PTC Therapeutics 2025

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CEO

Pam and Kelsey living with PKU

The **FUTURE** Is **NOW**

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 to 2025 total revenue guidance and 2025 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, 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," and similar expressions.

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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, Kebilidi, Upstaza, Evrysdi, Tegsedi, Waylivra, sepiapterin or vatiqunone.

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.

Execution in 2024 Provides Foundation for Success

All Development Milestones Achieved

Outstanding Revenue Performance

Strong Cash Position

Advanced Programs from Innovative Scientific Platforms

Four U.S. Regulatory Approval Applications Submitted in 2024

AADC Gene Therapy BLA	*Approved*
Sepiapterin PKU NDA	*Accepted*
Translarna nmDMD NDA	*Accepted*
Vatiquinone Friedreich's Ataxia NDA	*Submitted*

Outstanding 2024 Revenue Performance Driven by Inline Products

translarna™ ataluren

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

Upstaza™ (eladocagene exuparvovec)

Tegsedi® (inotersen) injection 284 mg/1.5 mL

waylivra® (volanesorsen) injection 285 mg/1.5 mL

Evrysdi® risdiplam

2024

Unaudited Total Revenue

~$814M

Strong Cash Position Enables Future Revenue Growth and R&D Innovation

Reach Cashflow Breakeven Without Additional Capital

Support Commercial Launches and Innovative R&D Programs

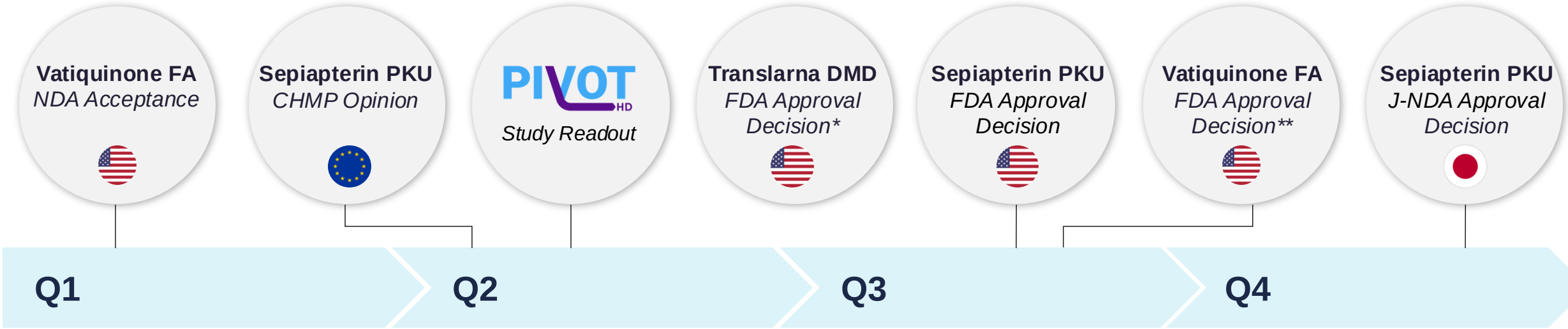
Fund BD Activities to Complement Product Portfolio

Research Platforms Provide Continuous Source of Innovative Therapies

Splicing

Inflammation & Ferroptosis

Numerous Potential Value-Creating Milestones Expected in 2025



*No action date provided
**If priority review granted

2025 Revenue and OPEX Guidance

translarna™ ataluren

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

Kebilidi™ (eladocagene exuparvovec-tneq)

Upstaza™ (eladocagene exuparvovec)

Tegsedi® (inotersen) injection 284 mg/1.5 mL

waylivra® (volanesorsen) injection 285 mg/1.5 mL

Evrysdi® risdiplam

2025

Revenue Guidance*

$600-800M

OPEX Guidance**

$730-760M

*Includes in-line products, potential new product launches and royalty revenue from Evrysdi.
**Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $75 million. GAAP R&D and SG&A expense for the full year 2025 is anticipated to be between $805 and $835 million.

PTC THERAPEUTICS™

Sepiapterin PKU Program

Owen living with PKU

Sepiapterin Demonstrates Potential to Meet Unmet Need for Majority of PKU Patients

aphenity

Dual mechanism of action provides stronger cofactor effect and enables efficacy in non-BH4 responsive mutations

Significant and meaningful Phe lowering with 84% of patients reaching target Phe levels (<360µmol/L)

Efficacy across all key subgroups in Phase 3 APHENITY trial including patients with classical PKU

Evidence of diet liberalization with ~50% of patients exceeding Recommended Daily Allowance for protein

Physician and Patient Statements Reflect Enthusiasm for Sepiapterin

"I'm excited about the opportunity to offer sepiapterin to all my patients"

– **Ania C. Muntau, MD,** Professor of Pediatrics, Chair, University Children's Hospital, University Medical Center, Hamburg Eppendorf, Germany

"Impressively, several participants with classical PKU…who had not shown response to sapropterin…showed clinically meaningful response to sepiapterin"

– **Cary O. Harding, MD,** *The Lancet*, Volume 404, Issue 10460, 1284 - 1286

Anecdotes on social media from patients about being able to liberalize their diets and "enjoy meat, fish, and chicken" while maintaining Phe control

APHENITY Results Support Potential to Address All Key PKU Population Segments

Patients Who Have Failed Current Therapies

Patients Who Are Not Well Controlled by Current Therapies

Therapy-Naive Patients Including Classical PKU

Greater than $1 Billion Potential Revenue Opportunity

Experienced U.S. Team Preparing for Planned 2025 Launch

Centers of Excellence & prescribers mapped

Payer & access discussions initiated

HCPs engaged through multifaceted approach

Patient & community partnerships established

Vatiquinone FA Program

Olivia living with FA

Vatiquinone Has Potential to be First Approved Treatment for Children with Friedreich's Ataxia

Vatiquinone data support safety and efficacy for **children and adults** with FA

NDA filing acceptance expected in February 2025 with approval decision potentially by August 2025

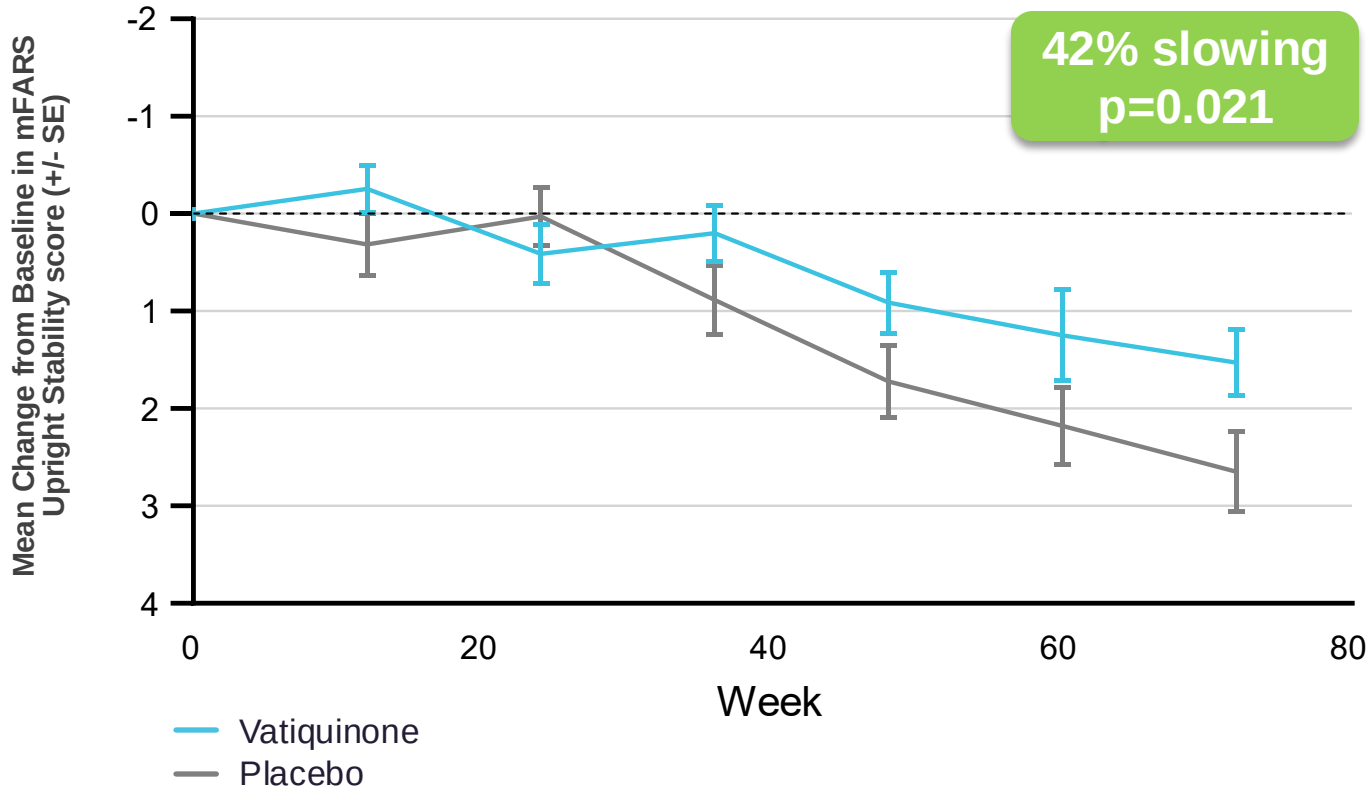
NDA based on findings of efficacy from MOVE-FA placebo-controlled study and two long-term studies

Additional marketing authorization submissions planned in 2025

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

MOVE-FA

72-week Placebo-Controlled Trial*



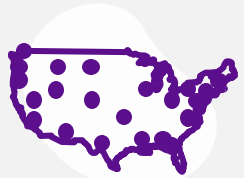
Long-term Extension Studies

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

*MOVE-FA did not meet its primary endpoint of statistically significant change in total mFARS at 72 weeks
**Relative to matched FACOMS (Friedreich's Ataxia Clinical Outcome Measures Study) natural history cohort

Vatiquinone Profile Supports Significant Commercial Opportunity Including All Friedreich's Ataxia Patients



~6,000 U.S. patients, ~1/3 are pediatric; global prevalence of ~25,000

Efficacy and safety data support opportunity for patients of all ages

Launch preparations underway including CoE and physician mapping

Strong 10+ year partnership with FA patient advocacy groups

PTC518
HD Program

April living with HD

Key Attributes of PTC518 Drive Differentiation

- Orally bioavailable
- Achieves excellent and broad CNS exposure
- Highly selective and specific for HTT target
- Uniform mHTT lowering in all regions of the brain
- Reduces HTT protein in the CNS & periphery
- Safe and well tolerated in clinical trials

Month 12 Interim Readout Met All Key Safety and Efficacy Objectives (June 2024)

Dose-dependent and durable lowering of mHTT protein in blood

Dose-dependent lowering of CSF mHTT protein levels

Dose-dependent trends of benefit on key clinical scales (TMS, cUHDRS)

PTC518 was well tolerated with no treatment-related NfL spikes

Planned Data Update in Q2 2025 to Include 12-month Results for Additional ~100 Subjects

Safety and tolerability of PTC518

Percent reduction in blood mHTT protein

Percent reduction in CSF mHTT protein

Changes in clinical scores (cUHDRS, TMS, TFC)

Readout will include both Stage 2 and 3 subjects

Results to Support Regulatory Discussions on Accelerated Approval Potential

Development and Commercialization Collaboration

$1 billion upfront payment

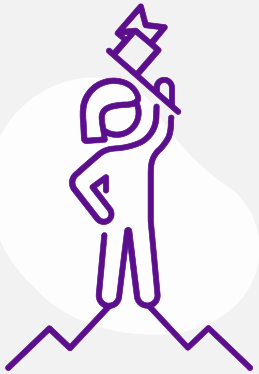
Up to **$1.9 billion** in development, regulatory and sales milestones

40% U.S. profit share, **double-digit** tiered royalties on ex-U.S. sales

Novartis to fund development activities following completion of PIVOT-HD

Expected to close in Q1 2025, subject to customary closing conditions, including regulatory clearance.
Refer to press release dated December 2, 2024 for additional details.

Validated Splicing Platform Provides Source of Innovative and Valuable Therapies

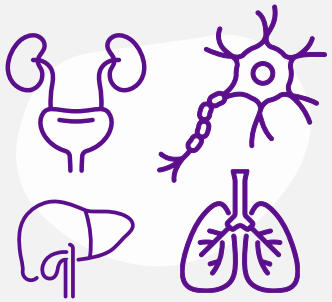


PTC has pioneered discovery and development of oral splicing therapies

PTSeek™

Platform innovations such as PTSeek™ accelerate discovery of novel splicing therapies

Multiple active CNS programs advancing towards clinic



Additional early-stage programs targeting non-CNS indications

Inflammation & Ferroptosis Programs Targeting CNS and Non-CNS Disorders

Focused on novel targets key to inflammation and oxidative stress

Active programs targeting CNS and non-CNS disorders

- Phase 2 ready DHODH inhibitor program for neuroinflammation indications
- NLRP3 inhibitor program entering IND-enabling studies
- Preclinical program targeting alpha synuclein for Parkinson's disease
- Preclinical program targeting nrf2 activation for both CNS and peripheral indications

PTC Vision for Successful 2025 and Beyond

Cash Flow Break Even

Path to $2 Billion Topline Revenue

Highly Differentiated and Innovative R&D Platforms

Business Development to Accelerate Revenue Growth

The **FUTURE** Is **NOW**

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TM