

PTC Therapeutics

May 2026



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 with respect to 2026 total product revenue guidance and total revenue guidance and 2026 operating expense 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, meetings with regulatory agencies, commercialization and other matters with respect to 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," "aim," 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 Saphience, including any regulatory submissions and potential approvals, commercialization, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in geographies in which it has been approved and the effect of the European Commission's adoption of the negative opinion from the Committee for Medicinal Products for Human Use (CHMP) on Translarna and the withdrawal of the Translarna NDA in the U.S. on other regulatory bodies; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation for voptoplam for the treatment of Huntington's disease including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis, the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; expectations with respect to Upstaza/Kebilidi, including commercialization, manufacturing capabilities, and the potential achievement of sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to vatiquinone, including with respect to the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses and potential approvals and other matters; expectations with respect to the commercialization of Evrysdi under PTC's SMA collaboration; expectations with respect to the commercialization of Tegsedi and Waylivra; 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; 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 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 Saphience, Translarna, Emflaza, Upstaza, Kebilidi, 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.

2025: A Year of Focus and Execution



US, EU & Japan
approvals of
Sephience



Strong start to
Sephience
global launch



Drive revenue
and effectively
manage OpEx



Advancement
of early-stage
R&D programs

2025

2025 Revenue Performance Exceeded Guidance

\$831M



Product Revenue \$587M

Sephience Launch Continues Strong Momentum



\$125M
Q1 2026 global revenue

\$112M US
\$13M ex-US

1,244 patients
on commercial
therapy worldwide*

* As of March 31, 2026

2026 Revenue and OpEx Guidance

Product Revenue¹

\$750 - 850M

28 - 45% YoY Growth

Total Revenue²

\$1.08 - 1.18B

OpEx³

\$680 - 720M

¹ Product revenue excludes Evrysdi royalty and collaboration revenues; YoY based on \$750-850M guidance.

² Total revenue includes Evrysdi royalty and collaboration revenues.

³ OpEx is a Non-GAAP measure that includes only R&D and SG&A expenses for full year 2026, and excludes estimated non-cash, stock-based compensation expense of approximately \$95M. GAAP R&D and SG&A expense for full year 2026 is anticipated to be between \$775 and \$815M.

2026: Planning for Continued Success

Continue
Sephience
global launch
momentum

Advance
Phase 3
votoplam HD
program

Advance
early-stage
R&D programs

Move toward
reaching cash
flow breakeven

2026

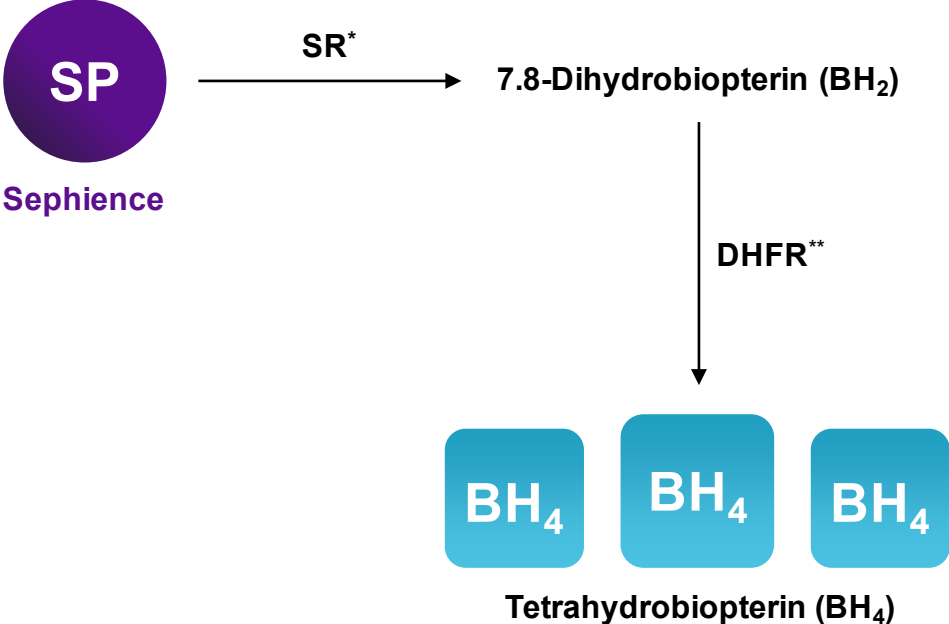
Sephience™ PKU Program



Katie, living w ith PKU

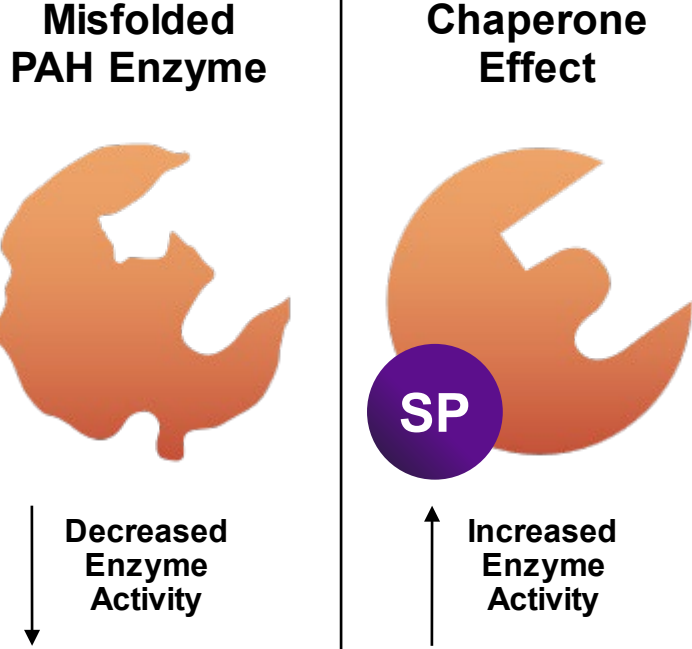
Sephience Dual Mechanism of Action Supports Potential for Broad Uptake

1. Precursor to BH₄



Sephience significantly increases intracellular BH₄ levels

2. Independent Chaperone Function



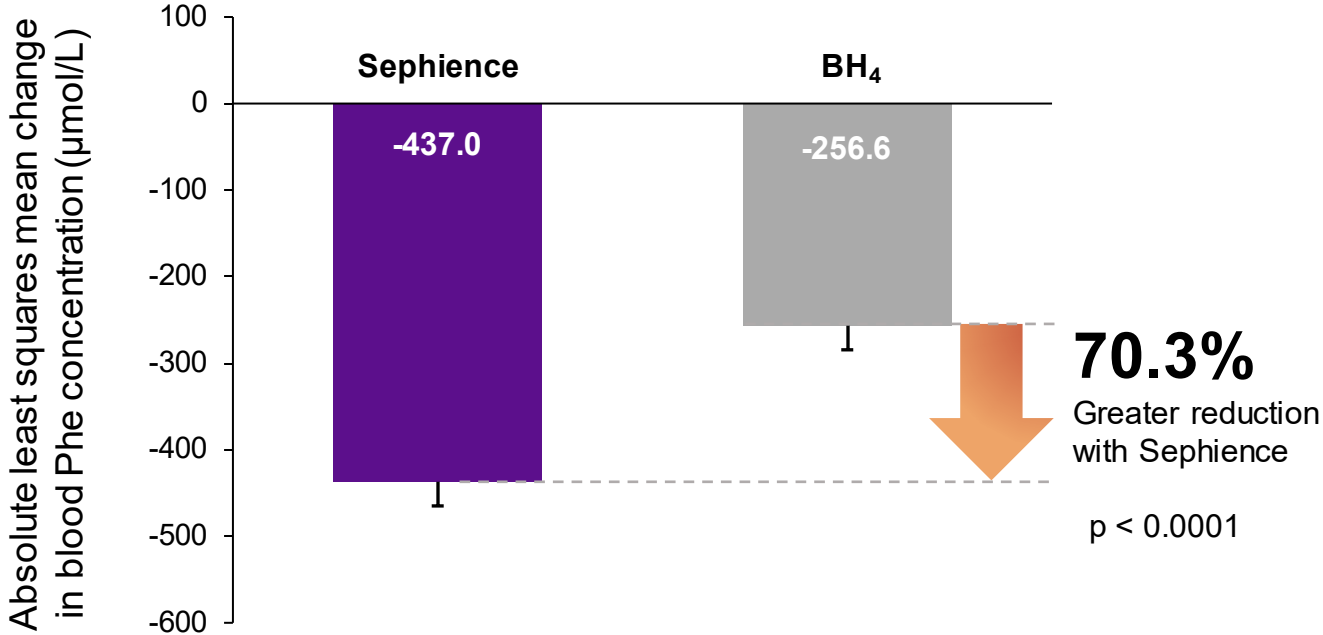
Chaperone effect stabilizes enzyme, increases function, and enables treatment of BH₄-non-responsive mutations

* SR, sepiapterin reductase (enzyme that converts Sephience to BH₂)
** DHFR, dihydrofolate reductase (enzyme that converts BH₂ to BH₄)
Lah, M. Advances in Therapy (In press)

Sephience Provides Significantly Greater Phe Reduction Compared With BH₄



Mean Absolute Change in Blood Phe from Baseline



AMPLIPHY was a crossover study allowing for intra-subject comparison of Sephience and BH₄ treatment effect

Sephience Provides Diet Liberalization, Cognitive and Quality of Life Benefits

Meaningful diet liberalization

69% of subjects reached age-adjusted protein RDA

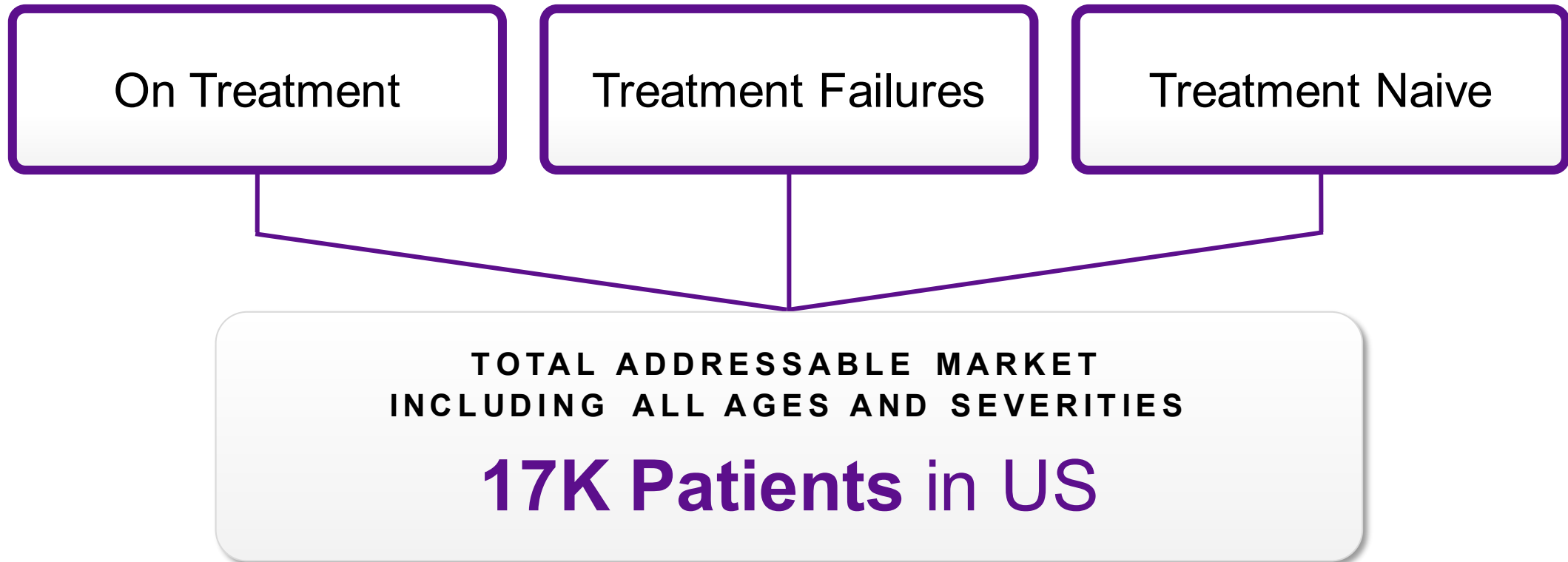
Improved cognitive function and mood

Improved concentration & slow thinking, and decreased irritability

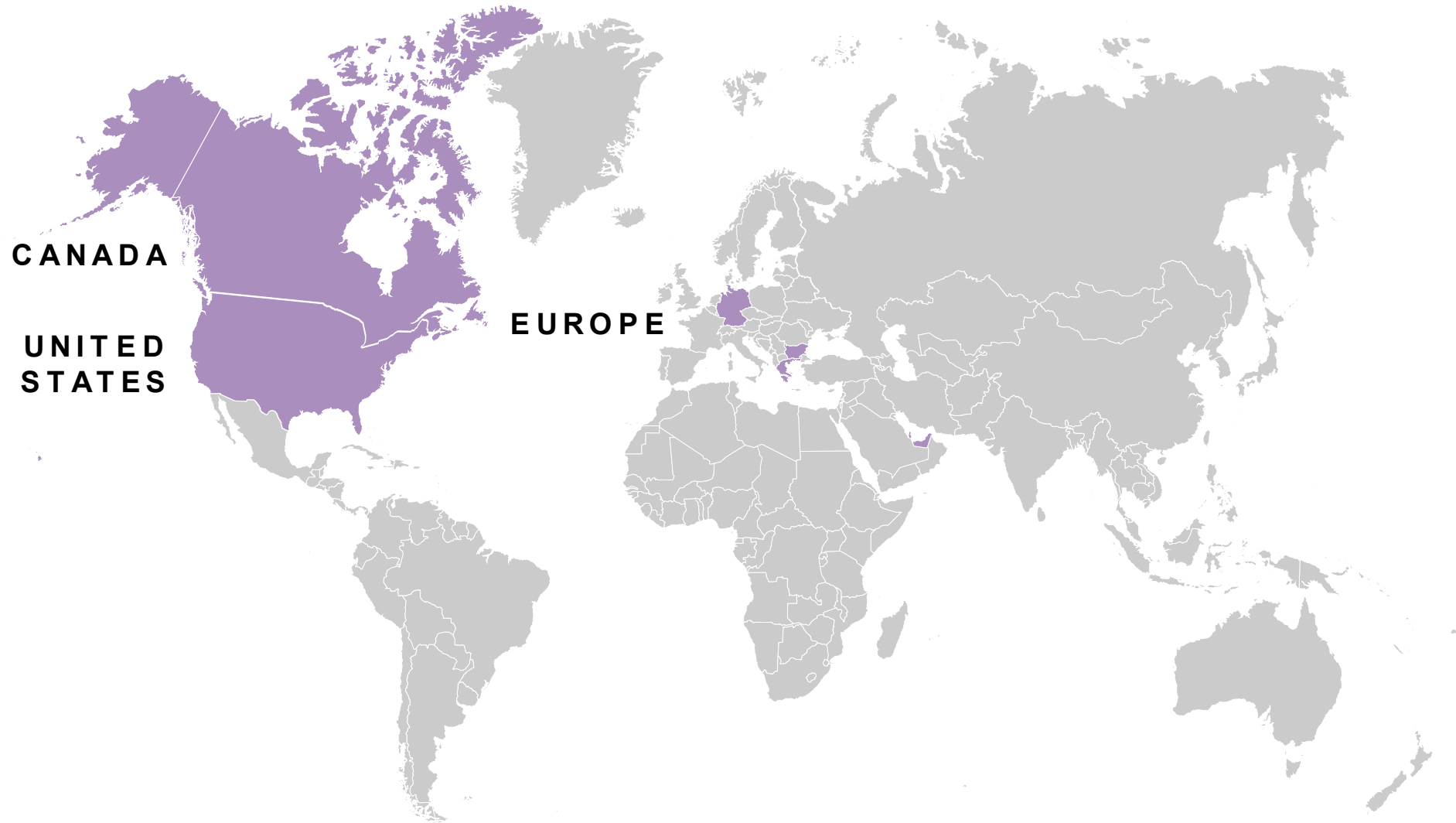
Improved quality of life

Reduced disease impact on emotional, social, and familial wellbeing

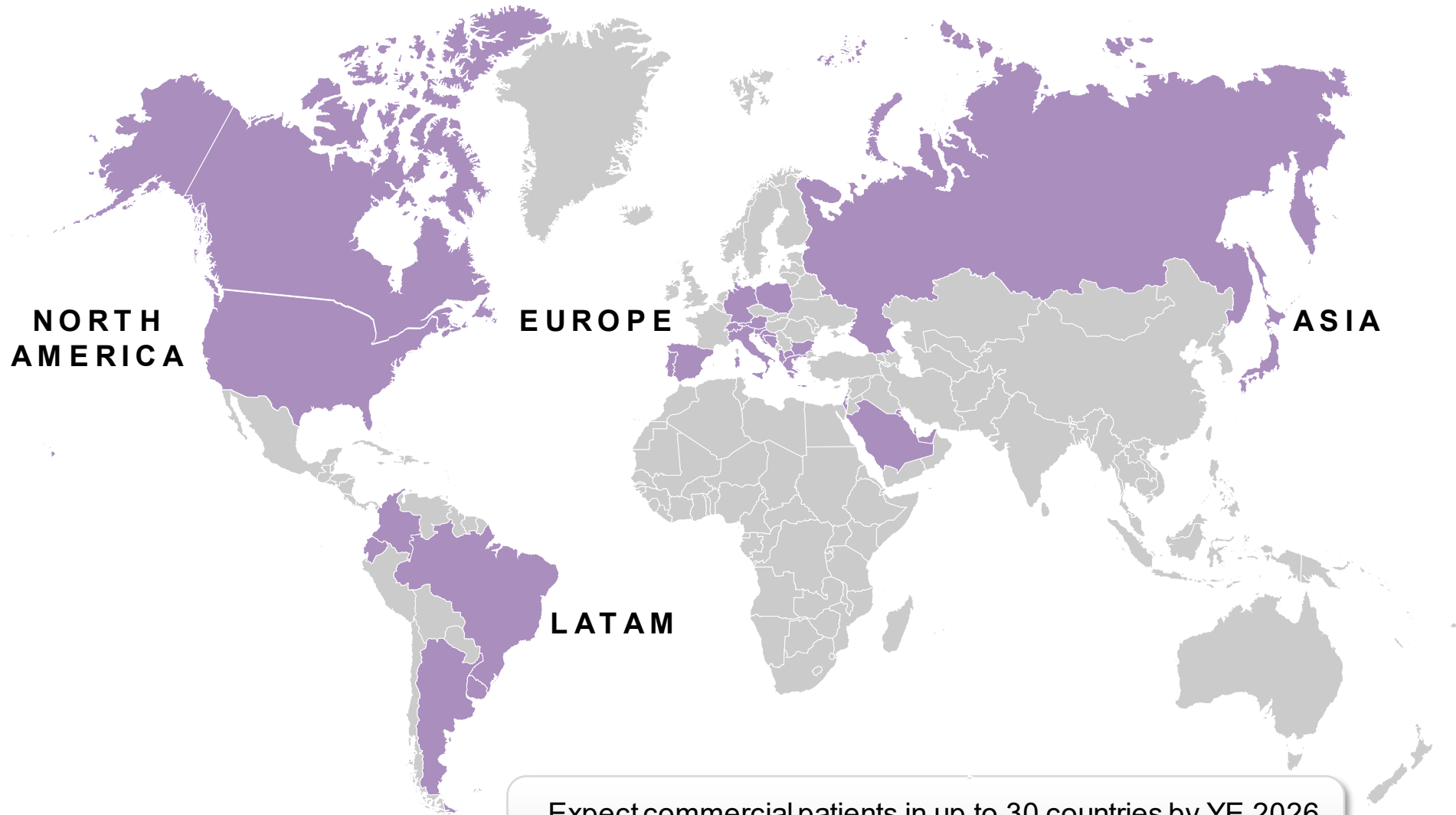
Sephience Differentiated Profile Enables Penetration Into All Key Market Segments



2025: Launch Initiated in US, EU and Canada



2026: Expanding the Sephience Global Launch



Votoplam

Huntington's Disease Program



April, living w ith HD

Phase 2 PIVOT-HD Trial Achieved Key Efficacy and Safety Objectives



PIVOT-HD study met primary endpoint of blood HTT protein lowering at Week 12 with durable dose-dependent lowering at Month 12

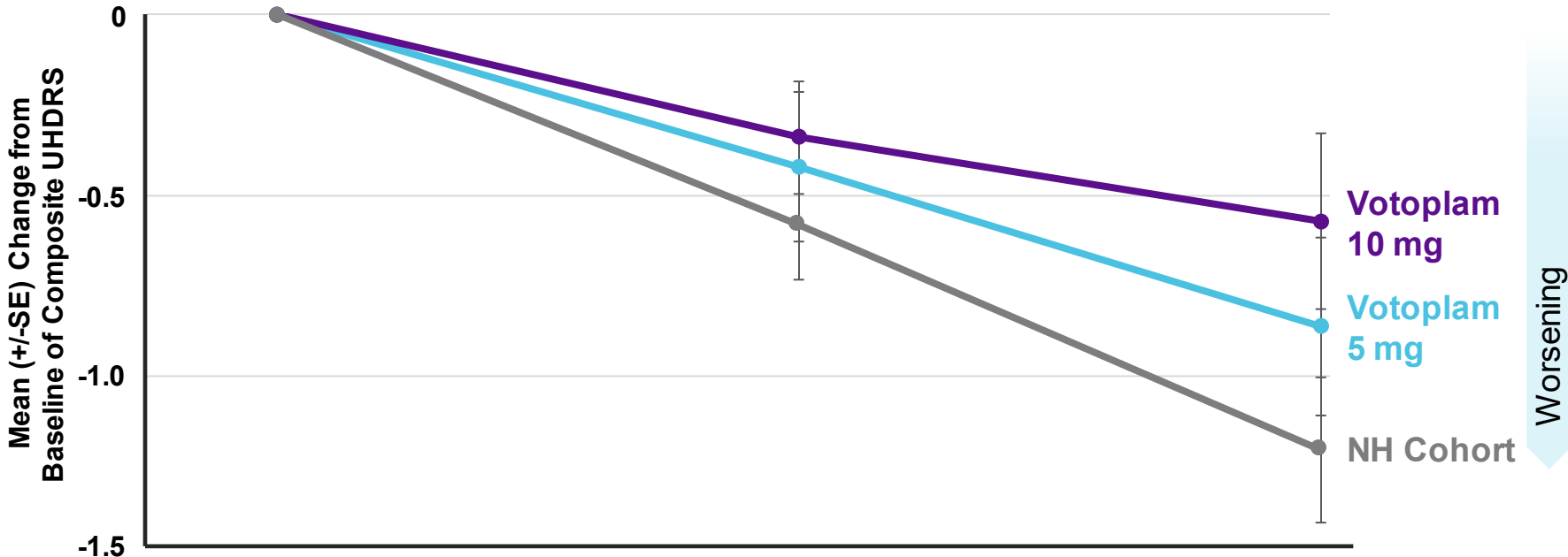


Evidence of dose-dependent disease slowing on cUHDRS in Stage 2 participants at 24 months



Continued evidence of favorable safety profile with no new AE signals identified for both dose levels and stages at 24 months

Dose-dependent Slowing of Disease Progression on cUHDRS at 24 months in Stage 2 Participants



Mean difference in cUHDRS progression of 52% (10mg) and 28% (5mg) at Month 24

Baseline	12 Month	24 Month
Treatment Group	Mean (SE)	Mean (SE)
Natural History (N=73)	-0.58 (0.15)	-1.20 (0.20)
5 mg (N=21)	-0.42 (0.16)	-0.86 (0.24)
10 mg (N=24)	-0.34 (0.21)	-0.57 (0.24)

Results based on observed data
SE = Standard Error

INVEST-HD Global Phase 3 Trial Initiated by Novartis

INVEST-HD* Global Phase 3 Study Overview

- Individuals with early symptomatic disease
- 3:2 randomization of voptoplam 10mg: placebo
- Target enrollment: ~770 participants in >30 countries
- Primary endpoint: Change from baseline in cUHDRS
- Treatment period up to 36 months
- Interim analysis planned for efficacy and futility



*Study sponsored and funded by Novartis; NCT#: NCT07326709

Vatiquinone

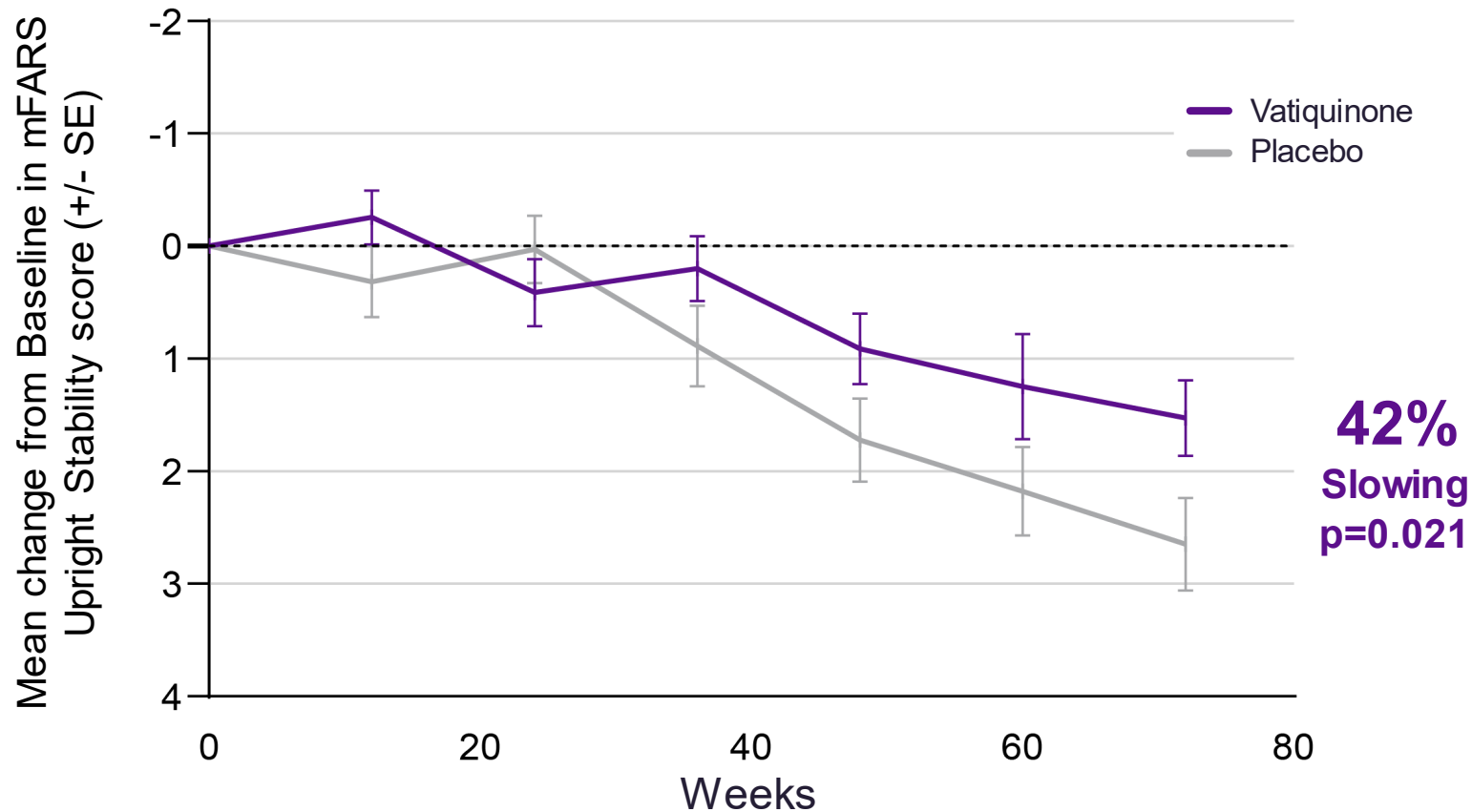
Friedreich's Ataxia Program



Olivia, living with FA

Vatiquinone Demonstrated Meaningful Slowing of Short- and Long-term Disease Progression

72-week Placebo-Controlled MOVE-FA Trial*



Long-term Extension Studies

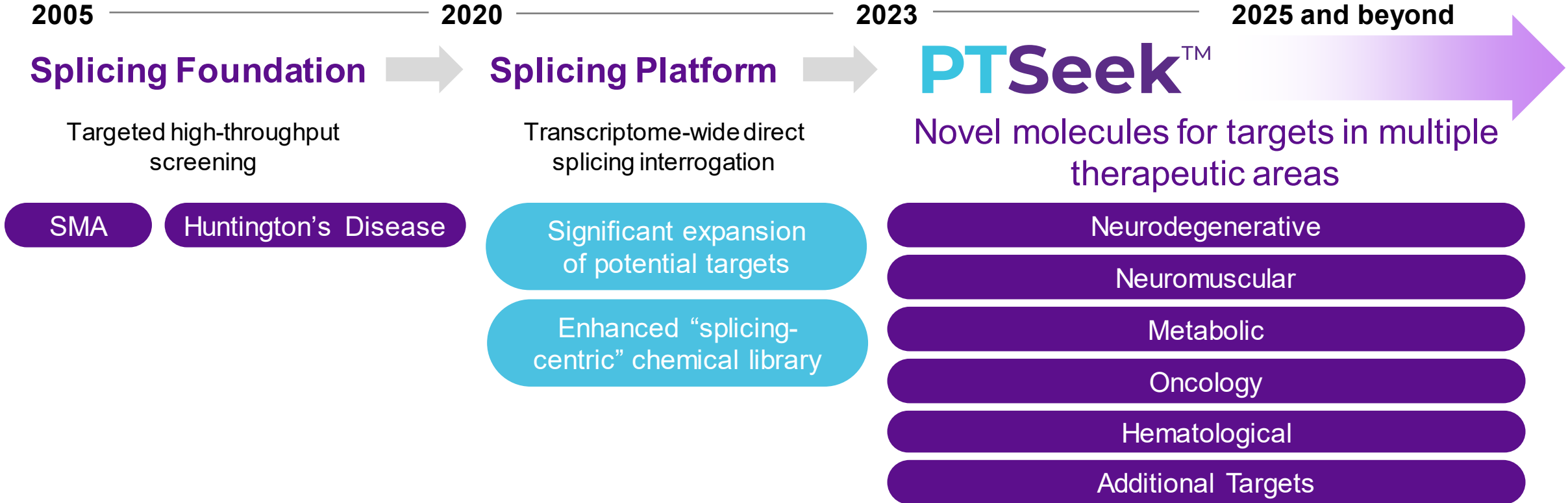
50% slowing (p<0.0001) of disease progression over 3 years** in MOVE-FA long-term extension study

4.8-point benefit (p<0.0001) on mFARS** over 2 years in ambulatory and non-ambulatory adults

Innovative Research Platforms



PTSeek Platform Provides Rapid and Reliable Identification of Splicing Therapy Candidates



R&D Pipeline Includes Innovative Programs from Differentiated Scientific Platforms



Splicing Platform

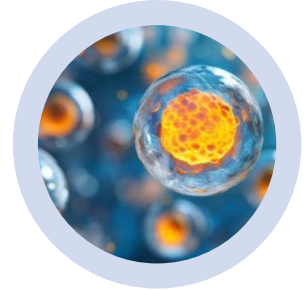
MSH3: Huntington's Disease, Myotonic Dystrophy I

ATXN3: Spinocerebellar Ataxia (SCA) 3

Undisclosed: Brain Tumors & Metastases

Undisclosed: Sickle Cell Disease, β -Thalassemia

Undisclosed: Neurodegenerative Diseases



Inflammation & Ferroptosis Platform

Ferroptosis: Parkinson's Disease

NRF2 Activation: CNS, Non-CNS Indications

NLRP3 Inflammasome: Inflammatory Lung Diseases

DHODH Inhibition: T-Cell Mediated Autoimmune Diseases

Well Positioned for Success in 2026 & Beyond

**Robust Global
Commercial
Engine**

**Innovative
R&D Platforms**

**Strong
Financial
Position**

2026

PTC Therapeutics

May 2026

