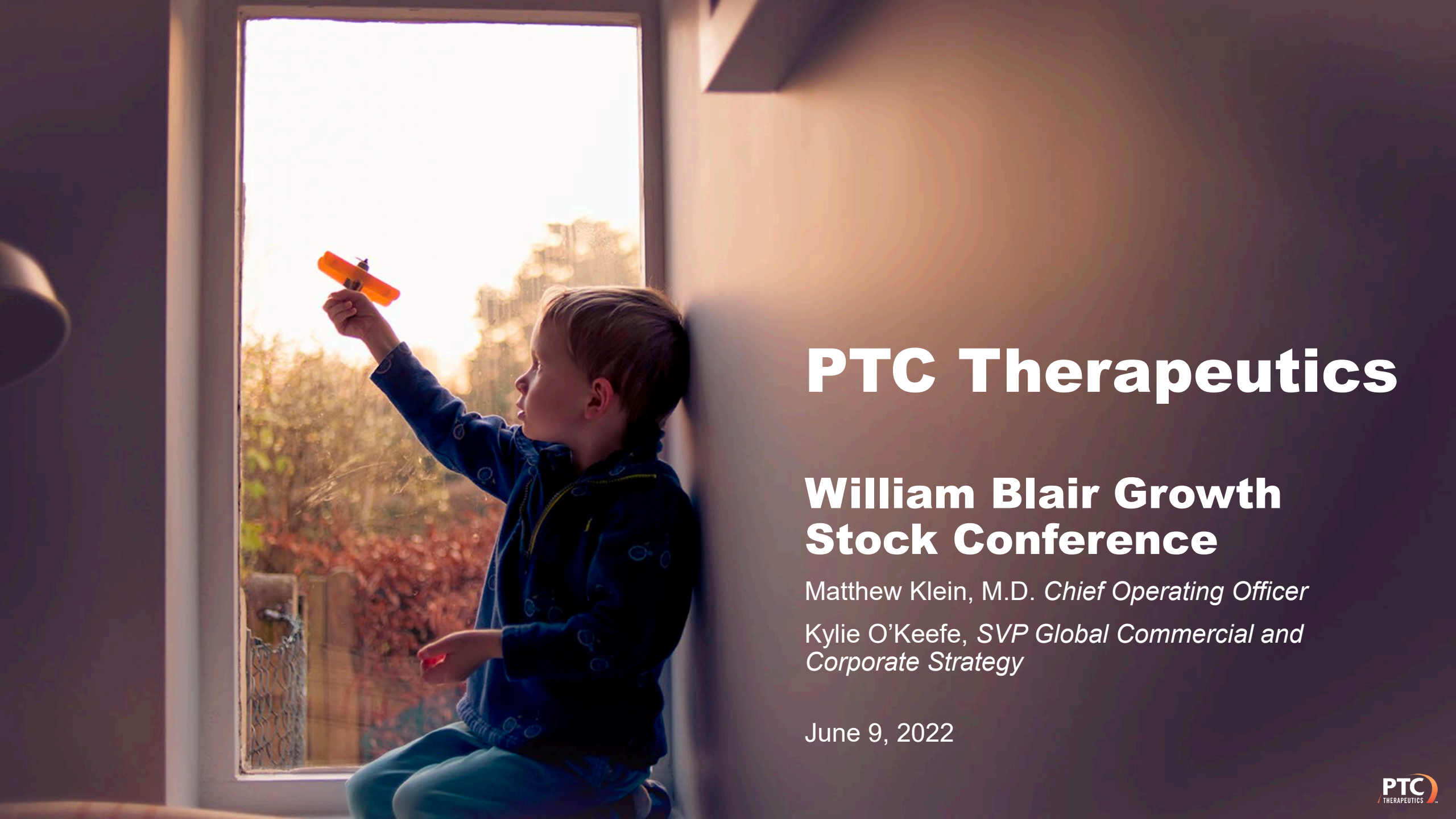


PTC Therapeutics

William Blair Growth Stock Conference

Matthew Klein, M.D. *Chief Operating Officer*

Kylie O'Keefe, *SVP Global Commercial and Corporate Strategy*

June 9, 2022

Forward Looking Statements

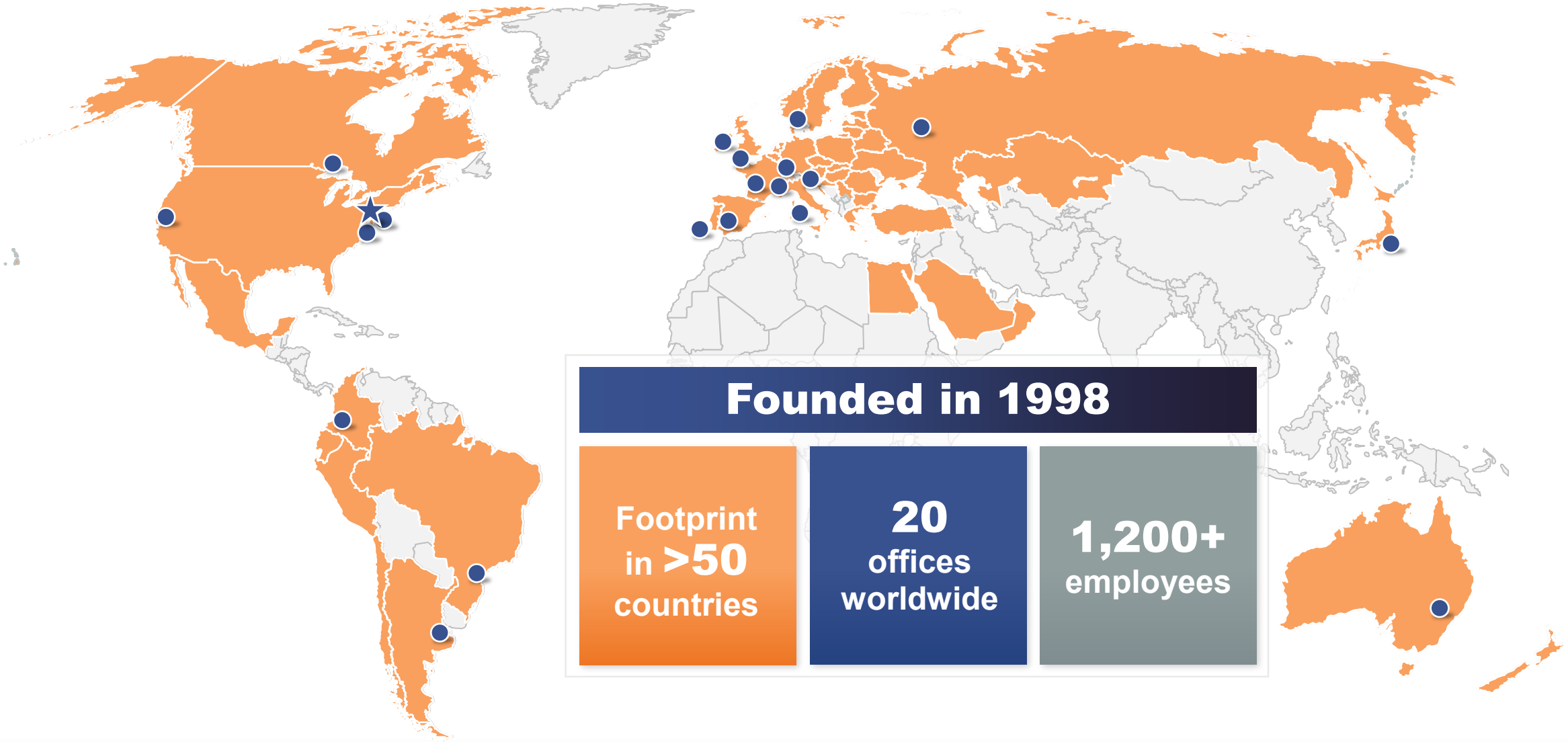
This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements with respect to guidance relating to 2022 total net product revenue, 2022 DMD franchise net product revenue, 2022 operating expenditure guidance and future revenue 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 approvals 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; 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 PTC's gene therapy platform, including any 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 regulatory submissions and commercialization with respect to Evrysdi; 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; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in 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 fund, complete and timely submit to the EMA the results of Study 041, 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 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 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 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 Upstaza.

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.

PTC Has a Growing Global Footprint



Founded in 1998

Footprint in >50 countries	20 offices worldwide	1,200+ employees

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

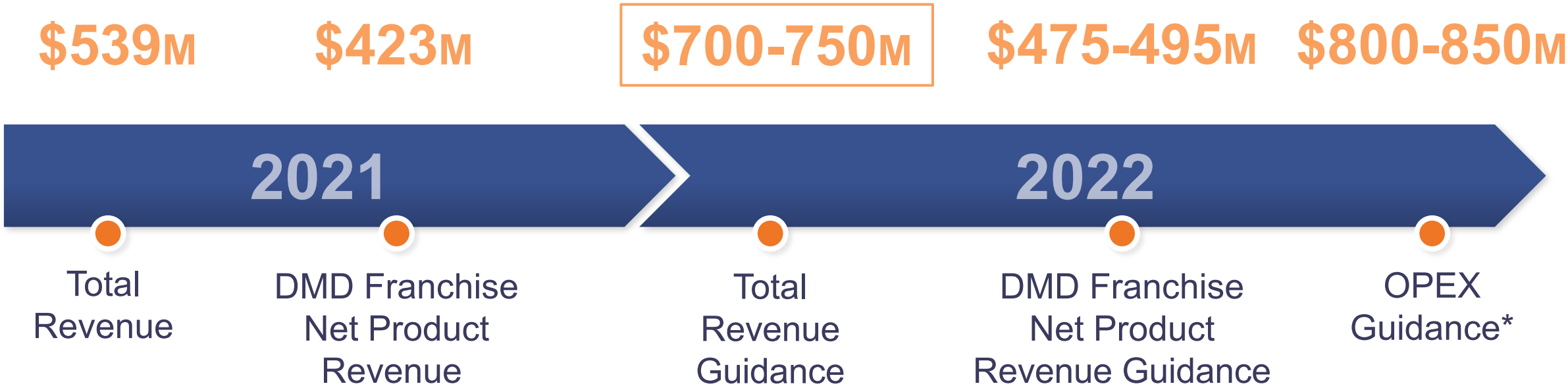
	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology	Virology
Commercial	Emflaza® (deflazacort) 6 mg | 18 mg | 30 mg | 36 mg tablets 22.75 mg/mL oral suspension	Tegsedi® (inotersen) injection 284 mg/1.5 mL; waylivra® (volanesorsen) injection 285 mg/1.5 mL	translarna™ ataluren	Evrysdi® risdiplam	Upstaza™* (eladocagene exuparvovec)				
Clinical			US Ataluren	PTC518 HD		Vatiquinone MDAS; Vatiquinone FA; PTC857 ALS	PTC923 PKU	Unesbulin DIPG; Unesbulin LMS; Emvododstat AML	Emvododstat COVID-19
Research			2 Undisclosed	SCA-3; MAP-Tau; 8 Undisclosed	FA; Angelman; IRDs; Cog Disorders	3 Undisclosed		3 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; ALS, amyotrophic lateral sclerosis; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3. * Positive CHMP opinion, EMA ratification expected in July

Potential registrational studies

Early-stage programs

Strong Financial Performance Driven by Innovation

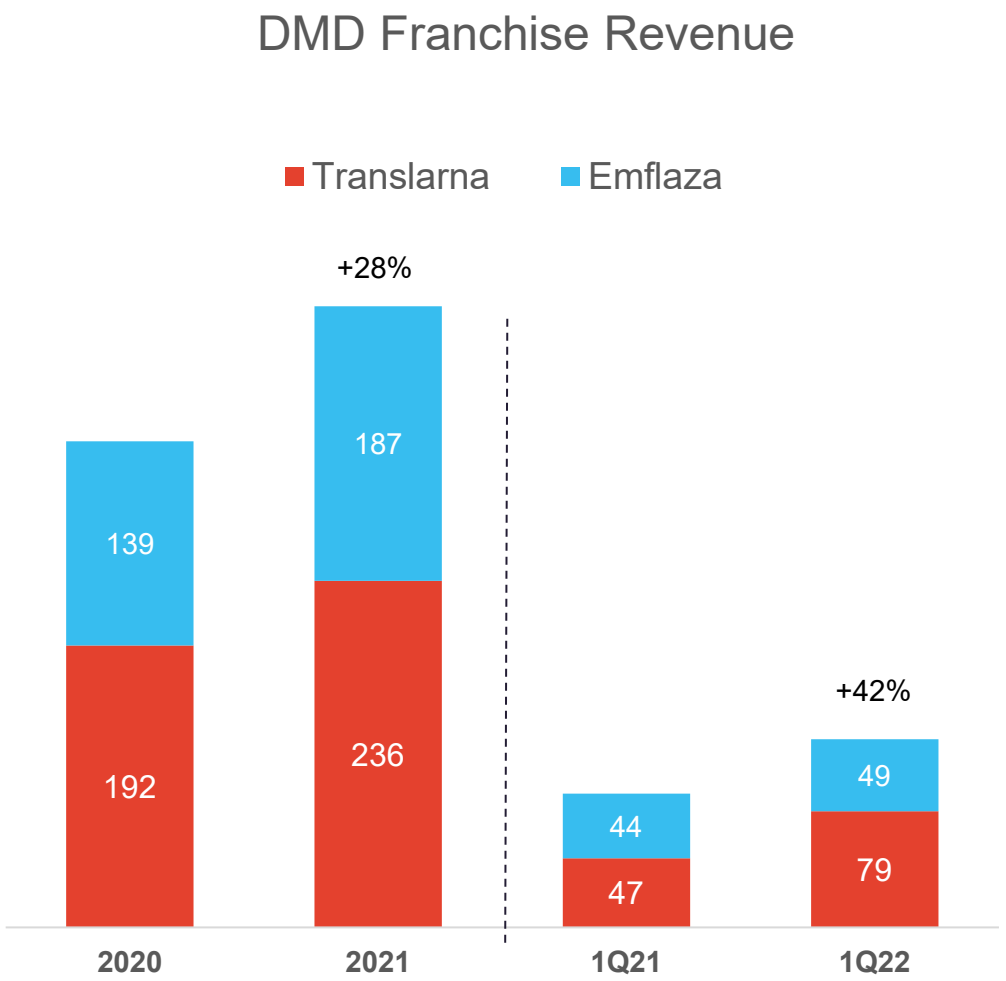


*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $115 million. GAAP R&D and SG&A expense for the full year 2022 is anticipated to be between $915 and $965 million.

Strong DMD Franchise Growth Expected to Continue

translarna™
ataluren

- Treatment for nonsense mutation DMD for ages 2 and older
- Distributed in 50+ countries
- New patients in existing geographies and geographic expansion



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

- First and only corticosteroid approved for DMD; approved for all US DMD patients >2yrs
- Data show clinical benefit over prednisone
- New patients starts, continued high compliance and operational excellence

Evrysdi Revenue Demonstrates Benefits of Safe and Effective Therapy for SMA

Evrysdi® risdiplam

Benefits of Evrysdi for SMA Patients

- First at-home, oral treatment for SMA
- Patients treated across all SMA types
- Patients are treatment-naïve or previously treated with Spinraza, Zolgensma
- Broadest range of ages treated

Newly Approved Indication in US for Pre-Symptomatic Infants

Based on interim data from the RAINBOWFISH study

Showed 80 percent of pre-symptomatic infants with SMA treated with Evrysdi for at least 12 months achieved motor milestones

Including sitting without support, rolling, crawling, standing unaided, and walking independently

PTC THERAPEUTICS

Leveraging Regulatory Expertise and Commercial Infrastructure to Support Patients in Latin America

Tegsedi® (inotersen) injection 284 mg/1.5 mL

- Innovative treatment for hATTR amyloidosis patients
- Disease awareness and patient ID continuing
- LATAM patients benefiting through early-access programs
- Received Category 1 pricing, in final pricing negotiations

Commercial efforts ongoing

Application submitted for additional indication of FPL

waylivra® (volanesorsen) injection 285 mg/1.5 mL

- For treatment of familial chylomicronaemia syndrome (FCS)
- LATAM patients benefiting through early-access programs
- Received Category 1 pricing, in final pricing negotiations

Upstaza™ 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 approved disease-modifying treatments

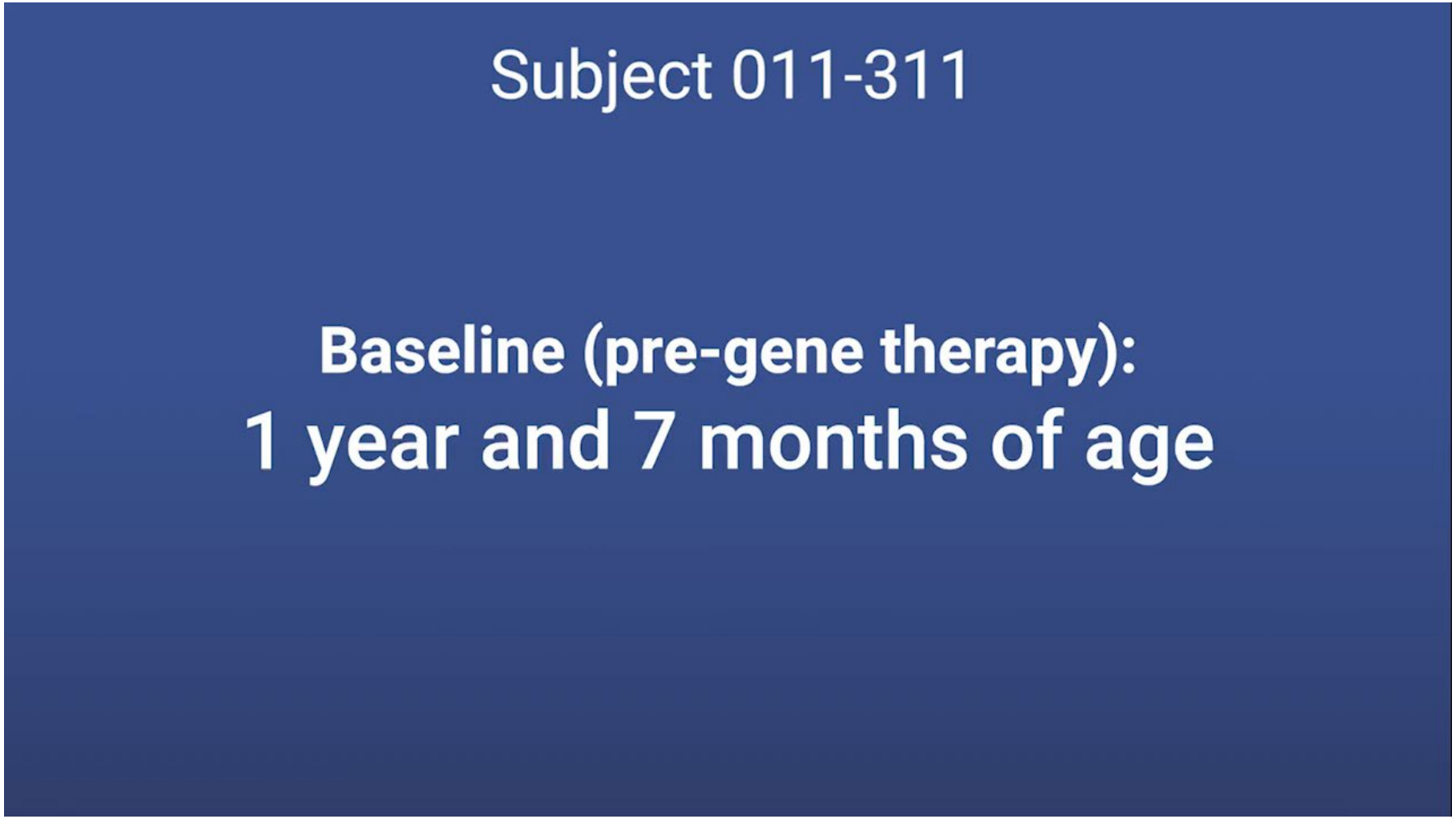
Mechanism of Action

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

~5,000
Global Prevalence

Upstaza™ -Treated Patients Make Significant and Sustainable Progress

Upstaza™
(eladocagene exuparvovec)



Upstaza™ Gene Therapy Opportunity and Launch Preparation

Upstaza™
(eladocagene exuparvovec)

Regulatory

Positive CHMP opinion received in May, ratification expected in July

Upstaza™ BLA submission expected in 4Q22

Disease Education

Development of virtual education: disease-specific webinars and congress symposia

Engaging with patient advocacy groups and payers

Treatment Centers

Identification and preparation of expert pediatric neurosurgical centers

Continued KOL engagement

Market Opportunity

Potential over $1B in cumulative revenue

Successful patient finding is ongoing

PTC THERAPEUTICS

Pipeline Progress Planned in 2022

Study Initiations

PIVOT-HD

CARDINALS

SUNRISE-LMS

DIPG Ph2 Initiation

GT-FA Ph1 Initiation

2022 **Q1** | 2022 **Q2** | 2022 **Q3** | 2022 **Q4**

FITE19

translarna ataluren Study 041

MIT-E

aphenity

MOVE-FA

Study Results

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

Commercial

Clinical

Research

Deflazacort

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

LatAm

Nonsense

Gene ...erapy

8 Undisclosed

IRDs

Cog Disorders

...elman

Bio-e

Vatiquinone MDAS

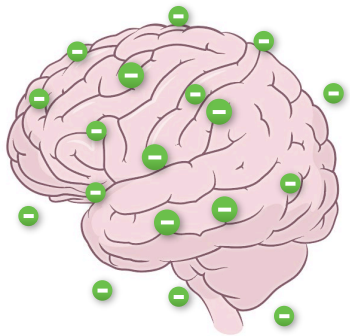
Vatiquinone FA

PTC857 ALS

3 Undisclosed

Metab...

Science of Bio-e



- Differentiated therapy for neurological indications
- High levels of oxidative stress and inflammation underpin multiple CNS disease pathologies
- Bio-e platform targets the key enzymatic hubs that regulate oxidative stress and inflammatory pathways to modulate disease progression

Reducing Oxidative Stress

Inhibition of 15 lipoxygenase (15-LO) blocks inflammation and oxidative stress response

- Small Molecule
- Orally Bioavailable
- Highly Selective
- Defined Redox Activity
- Catalytically recycled
- Low nanomolar potency
- Scalable manufacturing

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; MDAS, mitochondrial disease associated seizures; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

Potential registrational studies

Early-stage programs

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

Current Treatments

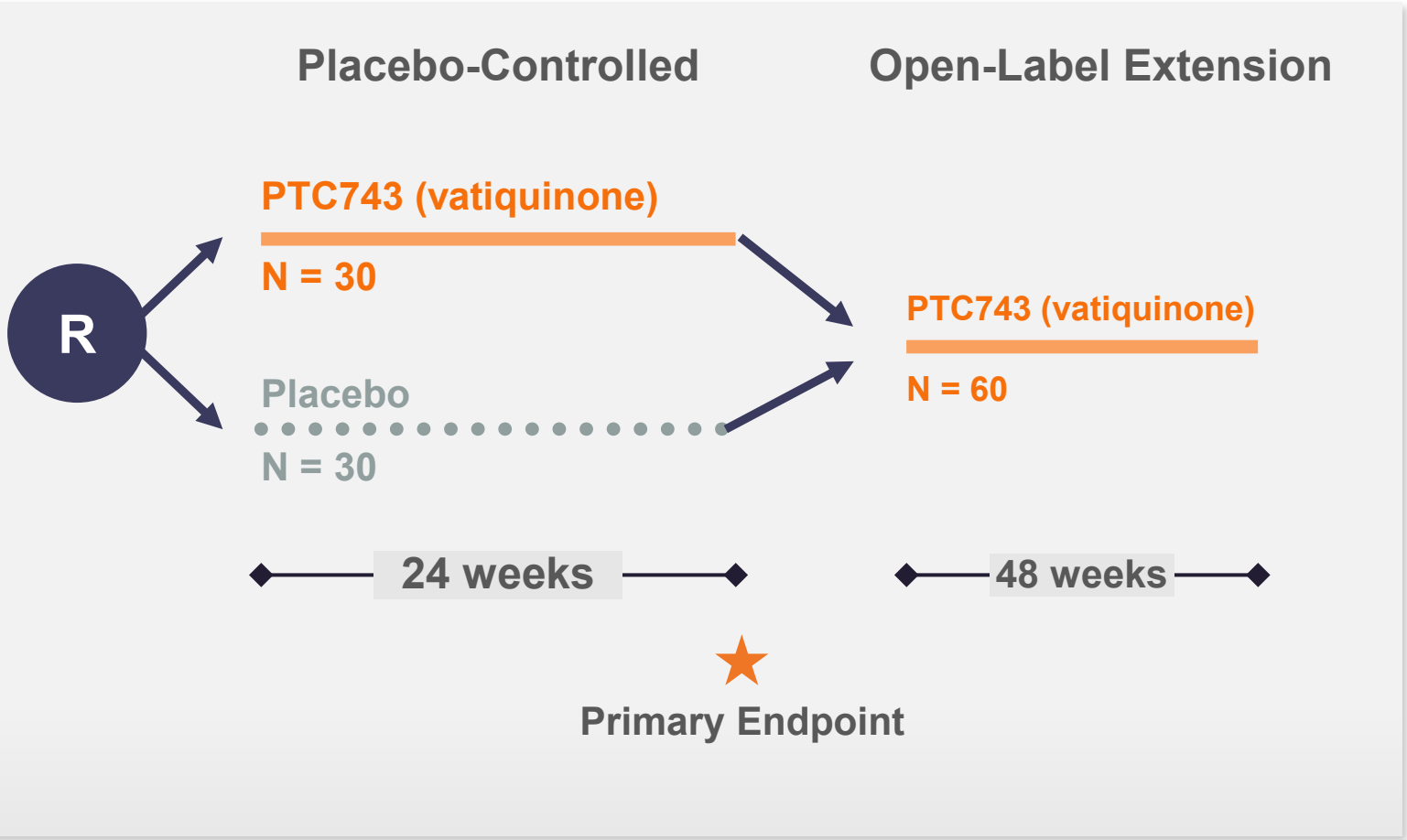
No approved disease-modifying treatments

~20,000
Global Prevalence

Mechanism of Action

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

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



Primary Endpoint

Change from baseline in frequency of observable motor seizures

Trial Status

- Enrolling
- Data expected 4Q 2022

Vatiquinone Has the Potential to Provide Improvement in Neurological Function

Disease

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

~25,000 Global Prevalence

Current Treatments

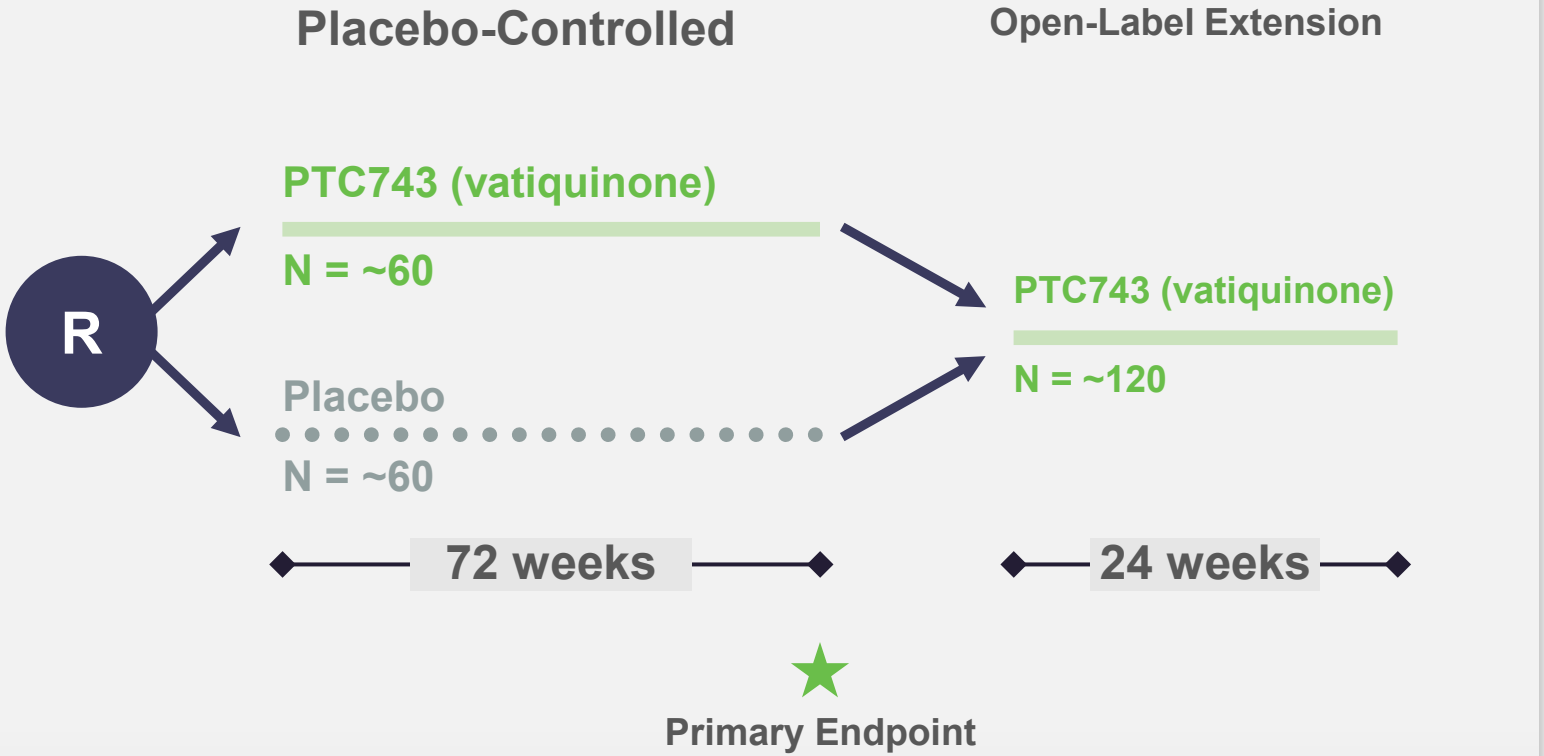
No approved disease-modifying therapies

Opportunity

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

Vatiquinone Has the Potential to Provide Improvement in Neurological Function

MOVE-FA



Primary Endpoint
Change in mFARS

Key Secondary Endpoint
Change in FA-ADL

Trial Status

✓ Enrollment complete

- Data expected in 2Q 2023

PTC857 Has the Potential to Slow Disease Progression in ALS

Disease

Amyotrophic lateral sclerosis (ALS) is a rapidly progressing neurodegenerative disease caused by oxidative damage, which leads to neuronal cell death and muscular atrophy

Current Treatments

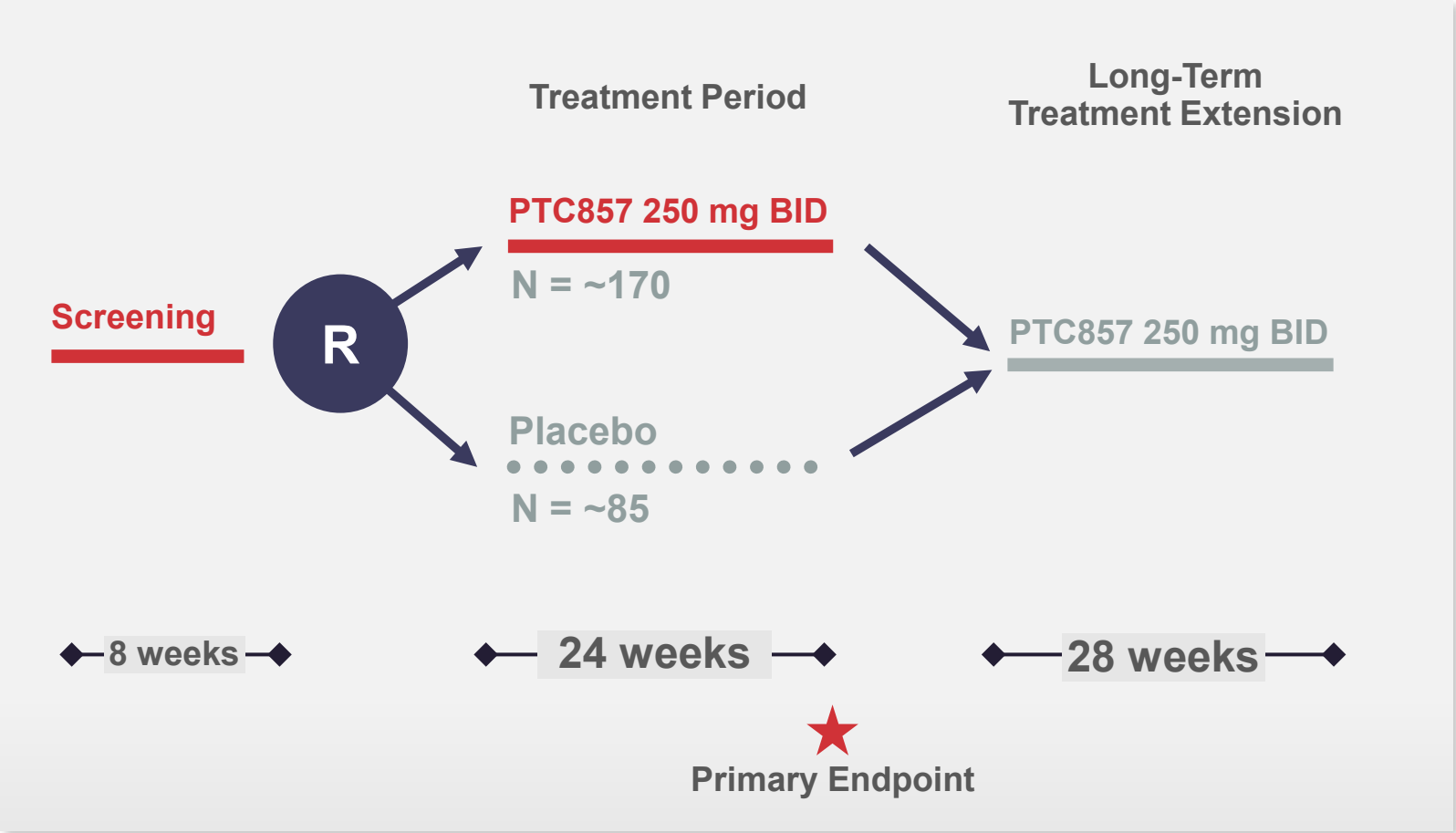
No approved disease-modifying therapies

Mechanism of Action

PTC857 inhibits pathways leading to oxidative damage and ferroptosis, resulting in protection of motor neurons

~150,000 Global Prevalence

PTC857 Has the Potential to Slow Disease Progression in ALS



Primary Endpoints
Change in ALSFRS-R

Secondary Endpoints
Safety and PK

Trial Status

- Enrolling
- Trial initiated in Q1 2022

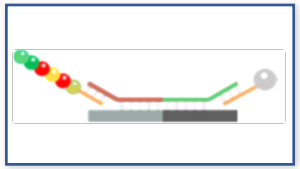
Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

LatAm | Nonsense | Splicing | Gene Thera

Leaders in Splicing Technology

Databases of Splicing Targets



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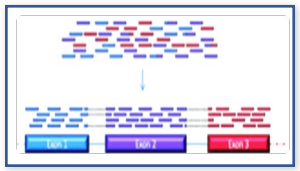


HTSpliceseq

Evrysdi® risdiplam

PTC518 HD

SCA-3

MAP-Tau

8 Undisclosed

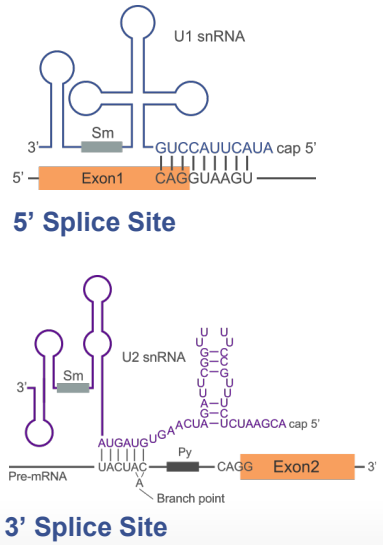
IRDs

Cog Disorders

Research

Splicing

- Pioneers in splicing
- Small-molecule regulation of splicing events
- Chemistry optimized for uniform distribution, blood-brain barrier penetration and limited efflux



5' Splice Site

3' Splice Site

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; MDAS, mitochondrial disease associated seizures; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

Potential registrational studies

Early-stage programs

PTC518 Reduces HTT mRNA and Protein to Target HD

Disease

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

Current Treatments

No approved disease-modifying therapies

Mechanism of Action

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

~135,000
Global Prevalence

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

Generally well tolerated

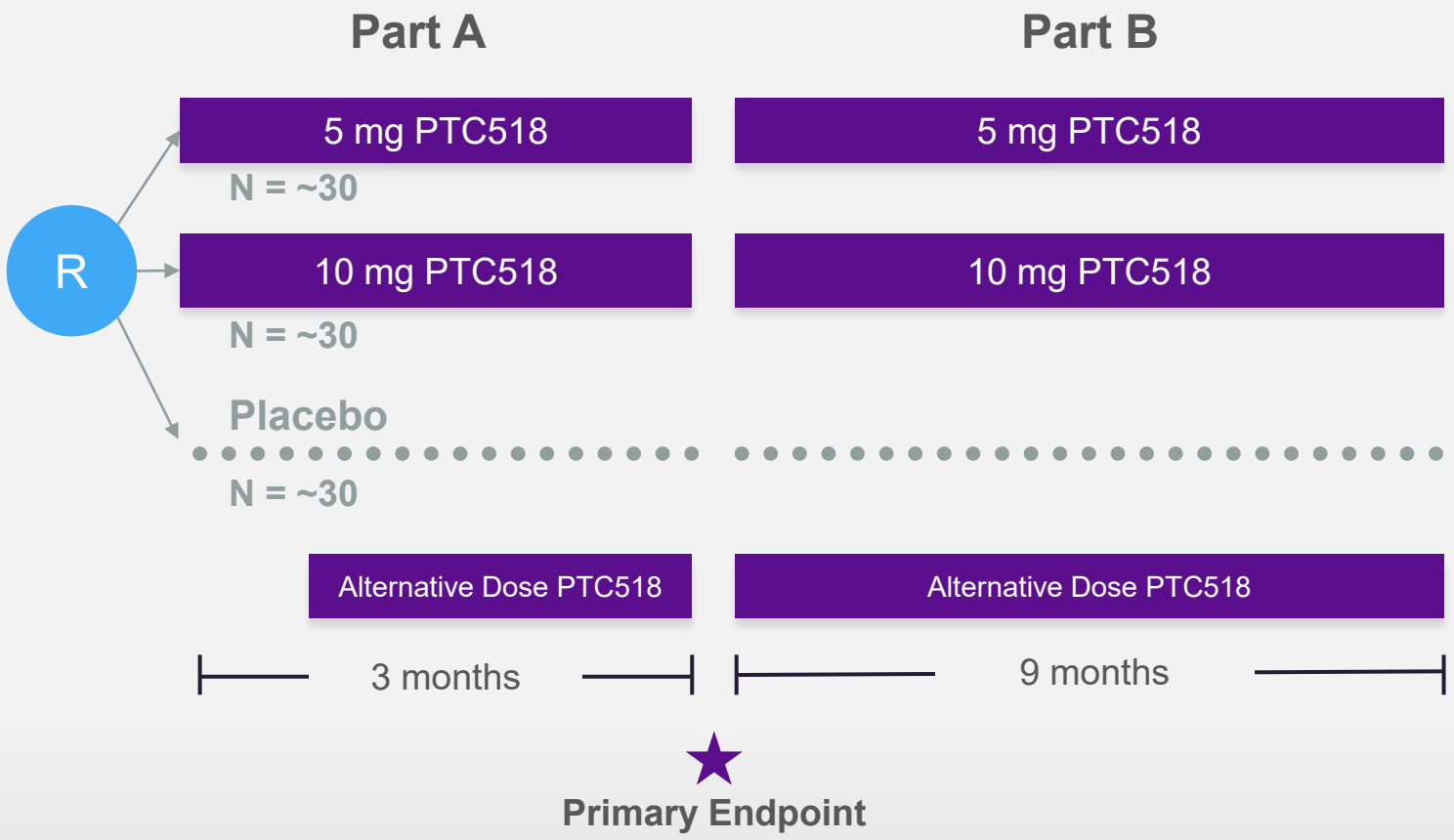
Consistent pharmacology

Dose-dependent reduction of HTT mRNA and protein

Crosses blood-brain barrier and is not effluxed

Results from Phase 1 Healthy Volunteer Study

PTC518 Has the Potential to Reduce HTT Protein in Huntington Patients



Primary Endpoints

- Safety and tolerability of PTC518 in Huntington disease patients
- Percent reduction in HTT mRNA and protein in blood

Trial Status

- Enrolling
- Trial initiated in Q1 2022

PTC518 Has the Potential to Reduce HTT Protein in Huntington Patients

Inclusion Criteria

- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 42-50 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

Primary endpoints

- Safety and tolerability of PTC518 in Huntington's disease patients
- Percent reduction in HTT mRNA and protein in blood

Secondary endpoints

- Percent reduction in HTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in caudate, putamenal, ventricular volume on volumetric MRI imaging
- Changes in clinical scales of motor and cognitive function

APHENITY Is a Global Registration-Directed Trial of PTC923 for PKU

Disease

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

Current Treatments

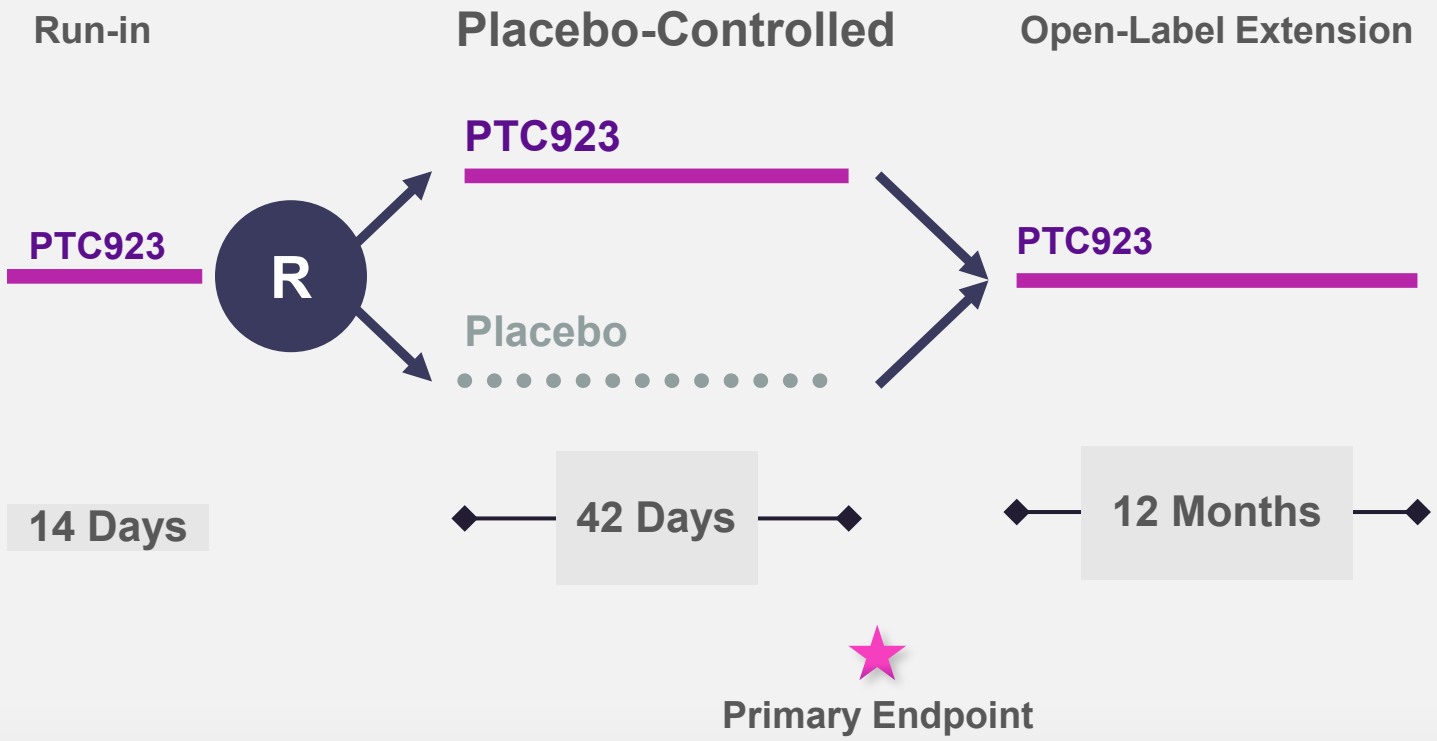
Majority of patients do not initially respond or are not well controlled by standard of care

Mechanism of Action

PTC923 is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat a broader range of PKU patients

~58,000 Global Prevalence

APHENITY Is a Global Registration-Directed Trial of PTC923 for PKU



Primary Endpoint

Reduction in blood phenylalanine levels

Trial Status

- Enrolling
- Data expected YE 2022

Unesbulin Has the Opportunity to Provide Additional Progression Free Survival in LMS

Disease

Leiomyosarcoma (LMS) is a rare and aggressive cancer with tumors found in smooth muscle

Current Treatments

Several chemotherapeutics are utilized but offer minimal meaningful clinical benefit

Mechanism of Action

Unesbulin is an oral small molecule tubulin inhibitor that arrests tumor cells, including cancer stem cells, in G2/M phase by inhibiting tubulin polymerization

~4,000 diagnosed annually in US

Not an actual LMS patient.

Unesbulin Has the Opportunity to Provide Additional Progression Free Survival in LMS

21 Day Treatment Cycles

Phase 1b Study Design

Ascending doses 200, 300 and 400 mg unesbulin + 1000mg/m^2 dacarbazine

N = 29

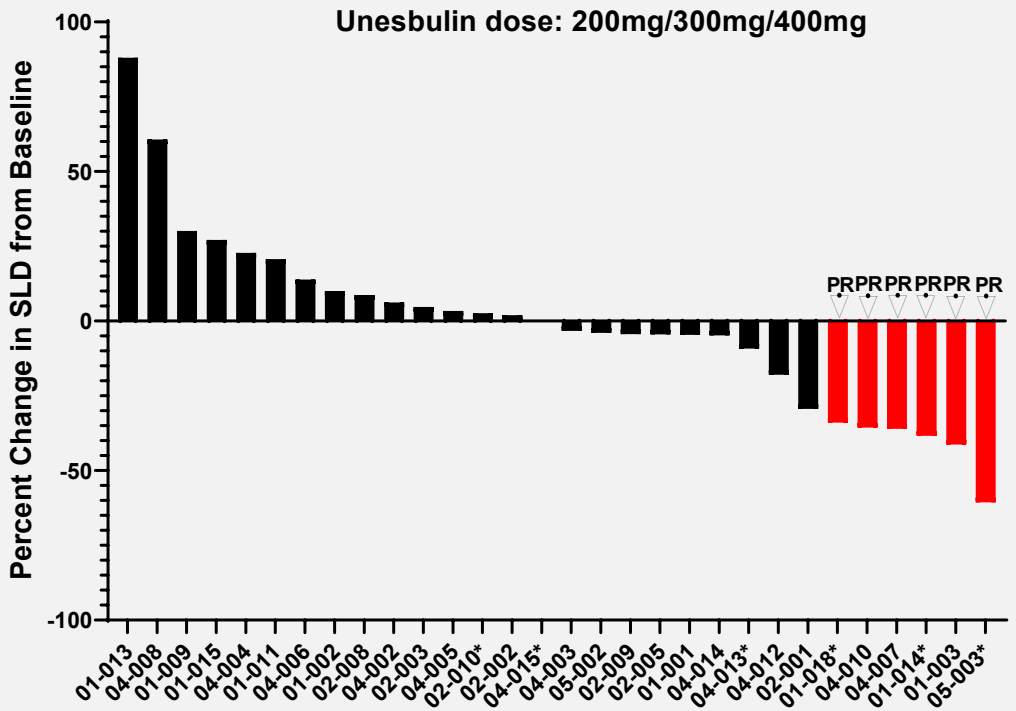
Inclusion Criteria

- Patients with locally advanced or metastatic LMS intolerant or refractory to standard therapy
- Any number of previous lines of treatments allowed

Phase 1b study results

- 300 mg was selected as RP2D
- Unesbulin was well tolerated

Percentage Change for SLD from Baseline

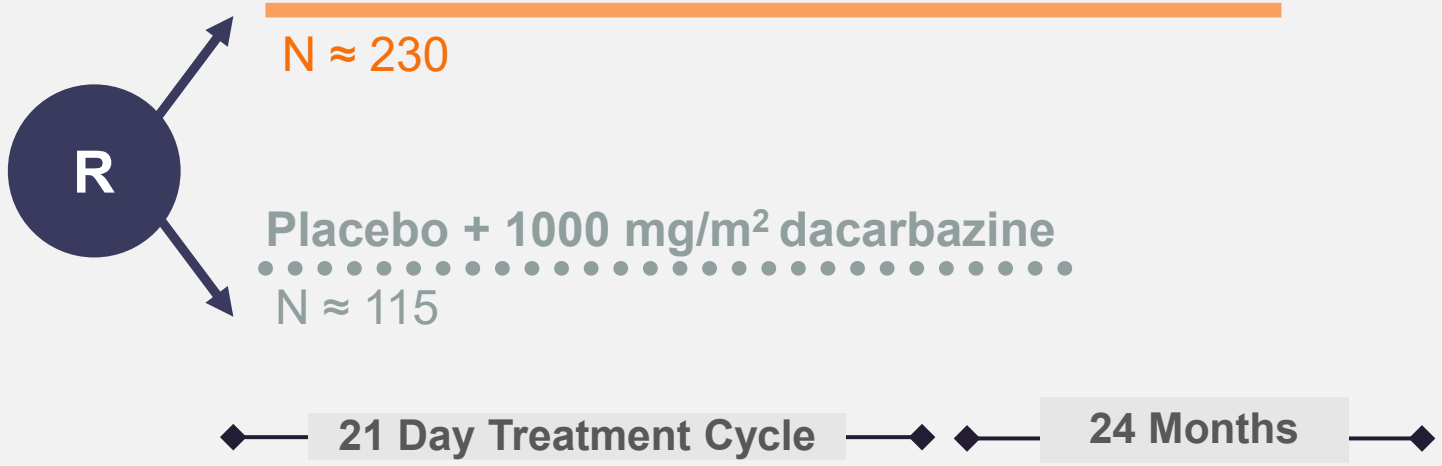


* Active subjects who had at least 1 response assessment

SLD = sum of largest diameter

Unesbulin has Opportunity to Provide Additional Progression Free Survival in LMS

SUNRISELMS



Primary Endpoint
PFS as determined by RECIST

Secondary Endpoints
OS, ORR, DCR, DOR

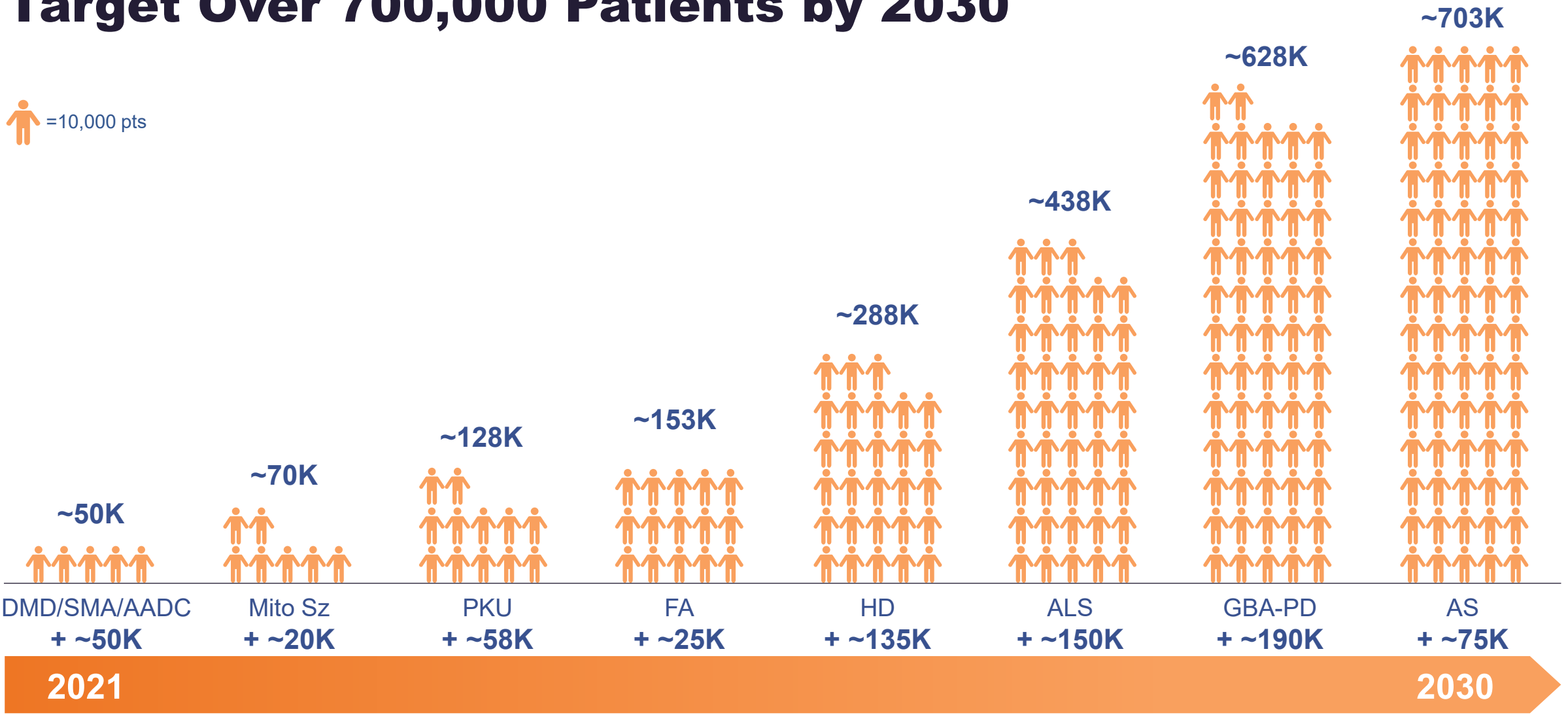
Interim Analysis

Trial Status

- Enrolling
- Initiated in Q1 2022

PTC THERAPEUTICS

Multiple Platforms Provide Opportunity to Target Over 700,000 Patients by 2030



Estimated Global Prevalence

Enduring Innovation Drives Value Creation

Commercial

2021

2026

Key Drivers: Evrysdi®, Upstaza™, Tegsedi®, Waylivra®, Translarna™, Vatiquinone, PTC923, Unesbulin

2030

Key Drivers: Evrysdi®, PTC-AADC, Tegsedi®, Waylivra®, Vatiquinone, PTC923, Unesbulin, PTC518, PTC857

~57% of revenues from new products

Total revenue

Clinical

Vatiquinone | PTC923 | PTC518 | PTC857 | Unesbulin | Emvododstat | GT-FA | GT-AS | Research