UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **September 7, 2022**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

Delaware	**001-35969**	**04-3416587**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

100 Corporate Court South Plainfield, NJ	**07080**
(Address of Principal Executive Offices)	(Zip Code)

Registrant's telephone number, including area code: **(908) 222-7000**

Not applicable

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions (*see* General Instruction A.2. below):

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.001 par value per share	PTCT	Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 7.01. Regulation FD Disclosure.

On September 7, 2022, PTC Therapeutics, Inc. (the "Company") updated its corporate presentation in anticipation of upcoming investor conferences. The Company's corporate presentation will be posted in the Events and Presentations page under the Investors section of the Company's website. A copy of the corporate presentation is also attached to this Current Report on Form 8-K (this "Report") as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into this Report, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	PTC Therapeutics, Inc. Corporate Presentation
104	The cover page from this Current Report on Form 8-K, formatted in Inline XBRL

Signature

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this Report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC Therapeutics, Inc.

Date: September 7, 2022

By: /s/ Emily Hill
Name: Emily Hill
Title: Chief Financial Officer


PTC 2022
Corporate Presentation
September 2022

Forward-Looking Statements

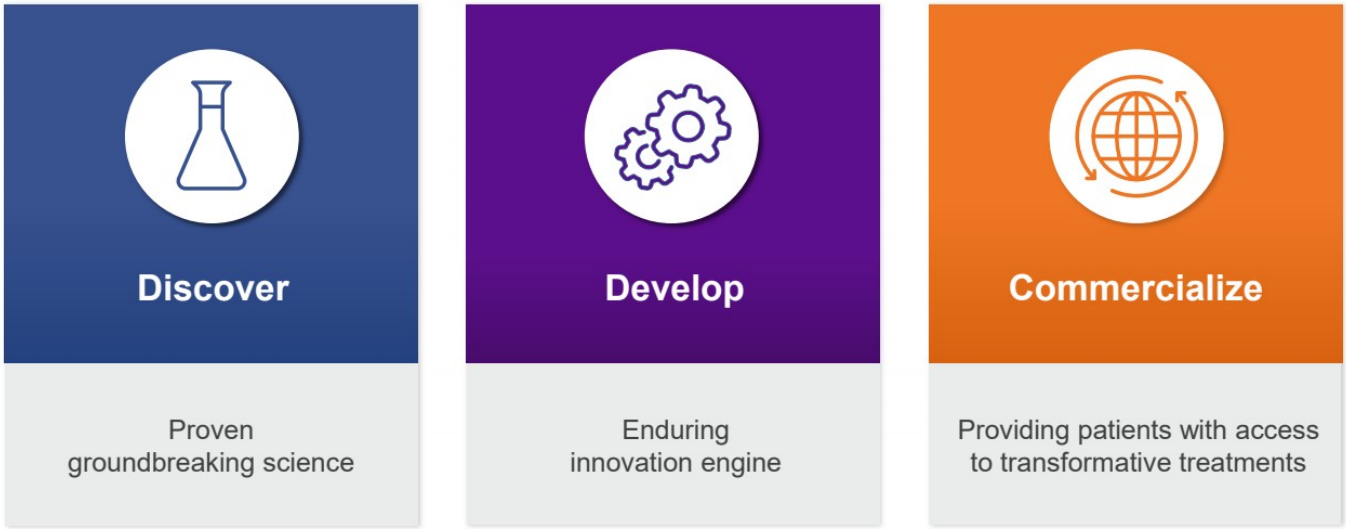
This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements with respect to guidance relating to 2022 total 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 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; 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; 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.

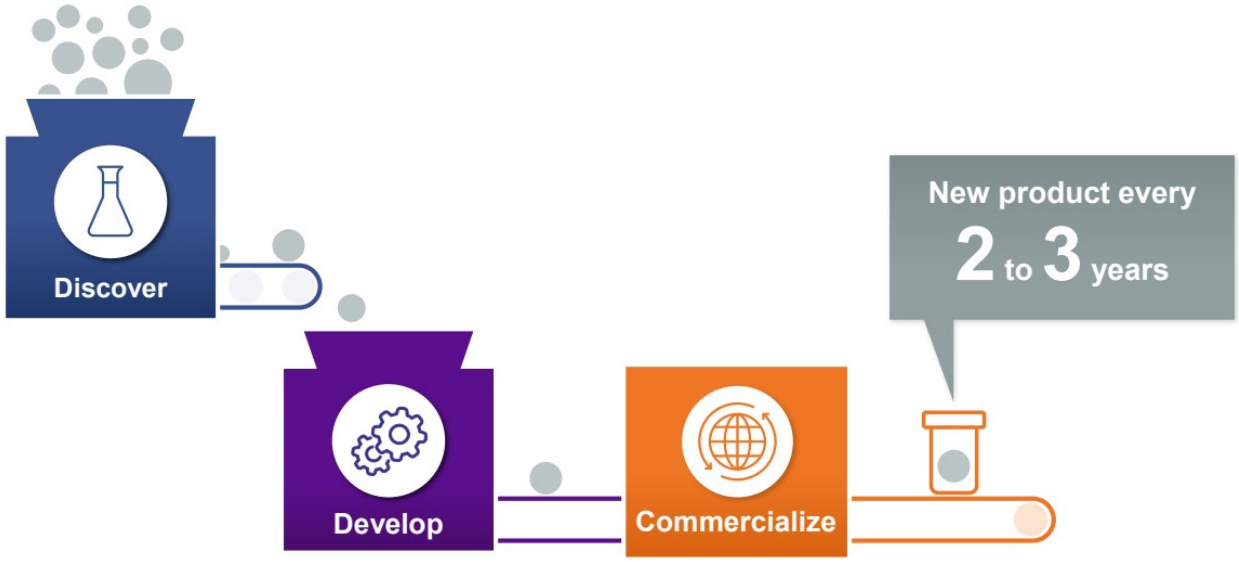
As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including 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.

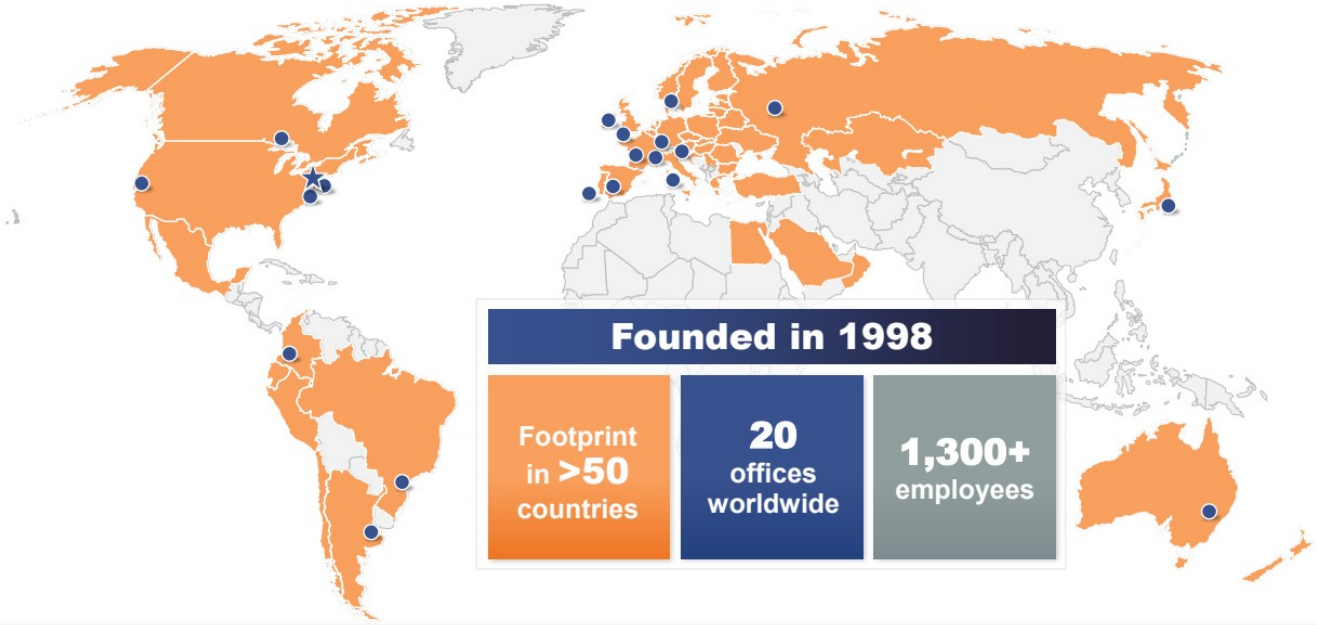
PTC Continually Innovates to Bring New Therapies to Patients



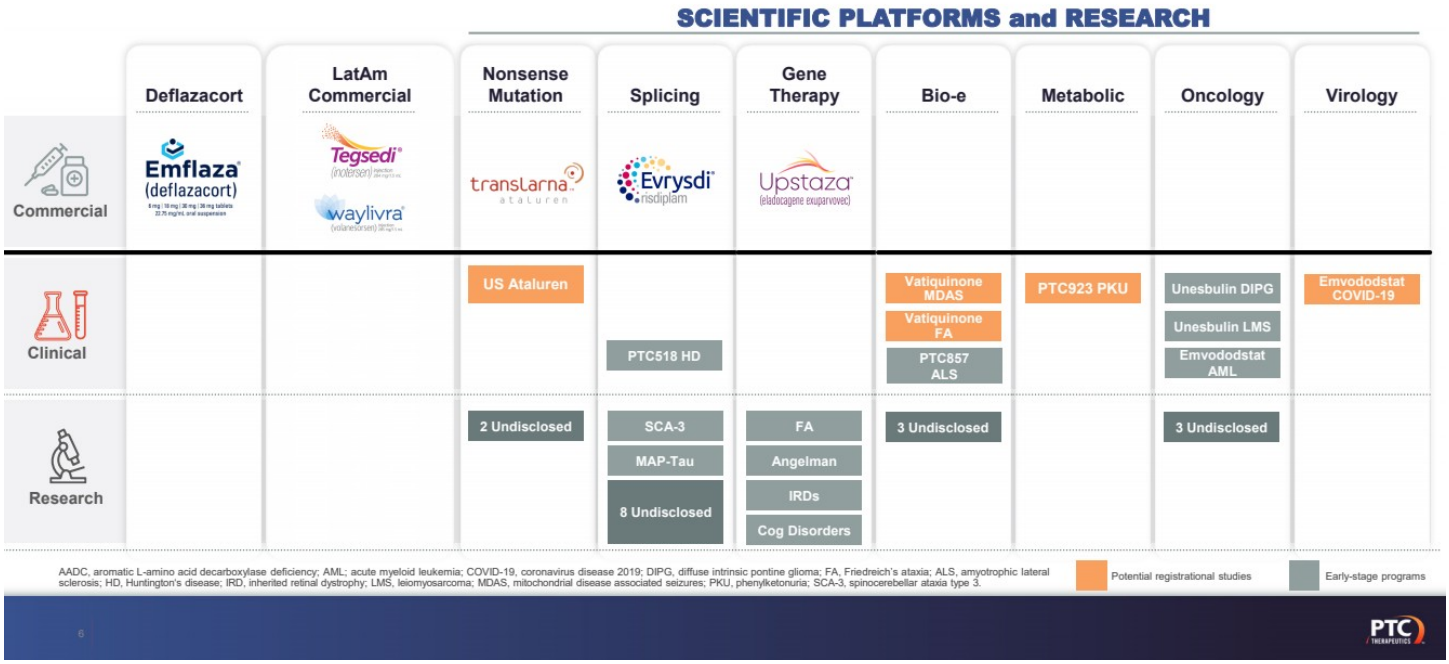
Building a Pipeline to Produce a Therapy Every 2-3 Years



PTC Has a Growing Global Footprint

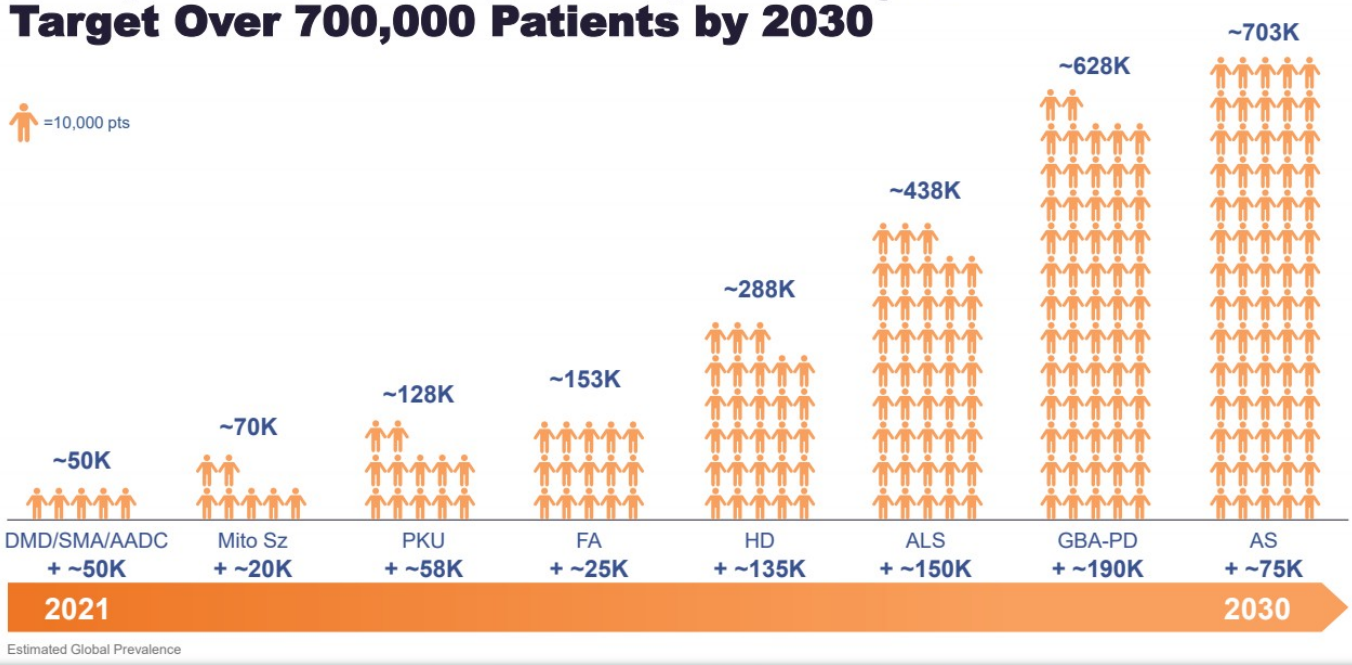


Diversified Platform Drives Strong Portfolio



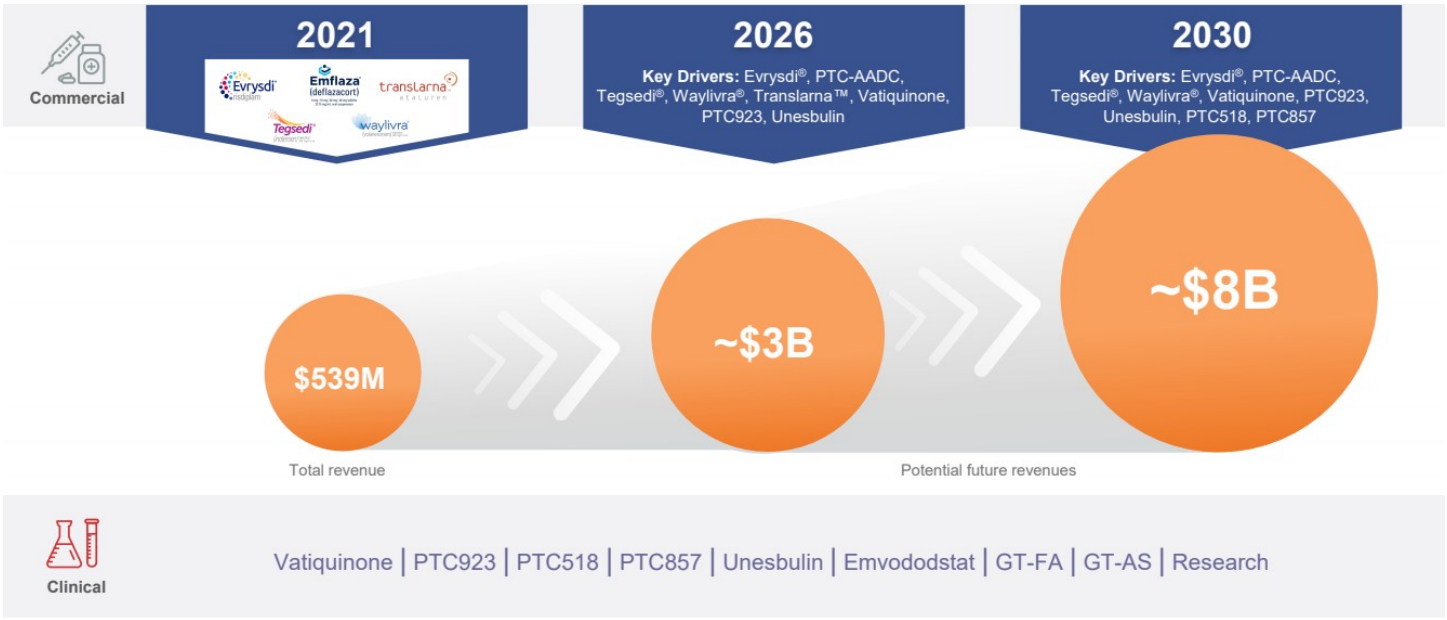
AADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich's ataxia; ALS, amyotrophic lateral sclerosis; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

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



Estimated Global Prevalence

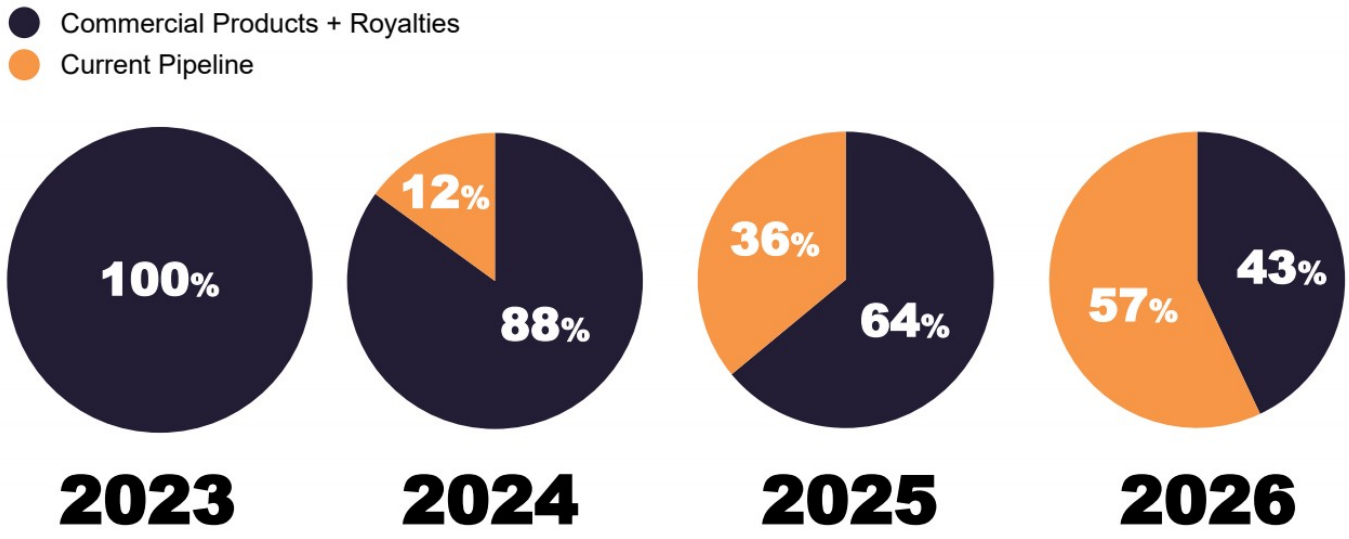
Enduring Innovation Drives Value Creation



Clinical

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

Revenue Contribution of Our Pipeline Grows

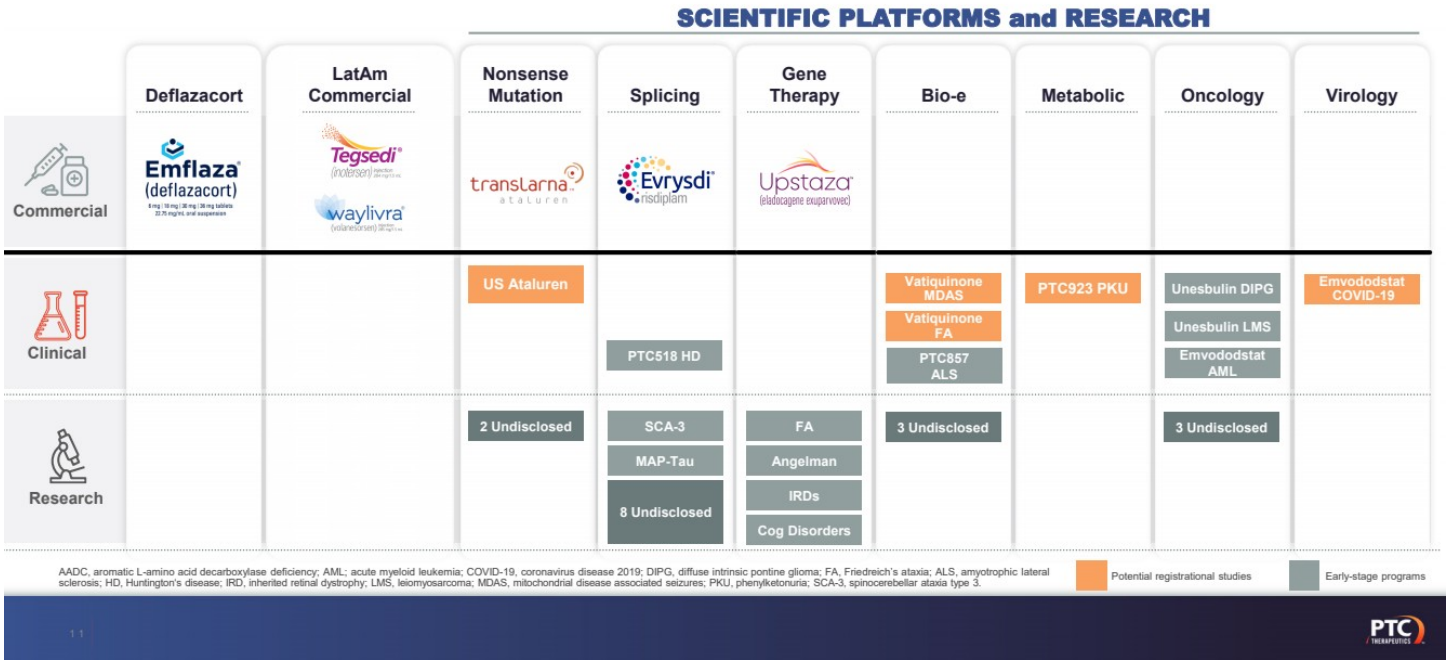


Potential future revenues

Strong Financial Performance Supports 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.

Diversified Platform Drives Strong Portfolio



Success Across Our Commercial Portfolio

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)

- First and only corticosteroid approved for DMD; approved for all US DMD patients >2yrs
- Data show clinical benefit over prednisone
- New patient starts, favorable access, high compliance, and appropriate weight-based dosing

Evrysdi® risdiplam

- Evrysdi now approved in 85 countries
- Continued strong uptake in the U.S. >20% market share
- Potential for $300M in sales-based milestones

Tegsedi® (inotersen) injection

- Innovative treatment for hATTR amyloidosis patients
- Disease awareness and patient ID continuing
- LATAM patients benefiting through early-access programs
- Fulfilled first group purchase order in Q2

waylivra® (volanesorsen) injection

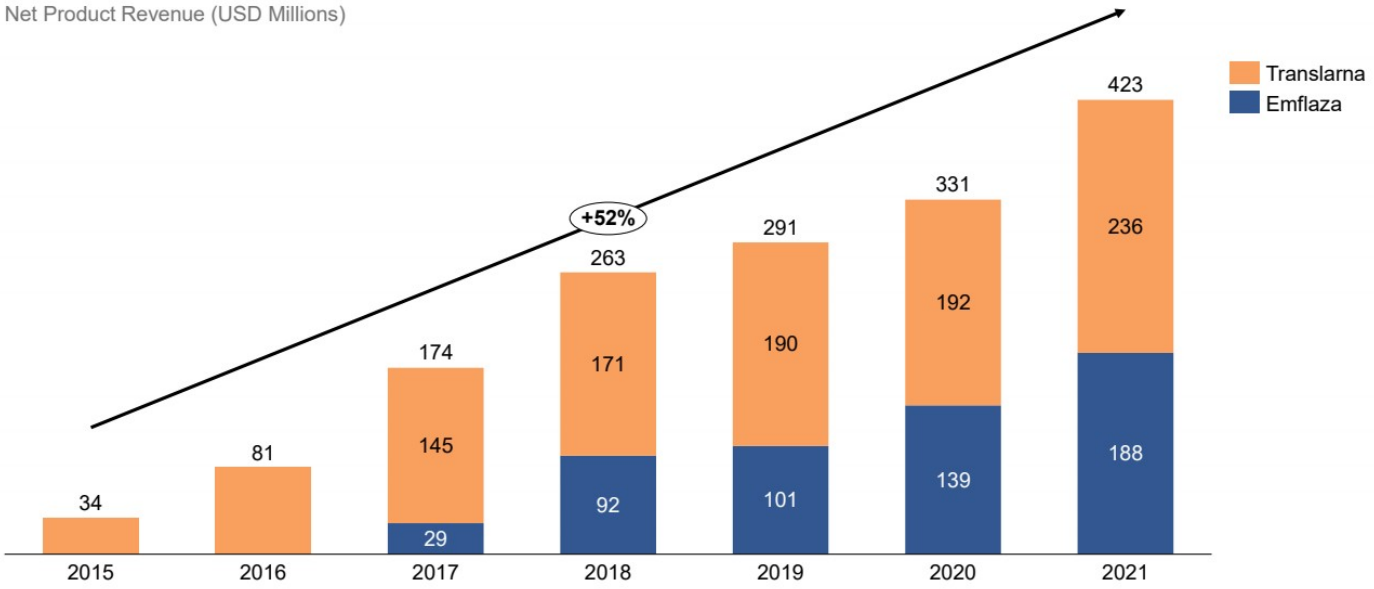
- For treatment of familial chylomicronemia syndrome (FCS)
- LATAM patients benefiting through early-access programs
- Regulatory decision on FPL indication expected in 2H22

Upstaza® (eladocagene exuparvovec)

- First approved disease-modifying treatment for AADC deficiency for patients 18 months and older
- First marketed gene therapy directly infused into the brain
- Approved by EMA in July

Continued Strong DMD Franchise Growth

Net Product Revenue (USD Millions)



Upstaza™ Has the Potential to Provide Significant Benefit to AADC Deficiency 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

Upstaza is the first and only approved disease-modifying therapy for AADC-d and will become the standard of care.

Mechanism of Action

Upstaza is the first marketed gene therapy directly infused into the brain.

Upstaza Gene Therapy for AADC Deficiency Approved by European Commission

Upstaza (eladocagene exuparvovec)

Regulatory

✓ **Approved by EMA in July**

- PTC-AADC 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

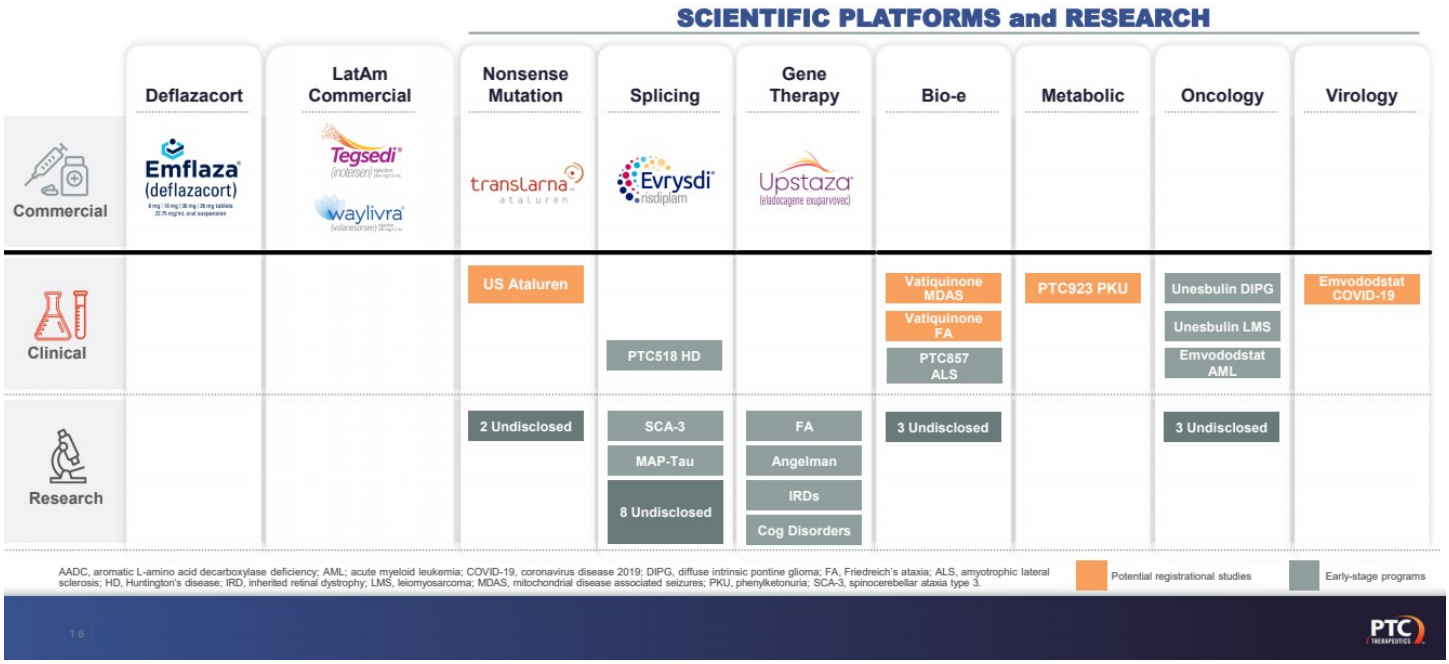
- Identification and preparation of expert pediatric neurosurgical centers
- Continued KOL engagement

Market Opportunity

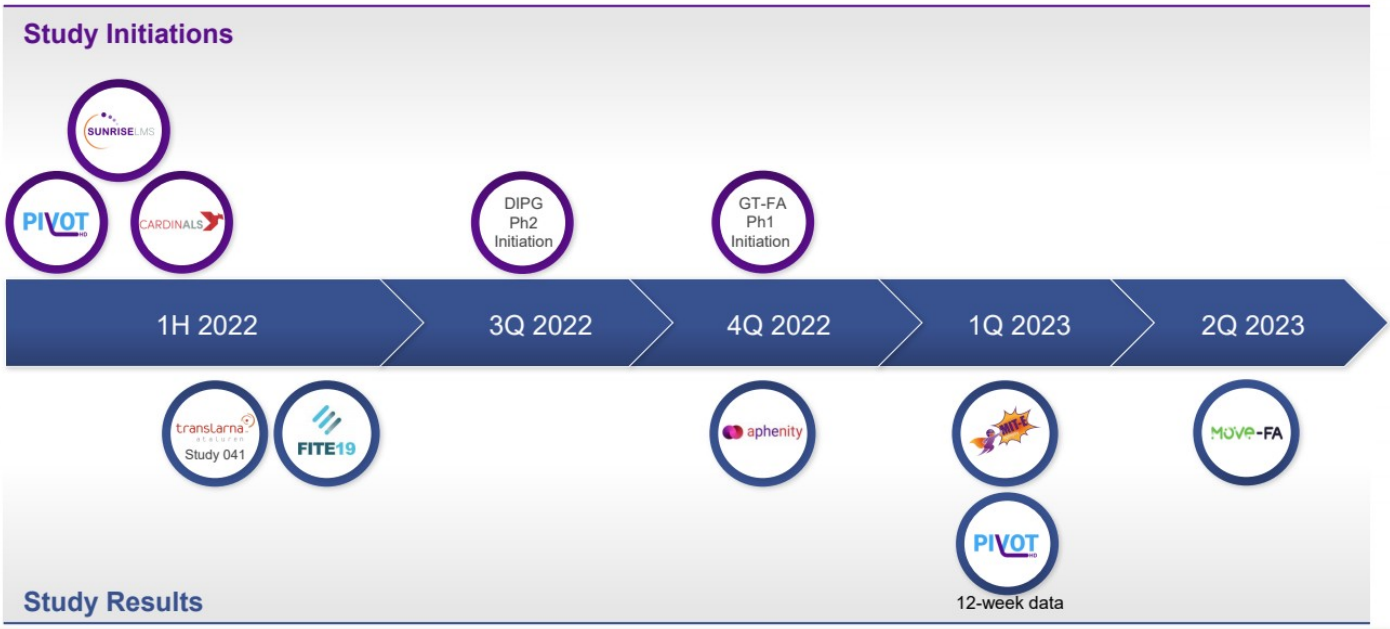
- Potential over $1B in cumulative revenue
- Successful patient finding is ongoing

PTC THERAPEUTICS

Diversified Platform Drives Strong Portfolio



Substantial Pipeline Progress Planned



Three Registration-Directed Clinical Trials Drive Near-Term Value

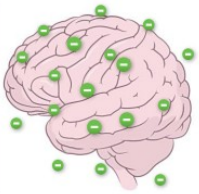
PTC923	Vatiquinone	Vatiquinone
PKU	Mitochondrial Disease Associated Seizures	Friedreich Ataxia
aphenity	MIT-E epilepsy	MOVE-FA
Data Expected **YE 2022**	Data Expected **1Q 2023**	Data Expected **2Q 2023**

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

	Deflazacort	LatAm	Nonsense	Gene Therapy	Bio-e	Metab
Commercial	Emflaza (deflazacort)					
Clinical					Vatiquinone MDAS Vatiquinone FA PTC857 ALS	
Research				8 Undisclosed IRDs Cog Disorders	3 Undisclosed	

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

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

PTC THERAPEUTICS

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

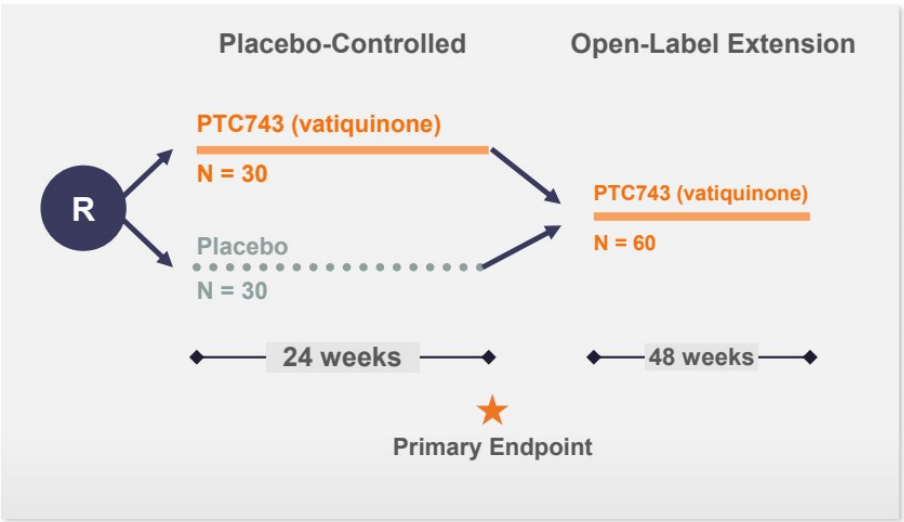
No approved disease-modifying treatments

~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 1Q 2023


MOVE-FA
MOVE-FA:
Registration-directed trial
of vatiquinone
for Friedreich Ataxia
23
PTC
THERAPEUTICS

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

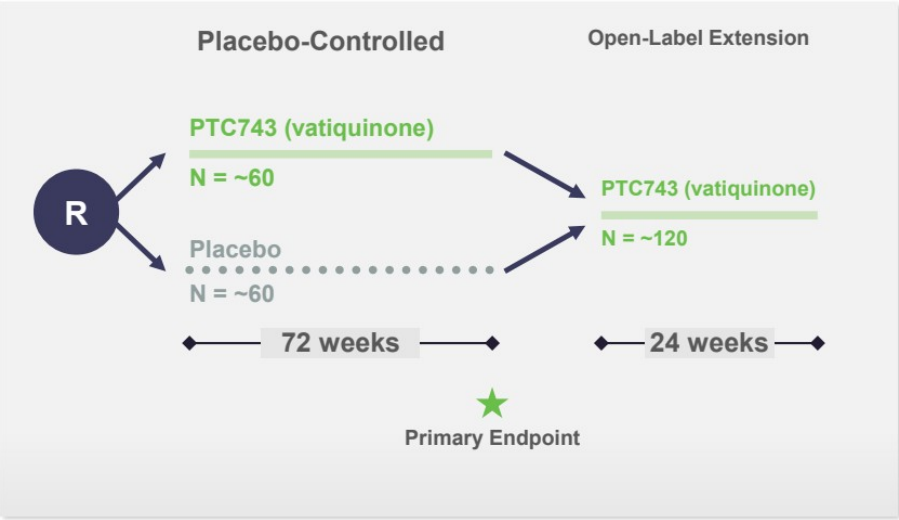
No approved disease-modifying therapies

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

PTC THERAPEUTICS

CardinALS:
Phase 2 trial of PTC857 for Amyotrophic Lateral Sclerosis

PTC THERAPEUTICS

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

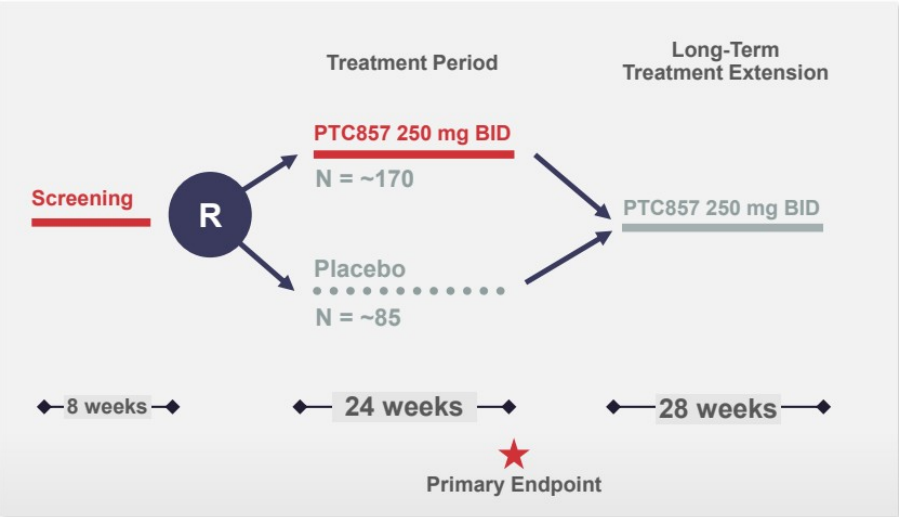
No approved disease-modifying therapies

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



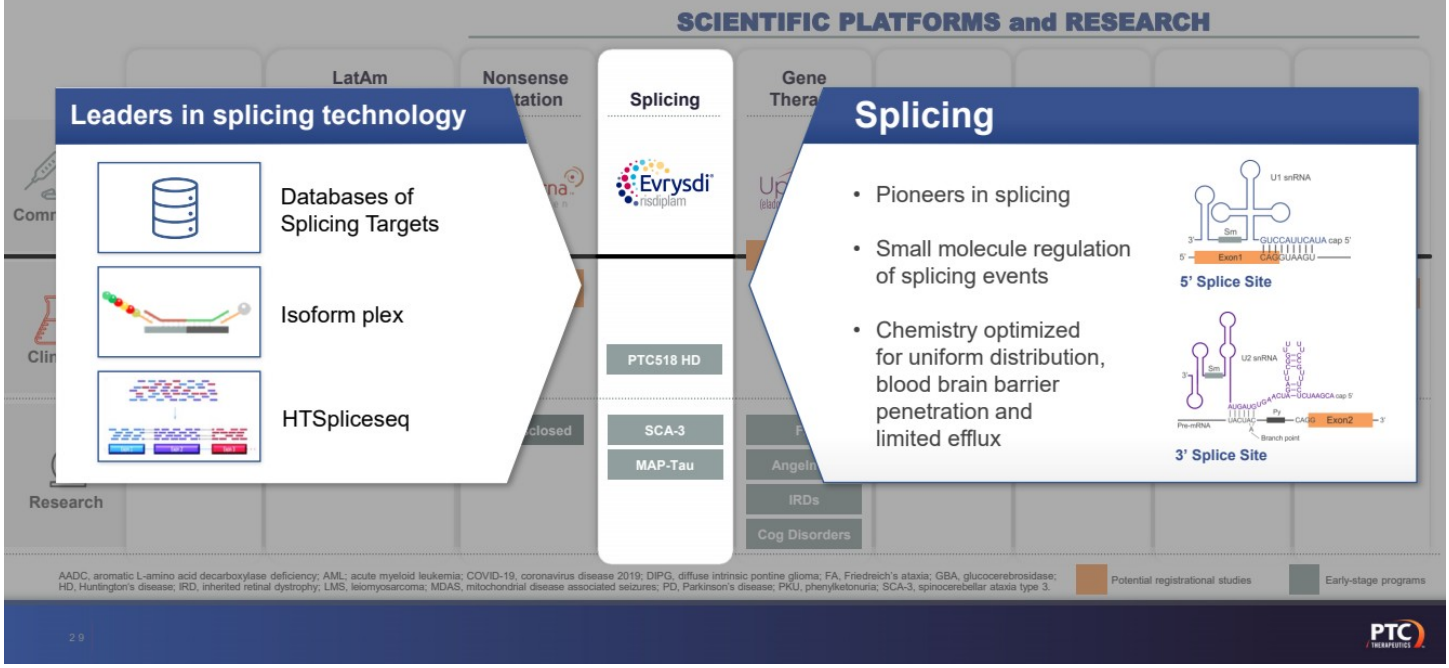
Primary Endpoints
Change in ALSFRS-R

Secondary Endpoints
Safety and PK

Trial Status

- Initiated in 1Q 2022

Diversified Platform Drives Strong Portfolio



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PIVOT
HD
PIVOT HD:
PTC518 for Huntington's Disease
30
PTC
THERAPEUTICS

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

Disease

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

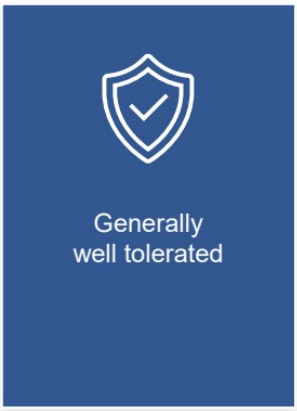
Current Treatments

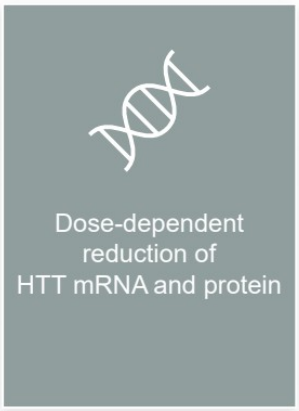
No approved disease-modifying therapies

Mechanism of Action

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

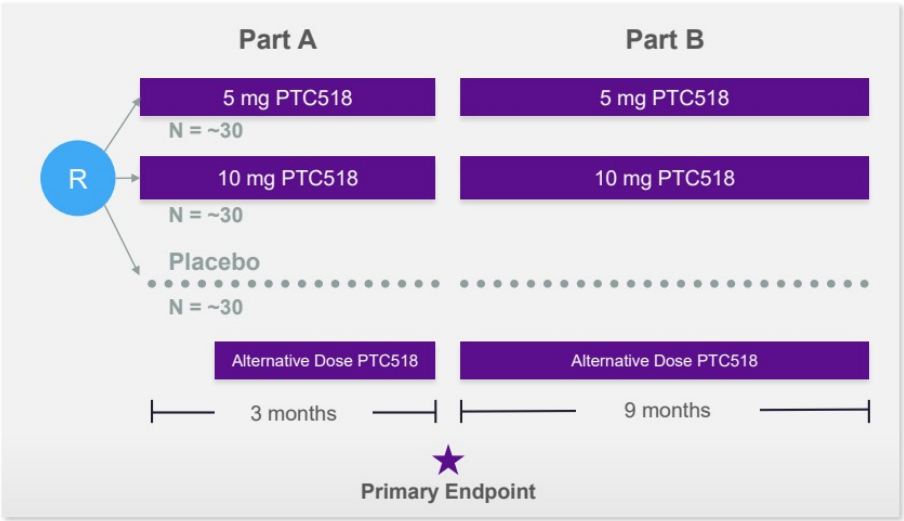
PTC518 Reduces HTT mRNA and Protein to Target the Proximal Cause of HD





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

- Initiated in 1Q 2022

PIVOT HD Trial Target Population and Endpoints

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:
Registration-directed trial of PTC923 for **PKU**

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

Disease

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

Current Treatments

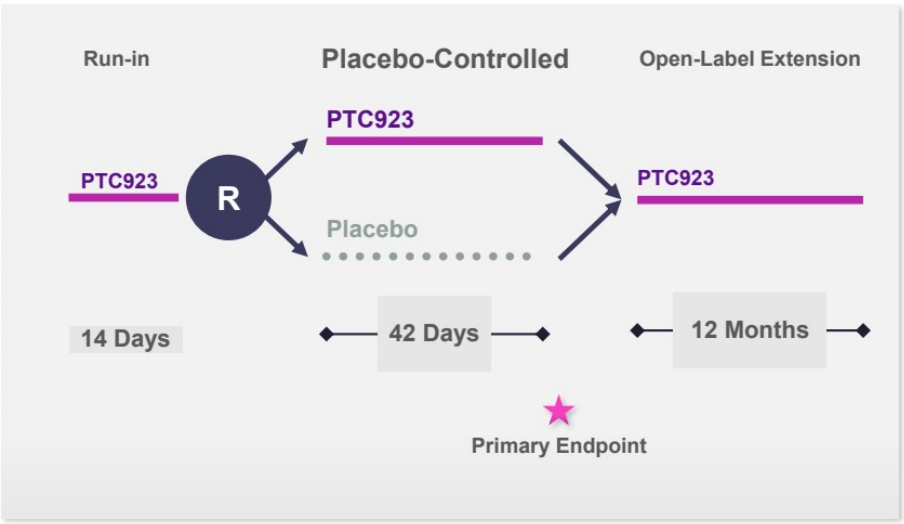
Majority of patients do not initially respond or are not well controlled by standard of care

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



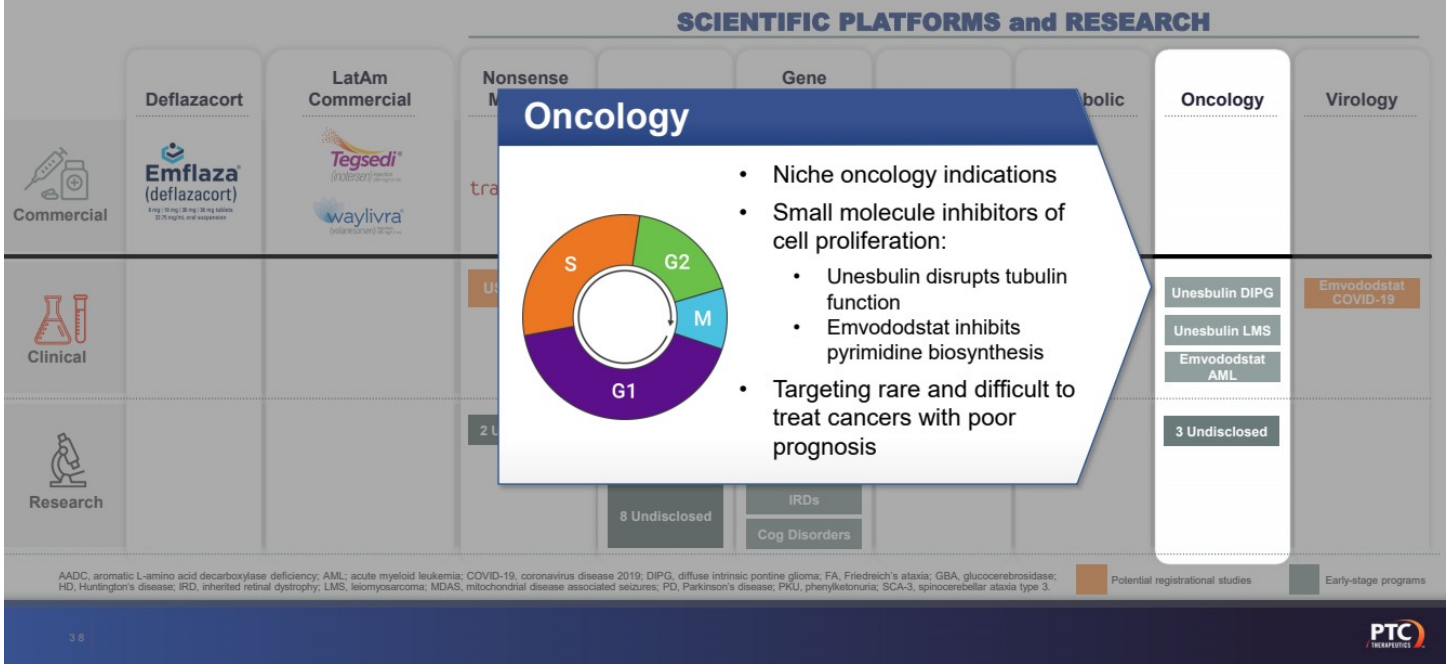
Primary Endpoint

Reduction in blood phenylalanine levels

Trial Status

- Data expected YE 2022

Diversified Platform Drives Strong Portfolio



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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

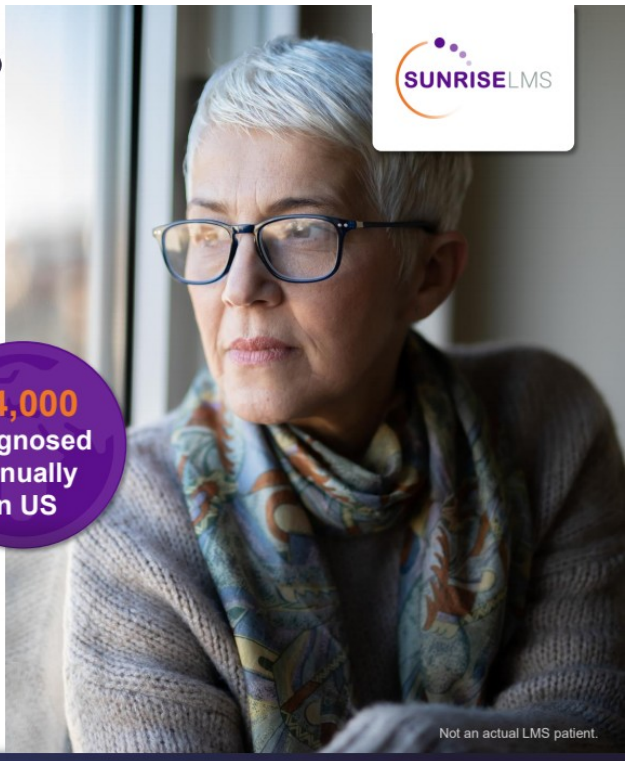
Current Treatments

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 in G2/M phase, including cancer stem cells by inhibiting tubulin polymerization

~4,000
Diagnosed annually in US



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/m² dacarbazine

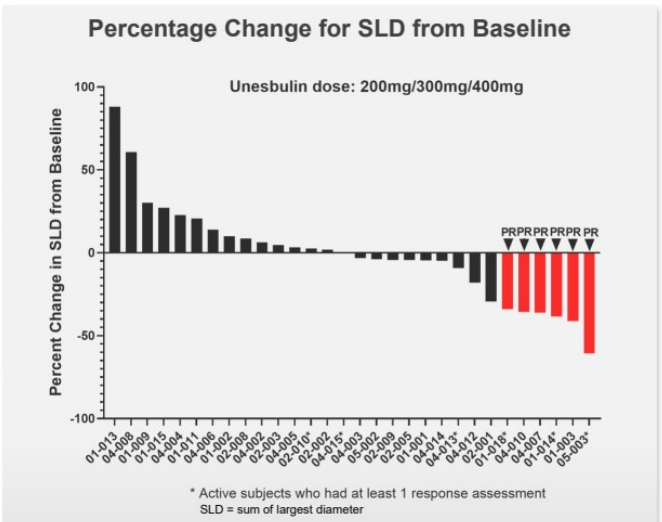
N = 29

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



* Active subjects who had at least 1 response assessment
SLD = sum of largest diameter

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

SUNRISELMS

Primary Endpoint
PFS as determined by RECIST

Secondary Endpoints
OS, ORR, DCR, DOR

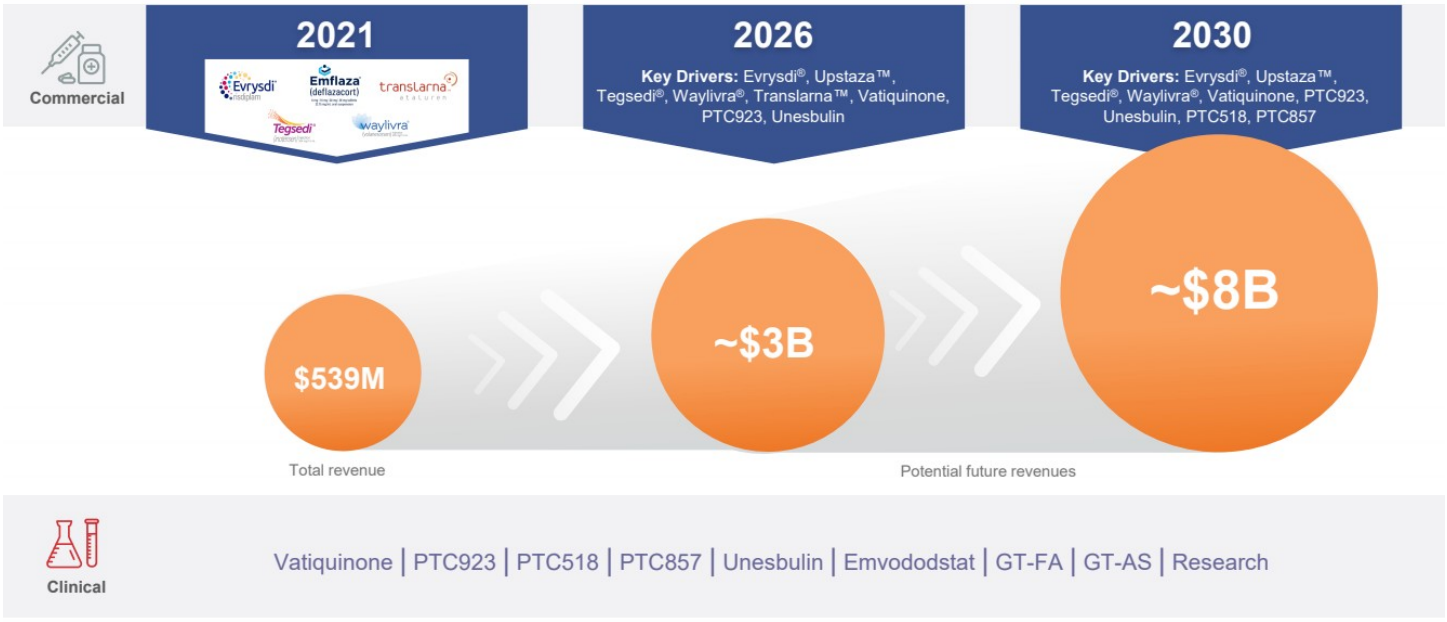
Interim Analysis

Trial Status

- Initiated in 1Q 2022

PTC THERAPEUTICS

Enduring Innovation Drives Value Creation



Clinical

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