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): **January 11, 2021**

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** (Address of Principal Executive Offices)	**07080** (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 2.02. Results of Operations and Financial Condition.

On January 11, 2021, PTC Therapeutics, Inc. (the "Company") issued a press release (the "press release") announcing certain preliminary (unaudited) financial information for its fiscal year ending December 31, 2020, including that the Company expects to report (i) total unaudited net product revenue of approximately $333 million, (ii) net product revenue for Translarna™ (ataluren) of approximately $192 million, with approximately $69 million in revenue in the fourth quarter of 2020, and net product revenue for Emflaza® (deflazacort) of approximately $139 million, with approximately $37 million in revenue in the fourth quarter of 2020, (iii) $42.5 million in revenue associated with Evrysdi™ (risdiplam) milestones and (iv) ending cash and cash equivalents of approximately $1.1 billion. Final results are subject to completion of the Company's year-end audit.

Item 7.01. Regulation FD Disclosure.

The Company also announced financial guidance for its fiscal year ending December 31, 2021 in the press release, including that the Company anticipates (i) full-year net product revenues for the Duchenne muscular dystrophy franchise to be between $355 and $375 million and (ii) GAAP R&D and SG&A expense for the full year 2021 to be between $825 and $855 million with non-GAAP R&D and SG&A expense for the full year 2021 to be between $725 and $755 million, excluding estimated non-cash, stock-based compensation expense of approximately $100 million.

The Company announced that on Monday, January 11, 2021 at 7:30 am EST at the 39th Annual J.P. Morgan Healthcare Conference, the Company will present its 2021 strategic priorities, preliminary 2020 financial results, and 2021 financial guidance. The presentation will be webcast live on the Events and Presentations page under the Investors section of the Company's website.

This Current Report on Form 8-K and Exhibits 99.1 and 99.2 include a forward-looking financial measure that was not prepared in accordance with accounting principles generally accepted in the United States (GAAP), non-GAAP R&D and SGA expenses (which excludes non-cash stock-based compensation expense). Management uses this measure when assessing and identifying operational trends and, in management's opinion, this non-GAAP measure is useful to investors and other users of its financial statements by providing greater transparency into the historical and projected operating performance of PTC and the Company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP.

The information in this Current Report on Form 8-K, including Exhibits 99.1 and 99.2, 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.

Forward Looking Statements: All statements, other than those of historical fact, contained in this Current Report on Form 8-K, are forward-looking statements, including reporting expectations with respect to financial information for fiscal year 2020 and financial guidance for fiscal year 2021. The Company'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 preliminary nature of the Company's 2020 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2021; and the factors discussed in the "Risk Factors" section of the Company's Annual Report on Form 10-K for the year ended December 31, 2019, Quarterly Reports on Form 10-Q for the periods ended March 31, 2020, June 30, 2020 and September 30, 2020 as well as any updates to these risk factors filed from time to time in the Company's other filings with the Securities and Exchange Commission. You are urged to carefully consider all such factors. The forward-looking statements contained herein and the exhibits hereto represent the Company's views only as of the date of this Current Report on Form 8-K and the Company 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 Current Report on Form 8-K except as required by law. All website addresses given in this Current Report on Form 8-K 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	Press Release, dated January 11, 2021 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – 39th Annual J.P. Morgan Healthcare Conference
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: January 11, 2021

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

PTC Therapeutics Provides Update on R&D Pipeline and Commercial Progress at 39th Annual J.P. Morgan Healthcare Conference

- ~$333 million preliminary unaudited 2020 total net product revenue representing 14% year-over-year growth -
- Huntington splicing program healthy volunteer data expected 1H 2021 -
- Multiple late-stage clinical programs advancing with six registration-directed trials -
- 2021 Duchenne franchise revenue guidance of $355-$375 million -

SOUTH PLAINFIELD, N.J., Jan. 11, 2021 – PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its R&D pipeline and commercial progress at the 39th Annual J.P. Morgan Healthcare Conference today, Monday, Jan. 11 at 7:30 a.m. EST. Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2020 accomplishments and highlight upcoming potential value-creating milestones. Preliminary 2020 unaudited financial results and 2021 financial guidance will also be provided. The presentation will be webcast live on the Events and Presentations page of the Investors section of PTC Therapeutics website at www.ptcbio.com.

Key 2020 Corporate Highlights:

- The Duchenne muscular dystrophy (DMD) franchise, consisting of Translarna™ (ataluren) and Emflaza® (deflazacort), continue to demonstrate year-over-year growth, with 2020 preliminary unaudited revenue of approximately $331 million.
  - Translarna growth is primarily driven by geographical expansion and label modifications allowing broader access.
  - Emflaza experienced strong 38% year-over-year revenue growth in 2020 due to increased patient prescriptions and high compliance.
- In August, Evrysdi™ (risdiplam) was approved by the FDA for patients with Spinal Muscular Atrophy (SMA). A strong global launch is underway with increasing U.S. patient uptake across all disease subtypes in both treatment naïve and switch patients. The European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP) opinion for Evrysdi is expected in 1H 2021, and priority review of the Japanese New Drug Application (NDA) is ongoing. Evrysdi is the first at-home, orally administered SMA treatment and was developed from PTC's proprietary splicing platform. Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation and Roche.

- PTC initiated three clinical trials from its novel Bio-e platform, including two registration-directed trials with vatiquinone (PTC743) in Mitochondrial epilepsy (MIT-E) and Friedreich ataxia (MOVE-FA) that are actively enrolling.

2021 Potential Value-Creating Milestones:
- Top-line results from the Translarna dystrophin study are expected in 1Q 2021. With positive results, PTC plans to submit an NDA to the FDA.
- PTC is preparing to launch its first potential gene therapy, PTC-AADC, for the highly morbid genetic pediatric disorder aromatic L-amino acid decarboxylase deficiency (AADC-d). Launch efforts include expanding genetic testing programs and the identification and preparation of expert pediatric neurosurgical centers.
- PTC-AADC expected regulatory milestones include a CHMP opinion for a potential approval and the submission of a Biologics License Application (BLA) to the FDA in 1H 2021.
- Results from PTC's Huntington disease program Phase 1 study of PTC518 in healthy volunteers are expected in 1H 2021. PTC518 was discovered from PTC's validated splicing platform.
- A registration-directed clinical trial of PTC299 for COVID-19 (FITE19) continues to enroll patients. PTC anticipates completing the study in 1H 2021.
- A registration-directed study, APHENITY, for PTC923 in patients with phenylketonuria (PKU) is expected to initiate in mid-2021.
- Results by year end are expected from ongoing clinical trials evaluating PTC596 in Leiomyosarcoma and Diffuse Intrinsic Pontine Glioma (DIPG).
- Gene therapy manufacturing for clinical trials will begin at PTC's state-of-the-art biologics production facility in Hopewell, N.J.

Preliminary Unaudited 2020 Financial Results:
- Total unaudited net product revenue for full year 2020 was approximately $333 million.
- DMD franchise revenue for year end 2020 included net product revenue for Translarna of approximately $192 million with $69 million in revenue in the fourth quarter and approximately $139 million year end 2020 revenue for Emflaza with $37 million in revenue in fourth quarter.
- PTC expects to report $42.5 million in 2020 revenue associated with Evrysdi milestones. PTC will report fourth quarter royalty revenue for Evrysdi on the company's next earnings call.
- PTC expects to report 2020 year-end cash and cash equivalents of approximately $1.1 billion.

PTC is currently in the process of finalizing its financial results for the 2020 fiscal year. The above information is based on preliminary unaudited information and

management estimates for the full year 2020, subject to the completion of PTC's financial closing procedures.

2021 Financial Guidance:

- PTC anticipates full year net product revenues for the DMD franchise to be between $355 and $375 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2021 to be between $825 and $855 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2021 to be between $725 and $755 million, excluding estimated non-cash, stock-based compensation expense of approximately $100 million.

Non-GAAP Financial Measures:

In this press release, the financial results and financial guidance of PTC are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, the non-GAAP financial measure excludes non-cash, stock-based compensation expense. This non-GAAP financial measure is provided as a complement to financial measures reported in GAAP because management uses this non-GAAP financial measure when assessing and identifying operational trends. In management's opinion, this non-GAAP financial measure is useful to investors and other users of PTC's financial statements by providing greater transparency into the historical and projected operating performance of PTC and the company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. A quantitative reconciliation of the non-GAAP financial measure to its closest equivalent GAAP financial measure is included in the table below.

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2021 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 825,000	$ 855,000
Less: projected non-cash, stock-based compensation expense	100,000	100,000
Projected non-GAAP R&D and SG&A expense	**$ 725,000**	**$ 755,000**

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

For More Information:
Investors & Media:
Jane Baj
+1 (908) 912-9167
jbaj@ptcbio.com

Forward Looking Statements:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including the information provided under the heading "2021 Guidance", including with respect to (i) 2021 net product revenue guidance and (ii) 2021 GAAP and non-GAAP R&D and SG&A 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 and other matters; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions 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; the timing with respect to orders for PTC's products; 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: 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 potential 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 potential regulatory submissions and potential commercialization with respect to Evrysdi; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), 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 enroll, fund, complete and timely submit to the EMA the results of 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, 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 PTC299 clinical trial for COVID-19; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 Quarterly Report on Form 10-Q and 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 PTC-AADC.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release 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 press release except as required by law.

Exhibit 99.2


PTC 2021
J.P. Morgan Healthcare Conference
Stuart W. Peltz, Ph.D., CEO

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 release, other than statements of historic fact, are forward-looking statements, including statements with respect to 2021 net product revenue guidance, statement respect to the 2021 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 PTC's gen therapy platform, including any potential regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, includi potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions an commercialization of its products and product candidates; the timing with respect to orders for PTC's products; PTC's strategy, future operations, future financial p future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipa "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

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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 r any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurr after the date of this presentation except as required by law.

Translating Science to Transform Lives

PTC THERAPEUTICS

Significant Execution and Value Creation in 2020

Clinical

- ✓ Initiated two potential registrational trials with vatiquinone in Mitochondrial Epilepsy & Friedreich ataxia
- ✓ Completed Translarna™ dystrophin trial for potential US accelerated approval
- ✓ Initiated trial of PTC518 in healthy volunteers for Huntington disease program

Regulatory

- ✓ Evrysdi™ approval in US and multiple additional countries
- ✓ Submitted MAA to EMA for gene therapy to treat AADC deficiency
- ✓ Translarna label modification related to non-ambulatory patients

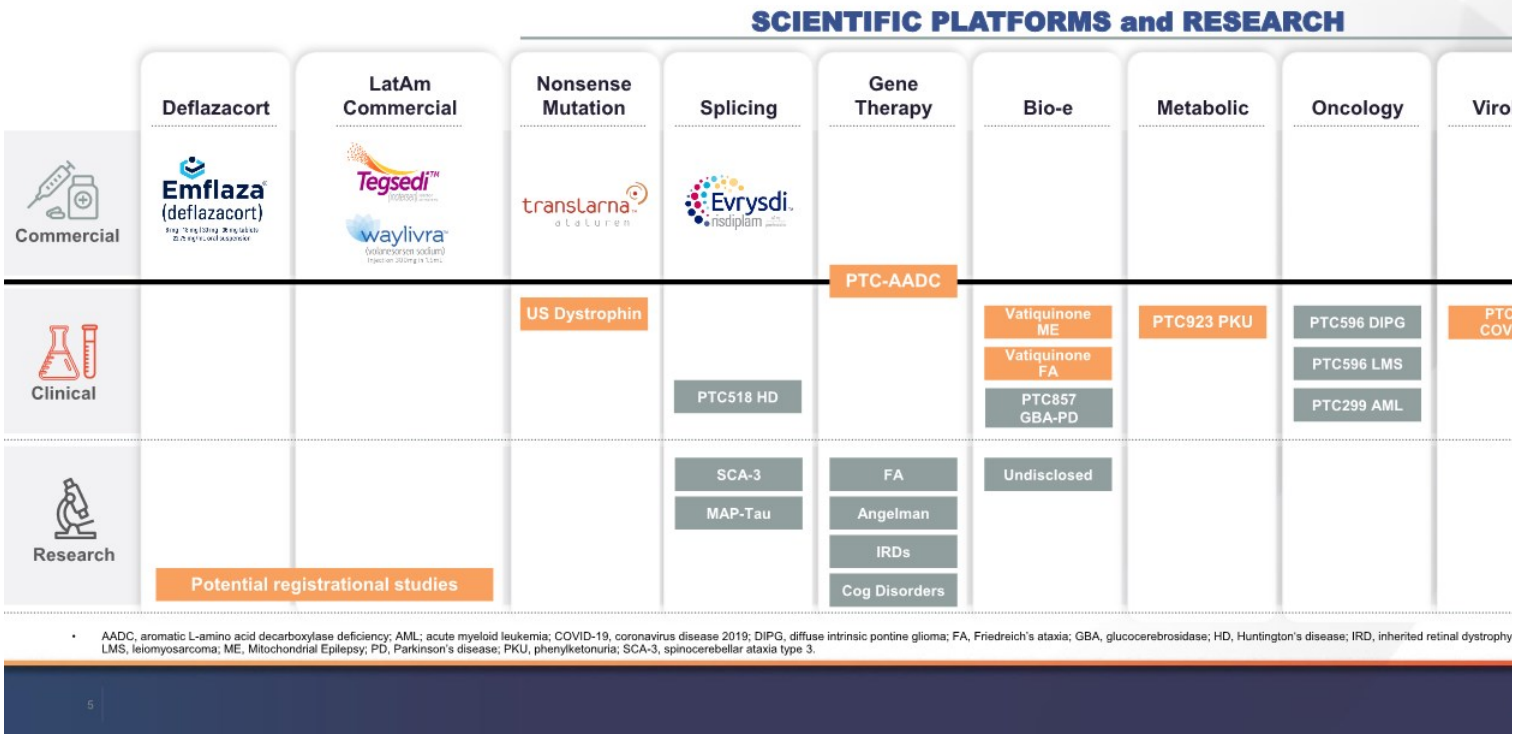
Commercial

- ✓ Broader patient access and continued geographic growth of Translarna
- ✓ Strong Emflaza growth; 38% YoY
- ✓ Evrysdi strong commercial launch

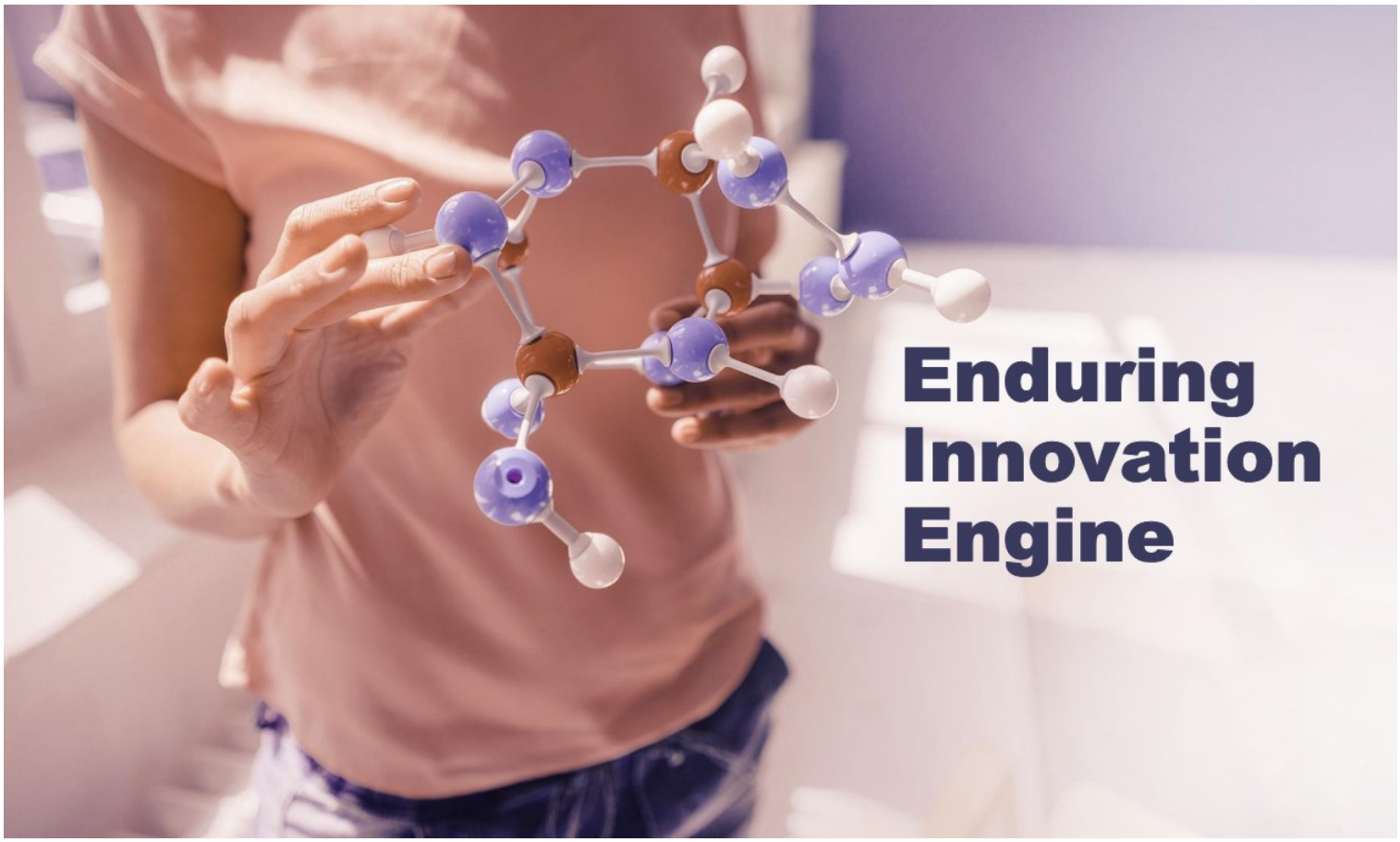
Financial

- ✓ Strengthened balance sheet; over $1B cash position
- ✓ 2020 unaudited: $333M Net Product Revenue; $331M DMD Franchise Net Product Revenue; $42.5M Roche Collaboration Revenue associated with Evrysdi regulatory and sales milestones

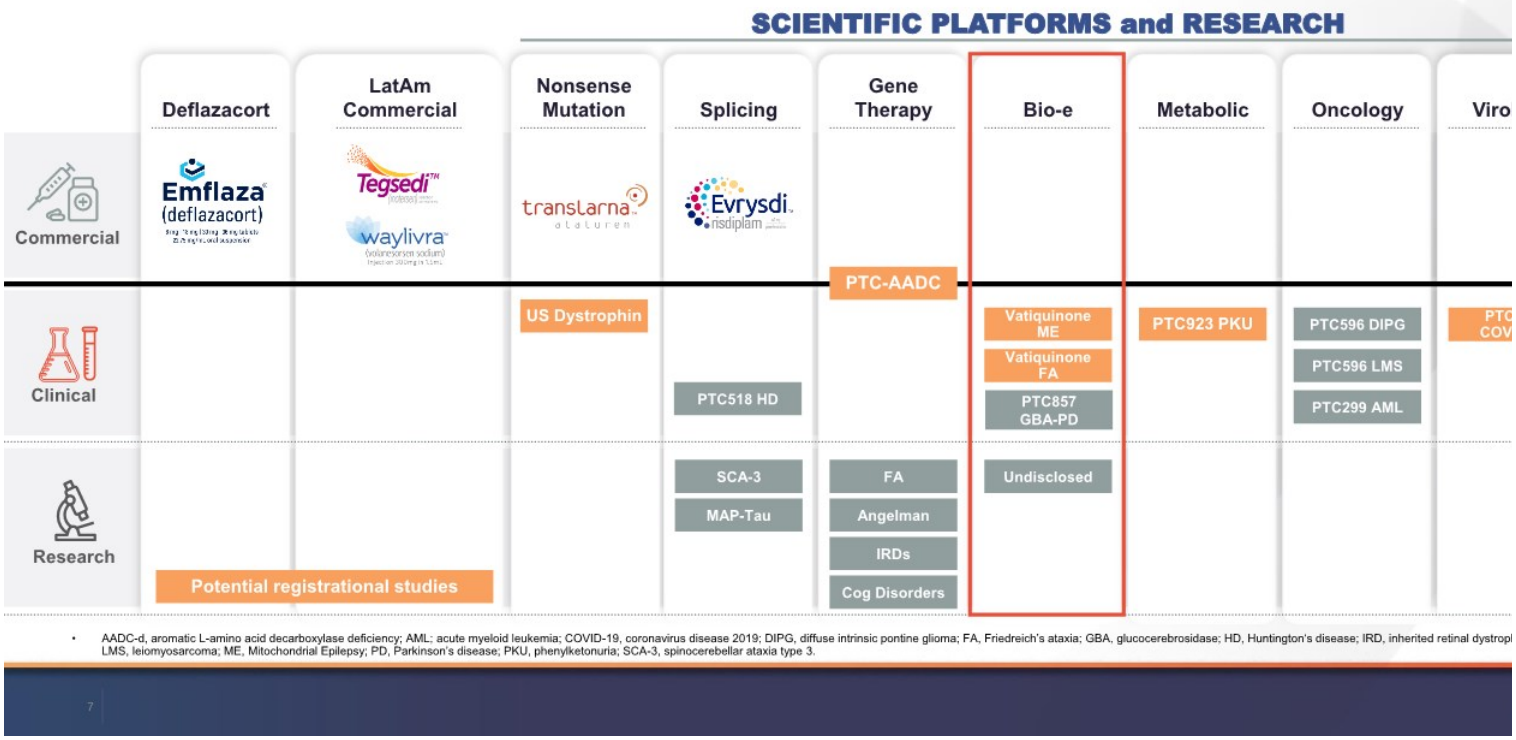
Diversified Platform Drives Strong Portfolio



- 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; GBA, glucocerebrosidase; HD, Huntington's disease; IRD, inherited retinal dystrophy LMS, leiomyosarcoma; ME, Mitochondrial Epilepsy; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.


Enduring
Innovation
Engine

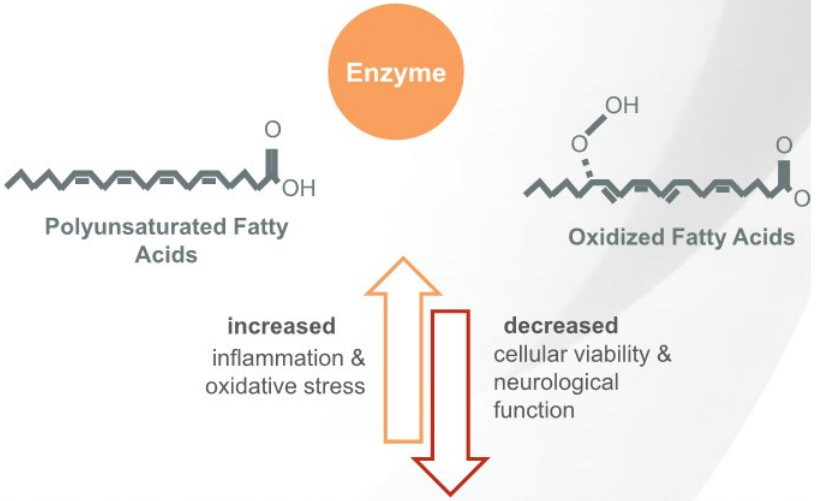
Diversified Platform Drives Strong Portfolio



- AADC-d, 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 dystrop LMS, leiomyosarcoma; ME, Mitochondrial Epilepsy; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

Bio-e Platform is a Novel Approach to Treating Rare Disorders

- Bio-e platform targets a family of oxidoreductase enzymes critical to generation and regulation of energy key to disease pathology
- Dysregulation of this pathway results in several CNS disease pathologies including epilepsy
- 15-lipoxygenase is a well-known regulator key to CNS and other diseases



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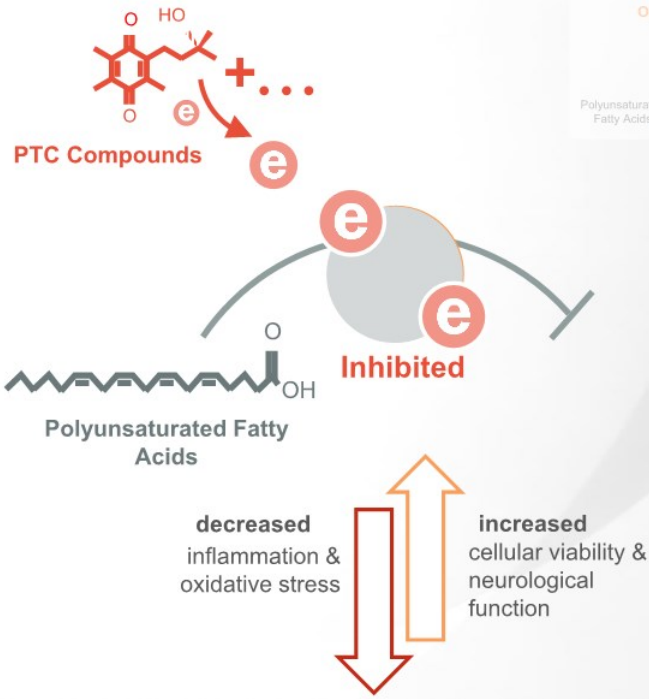


MIT-E: Registrational trial of vatiquinone for **Mitochondrial Epilepsy**

Mitochondrial Epilepsy is a Highly Morbid Disorder with High Unmet Medical Need

Disease

- Mitochondrial epilepsy is the highly morbid condition of refractory seizures in patients with inherited mitochondrial disease

Current Treatments

- No approved disease modifying treatments for mitochondrial epilepsy

Opportunity

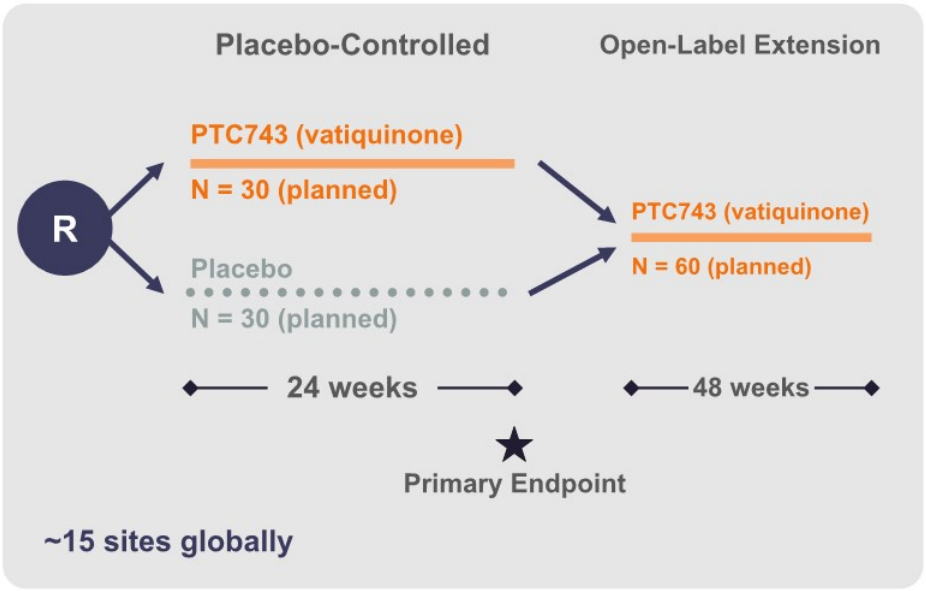
- Vatiquinone targets the energetic and oxidative stress pathways that underpin seizures in these patients

Vatiquinone Reduced Seizure Frequency and Improved Neurological Function in Mitochondrial Epilepsy Patients

Data from previous studies demonstrate a positive effect on seizures and seizure related-morbidity across multiple disease subtypes

- Reduction in seizure frequency
- Disruption of refractory status epilepticus
- Decrease in seizure-related hospitalizations
- Decrease in disease-related mortality risk

Vatiquinone has Potential to Show Clinically Differentiated Improvement for Mitochondrial Epilepsy Patients



Primary Endpoint

Change from baseline in frequency of observable motor seizures

Trial Status

- Enrolling
- Data expected 3Q 2022

MOVE-FA: Registrational trial of vatiquinone for **Friedreich Ataxia**

Friedreich Ataxia is a Highly Morbid, Neuromuscular Disorder with no Approved Therapy

Disease

- Friedreich ataxia (FA) is a rare, inherited, progressive disease resulting from mitochondrial dysfunction that mainly affects the central nervous system and the heart

Current Treatments

- No approved disease modifying therapies

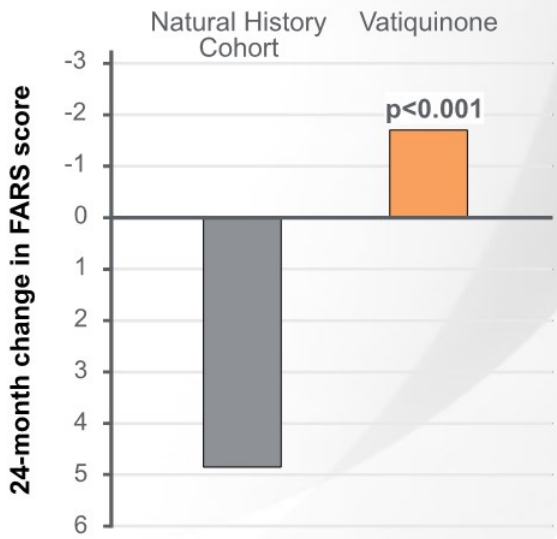
Opportunity

- Vatiquinone is a potent protector of oxidative stress-mediated cel death in FA patients

Vatiquinone Demonstrated Significant Improvement in Long-term Disease Severity & Neurological Function in Friedreich Ataxia Patients

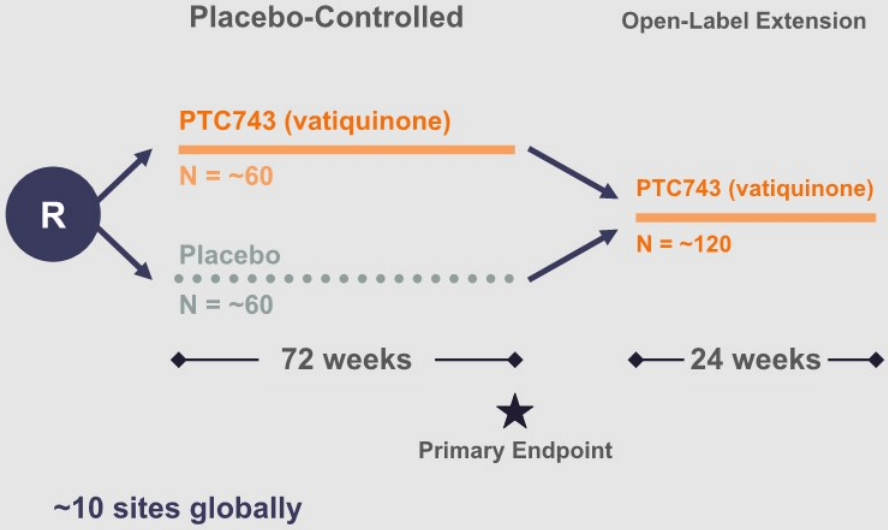
Clinical Study Summary

- Double-blind, placebo-controlled with delayed start
- N=63 subjects
- Three US clinical sites
- Key endpoint: FA disease rating scale (FARS)



Vatiquinone has the Potential to Provide Improvement in Neurological Function

MOVE-FA



~10 sites globally

Primary and Key Secondary Endpoints

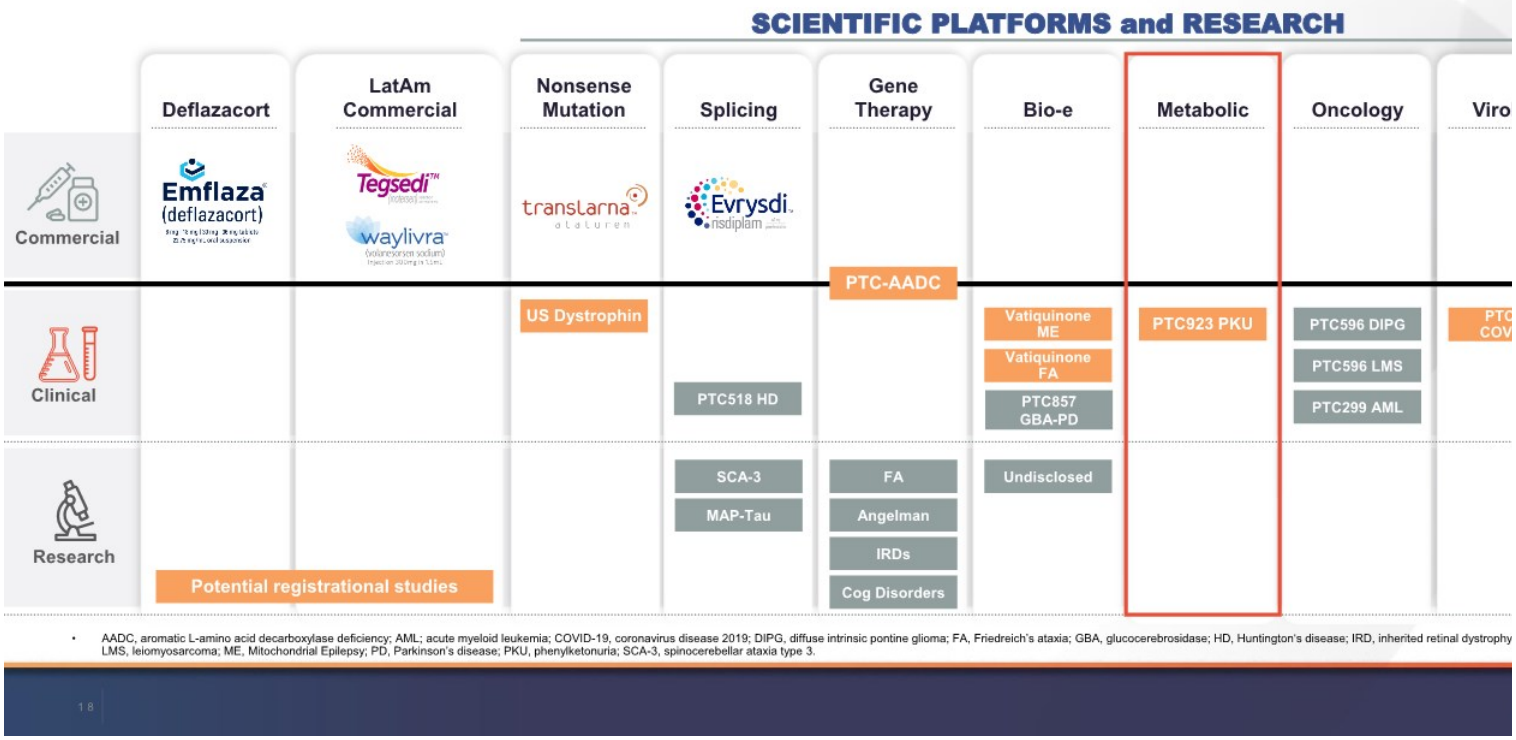
Change from baseline in the Modified FA Rating Scale (mFARS) Score at Week 72

Improvement in activities of daily living (FA-ADL)

Trial Status

- Enrolling
- Data expected in 2023

Diversified Platform Drives Strong Portfolio



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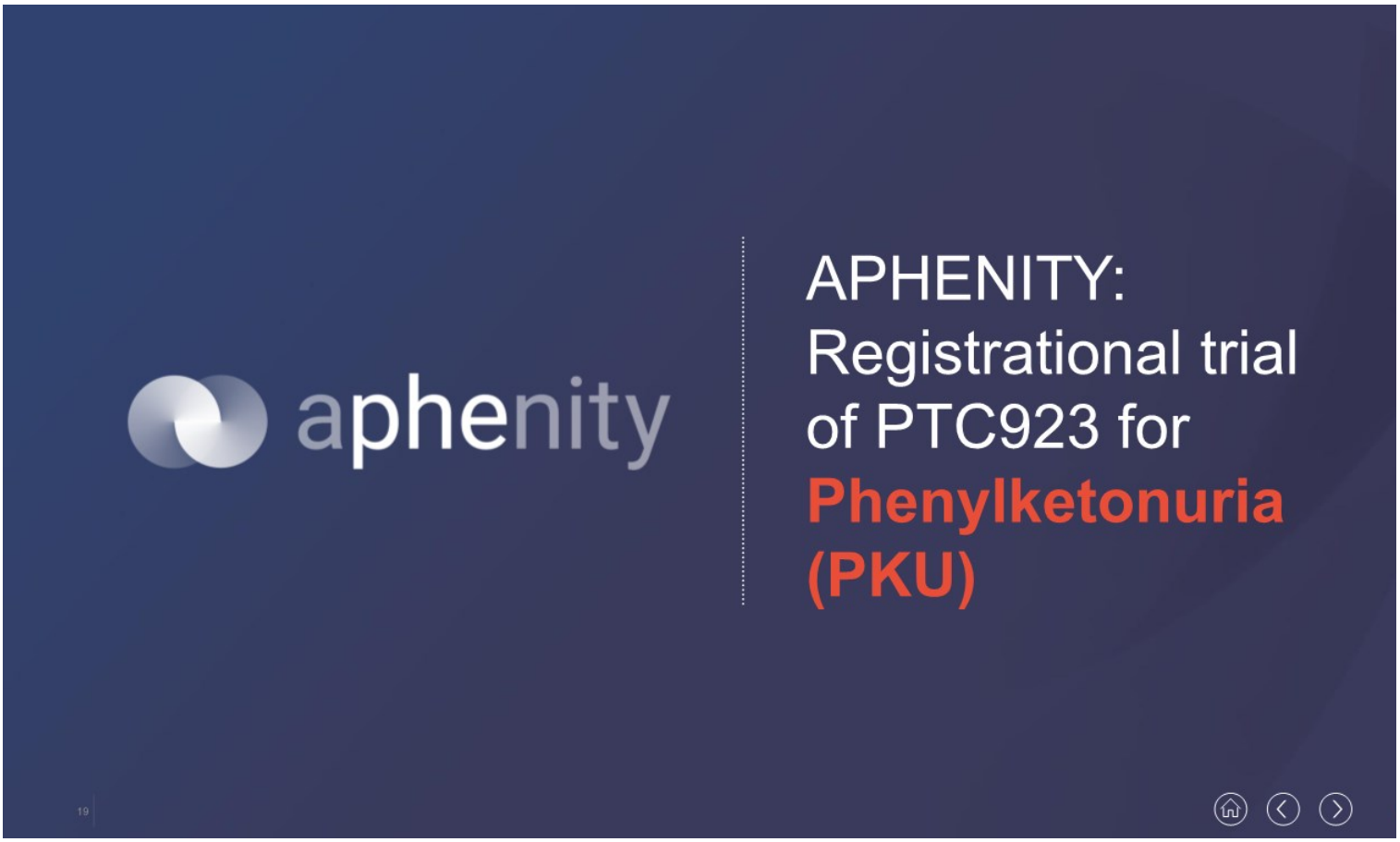

aphenity
APHENITY:
Registrational trial
of PTC923 for
Phenylketonuria
(PKU)
19

Phenylketonuria is a Serious Metabolic Condition with High Unmet Medical Need

Disease

- Phenylketonuria is a metabolic condition caused by mutations to phenylalanine hydroxylase that can lead to cognitive disabilities and seizur

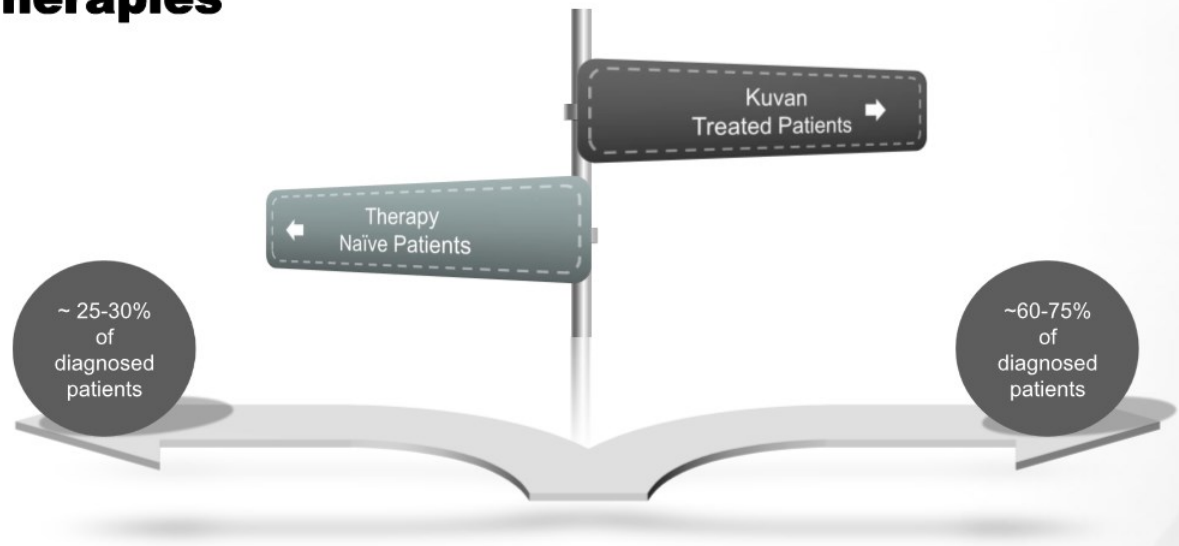
Current Treatments

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

Opportunity

- PTC923 can potentially treat a broad population and is more effective than exogenously administered synthetic BH4 in increasing the intracellular leve of natural tetrahydrobiopterin

Majority of PKU Patients not Addressed by Current Therapies



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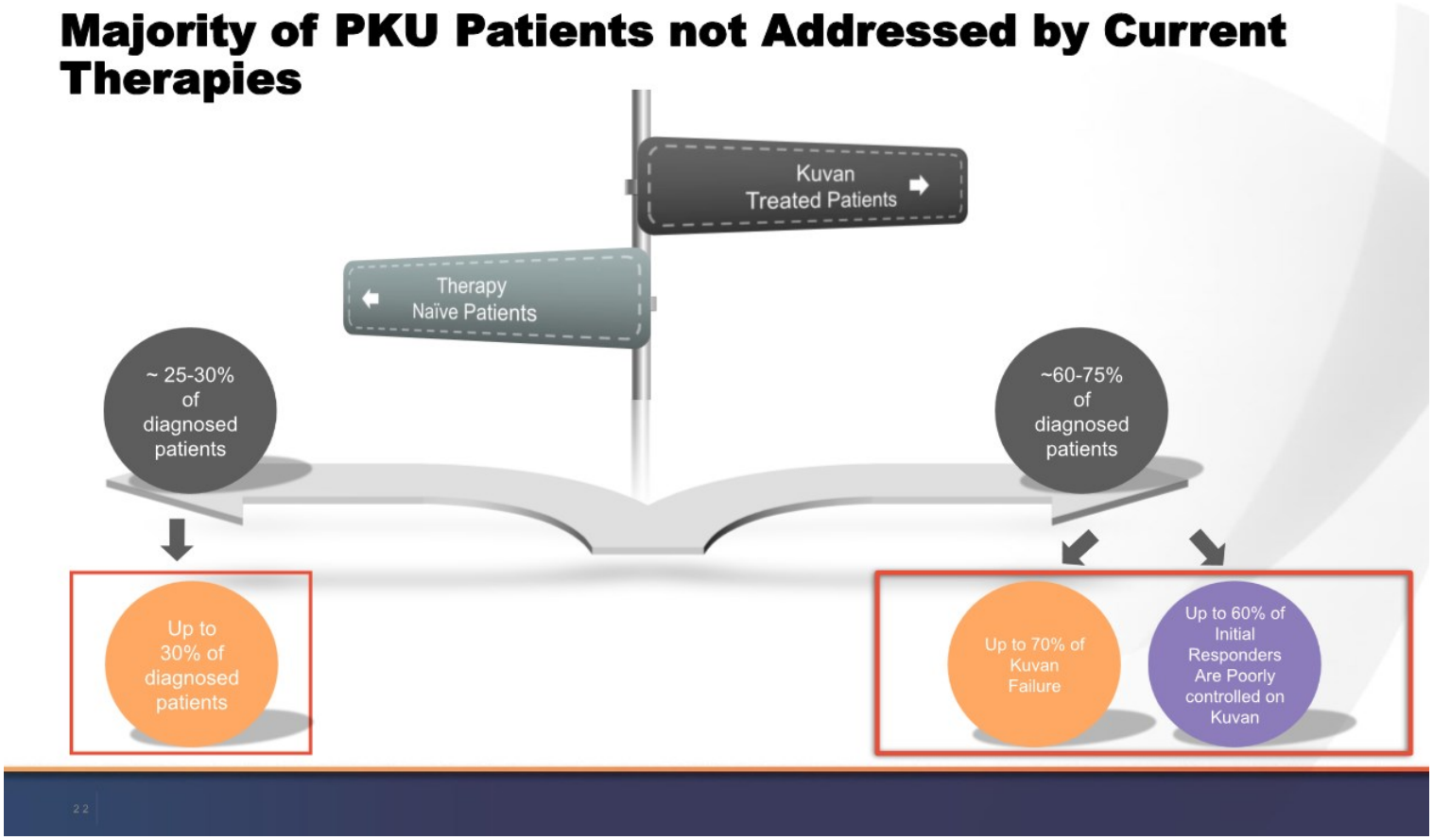
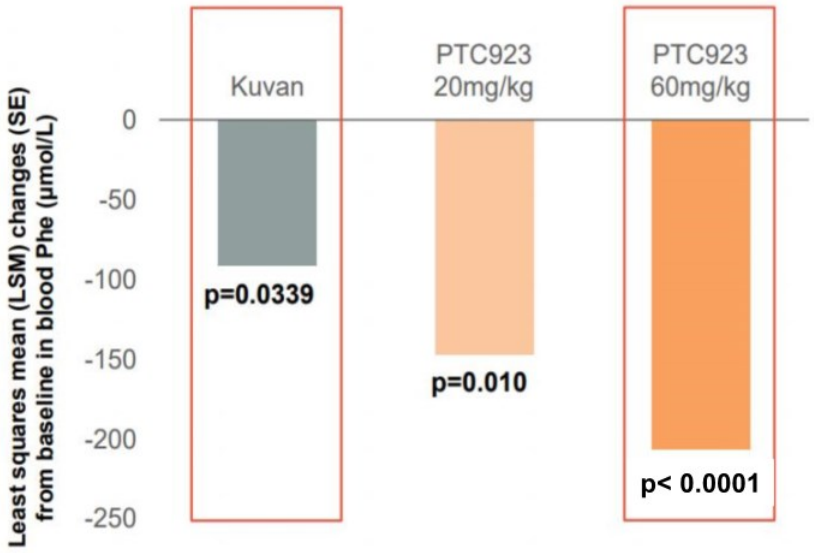


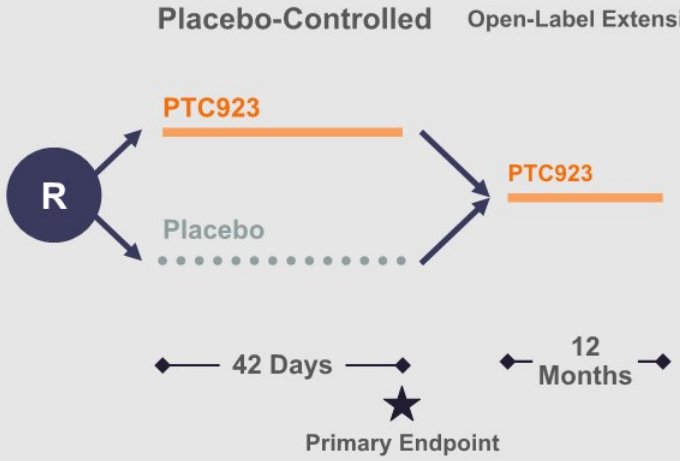
PTC923 Demonstrated Statistically Significant Differences in Reduction of Phenylalanine (Phe) Compared to Kuvan in Phase 2 Study



- 60 mg/kg/day most effective dose
- 114.9 greater µmol/L reduction of Phe with 60 mg/kg/day PTC923 relative to Kuvan; p=0.0098
- 50% increased responder rate with PTC923 as compared to Kuvan (12/19 vs. 8/19)

APHENITY is a Global Registrational Trial of PTC923 for Phenylketonuria

aphenity



~12 sites in 15-19 countries

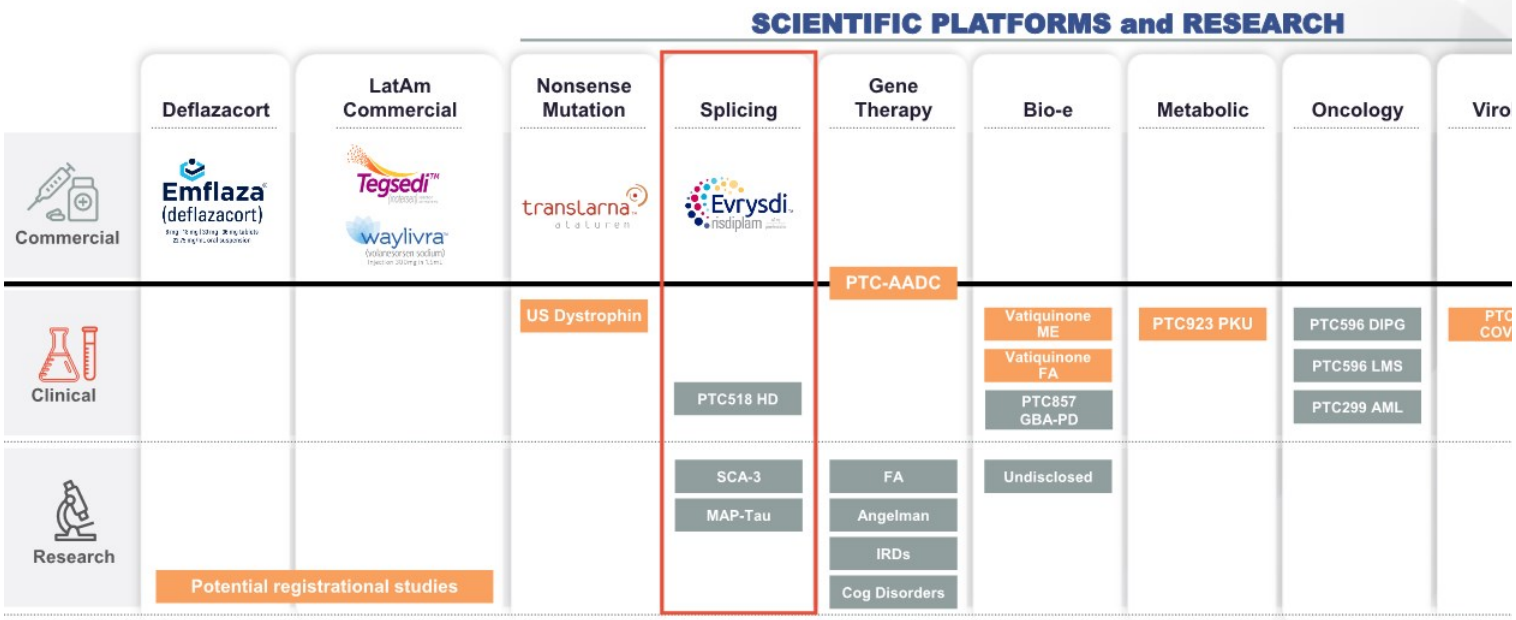
Primary Endpoint

Reduction in blood of phenylalanine hydroxylase levels

Trial Status

- Initiating in mid-2021
- Data expected YE 2022

Diversified Platform Drives Strong Portfolio



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PTC518: Program for **Huntington Disease**

Huntington Disease is a Debilitating Neurological Disorder with No Available Disease Modifying Treatments

Disease

- Huntington disease is a progressive brain disorder that causes uncontrolled movements and cognitive loss

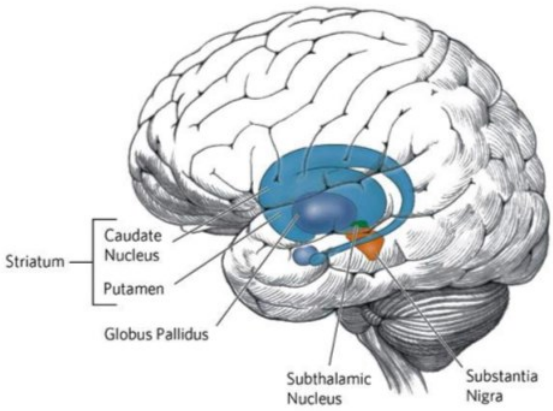
Current Treatments

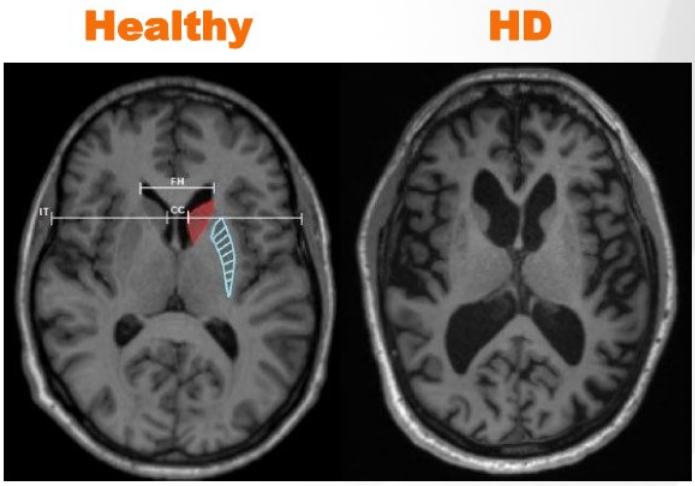
- No approved disease modifying therapies

Opportunity

- PTC518 reduces HTT protein in Huntington disease

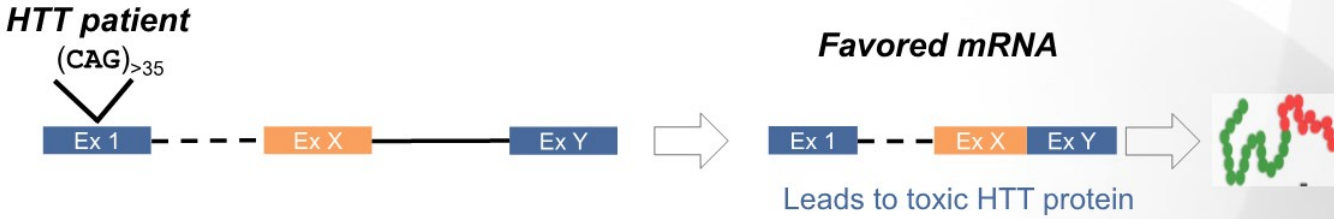
Small Molecules Have a Critical Advantage for Pan Brain Distribution





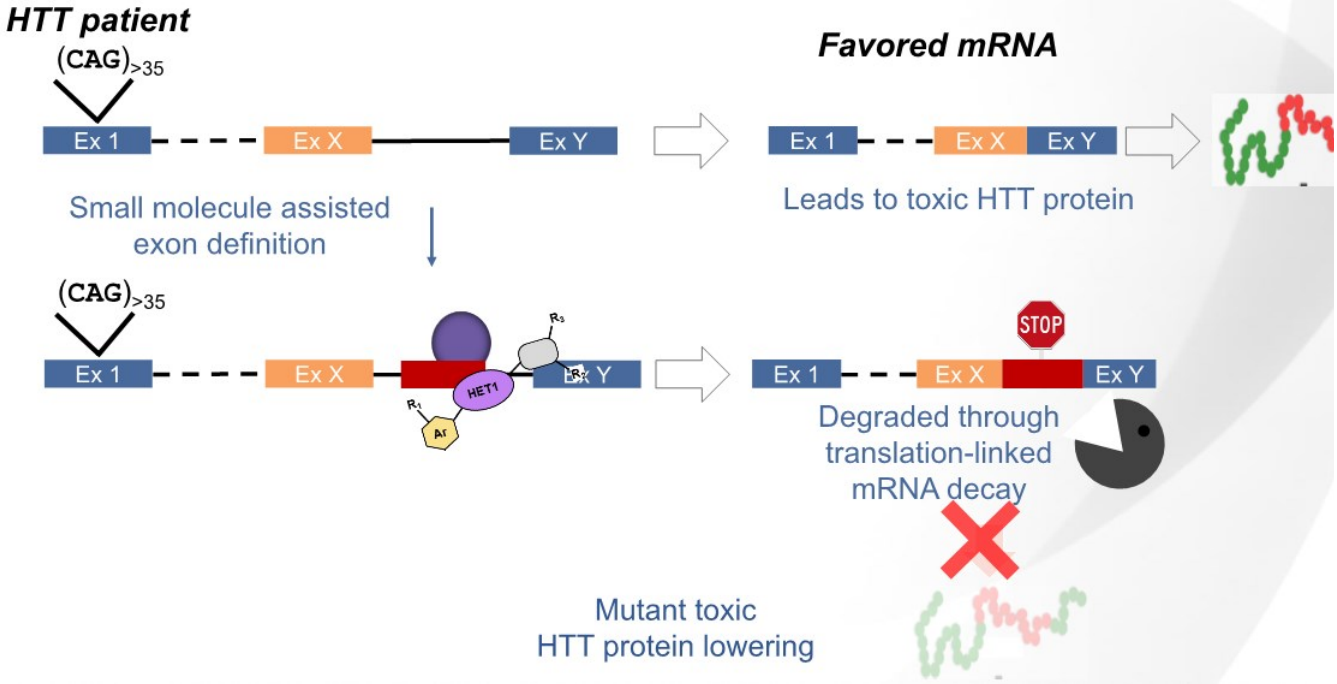
 Goh et al. *Aus Psychiatry*. 2018

Identification of a Novel Approach to Lower HTT



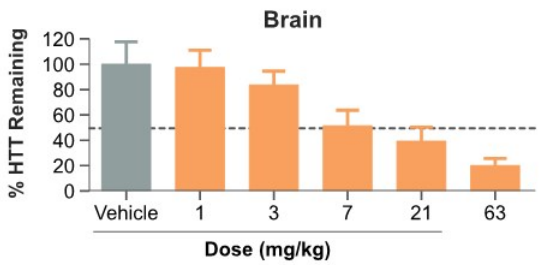
Leads to toxic HTT protein

Identification of a Novel Approach to Lower HTT



PTC518 Has Broad Tissue Distribution with Strong Correlation between Brain and Blood

Dose dependent HTT lowering in the brain in BACHD mice



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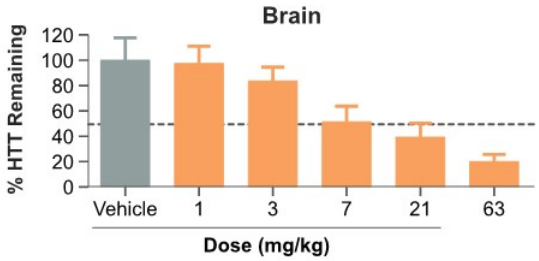
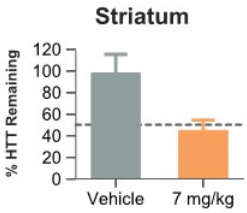
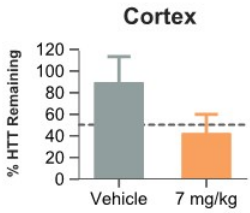
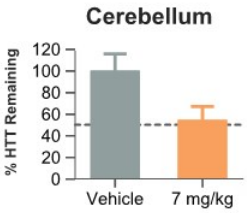
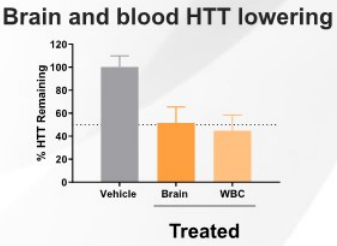


Measurements demonstrate uniform HTT lowering across brain regions with ~1:1 brain and blood concentration



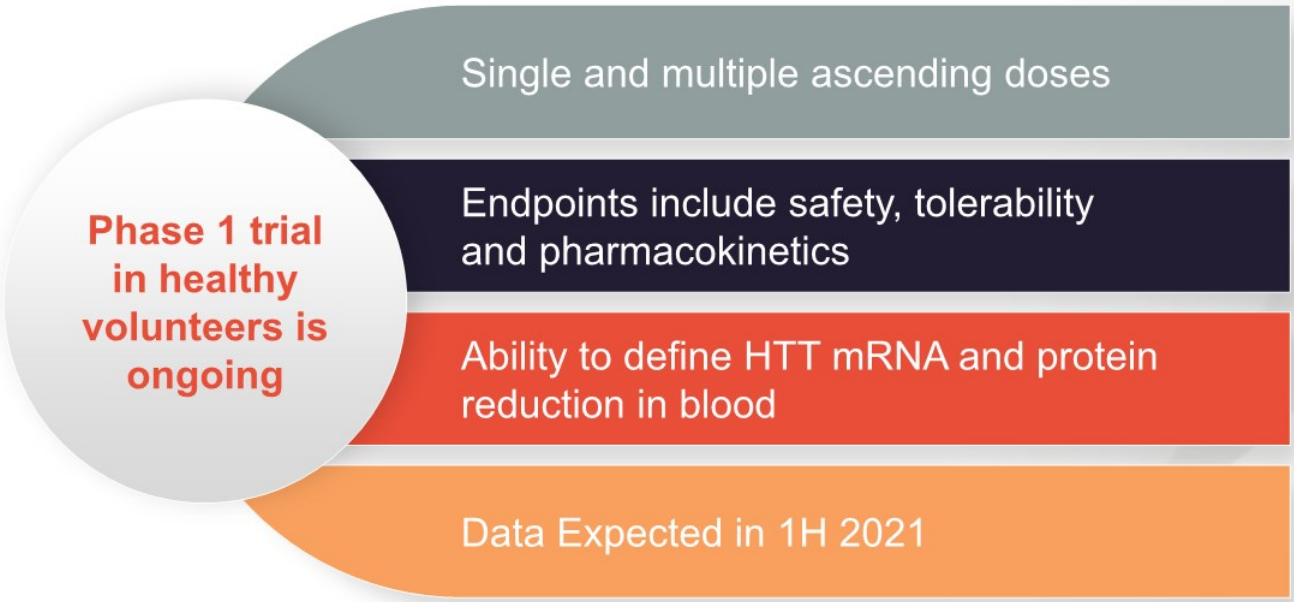




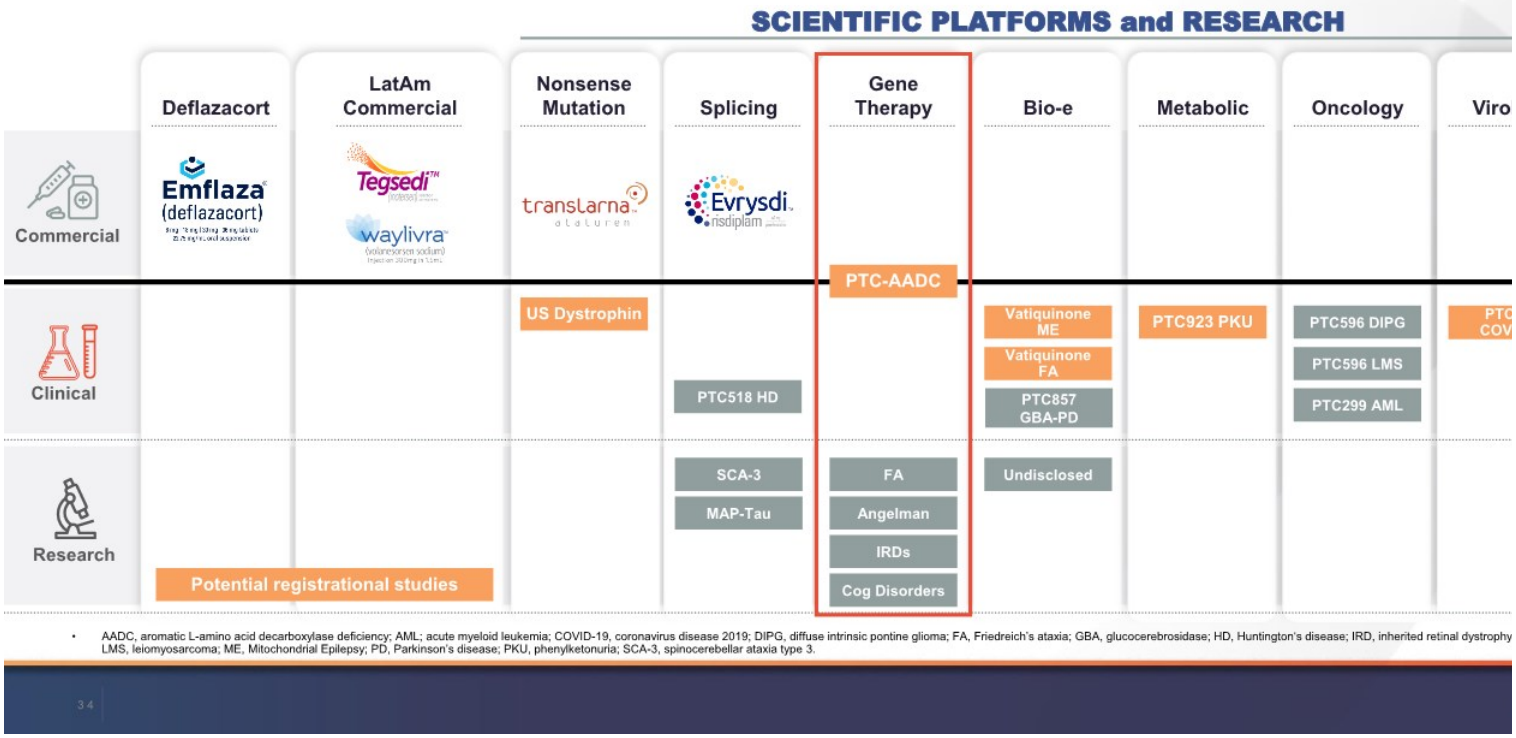


*Data on file from multiple studies

Potential Proof of Concept: Ability to Define HTT mRNA and Protein Reduction



Diversified Platform Drives Strong Portfolio



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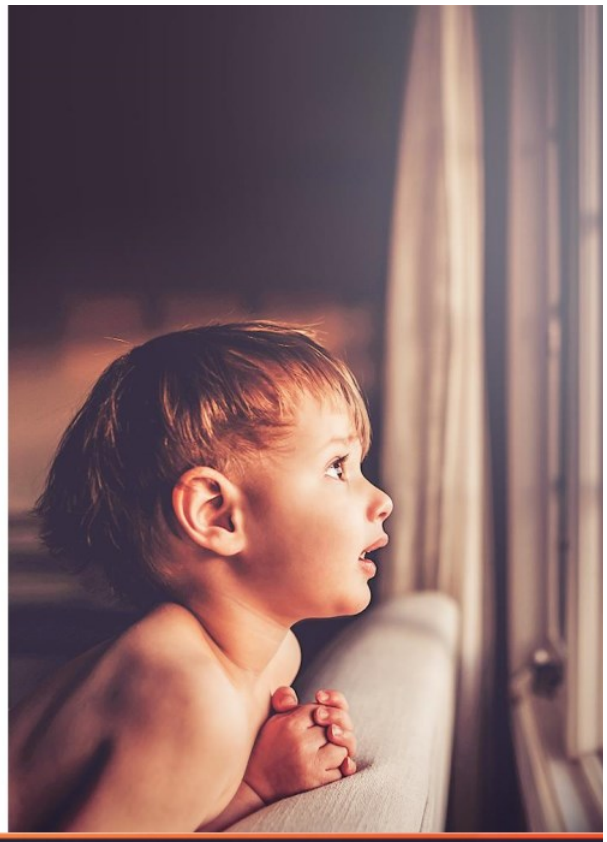
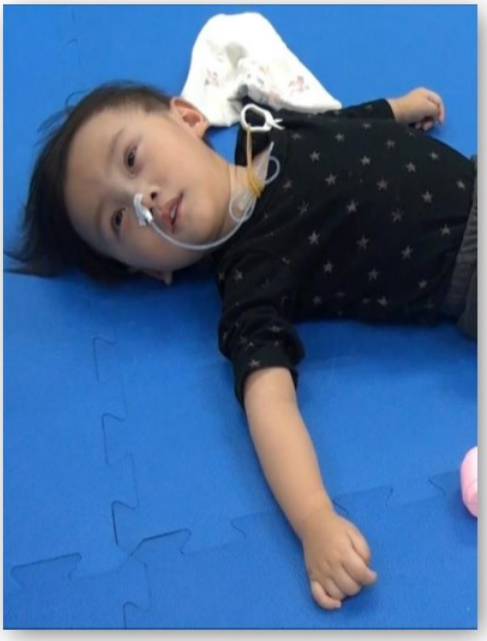
Treating Rare Monogenic Disorders with Targeted Gene Therapy

Potential advantages of targeted therapy

- Local administration lowers systemic immunogenicity and exposure
- Low turnover cells may lead to improved durability
- Leveraging stereotactic neurosurgery technologies to enable precise and accurate delivery
- Lead treatment for AADC-d, pipeline includes FA and AS



Gene Therapy 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

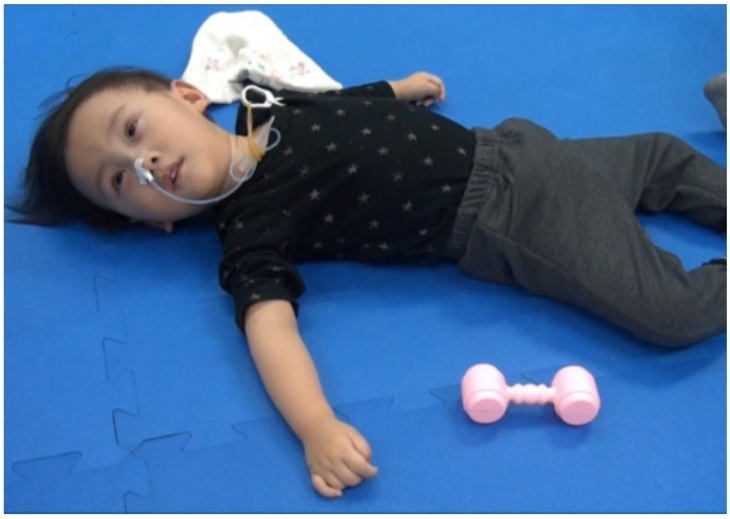
- No disease modifying therapies approved

Opportunity

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

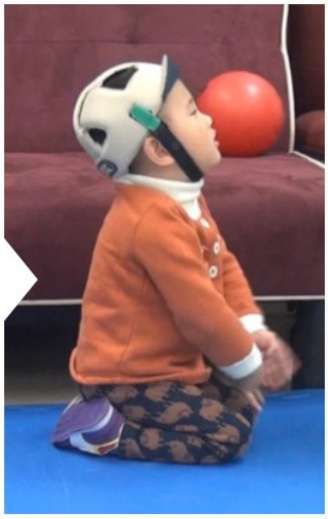
PTC-AADC Treated Patients Make Significant and Sustainable Progress

Untreated



Age 2

Post-Treatment



Age 3

Age 4.5

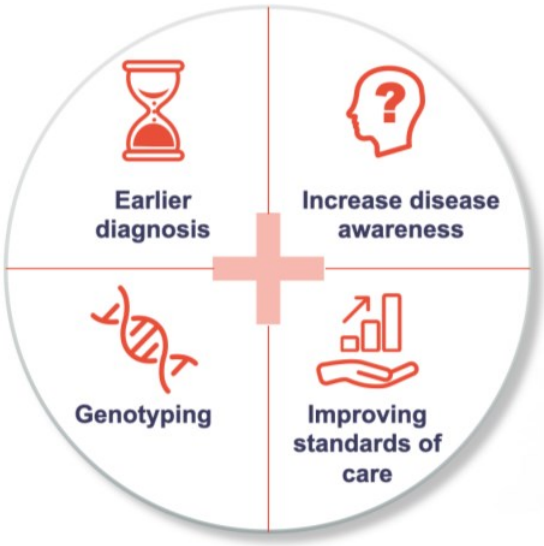
Preparing for PTC's First Gene Therapy Launch

EU Regulatory	US Regulatory	Treatment Centers	Patient Finding
PTC-AADC MAA submitted CHMP opinion expected in 1H21	PTC-AADC BLA submission expected in 1H21	Identification and preparation of expert pediatric neurosurgical centers	Ongoing patient finding targeting 300 patients identified at launch


Providing
Patients
Access to
Transformative
Treatments

Global Geographic Presence Supports Growing Product Portfolio

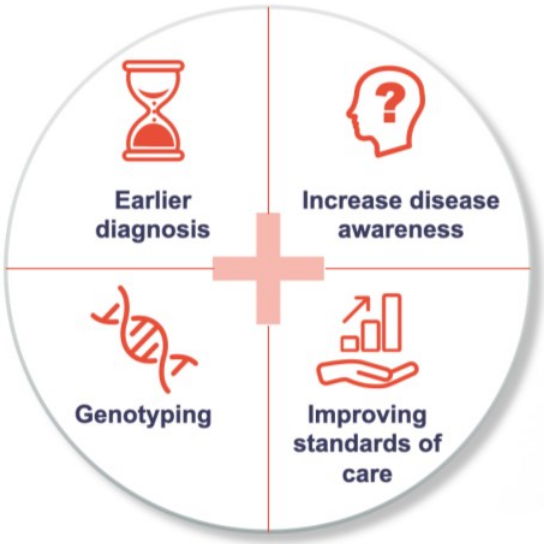
DMD Commercial Franchise – A Growing Global Business



DMD Commercial Franchise – A Growing Global Business

translarna™
ataluren

- YE 2020 unaudited revenue of $192M
- Treatment for nonsense mutation DMD for ages 2 and older
- Distributed in over 50 countries worldwide
- Dystrophin data in 1Q21 for potential US NDA submission



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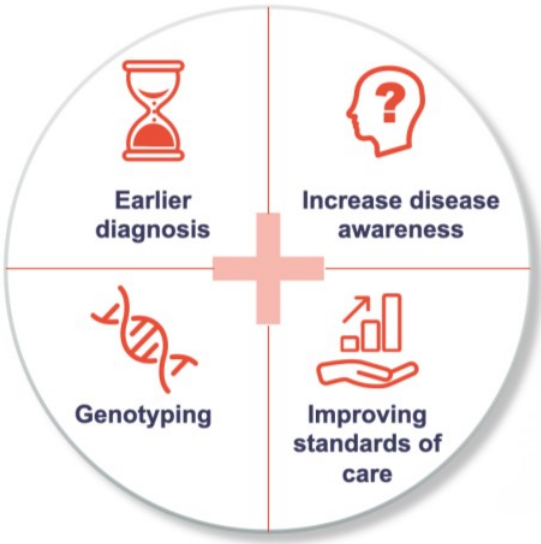
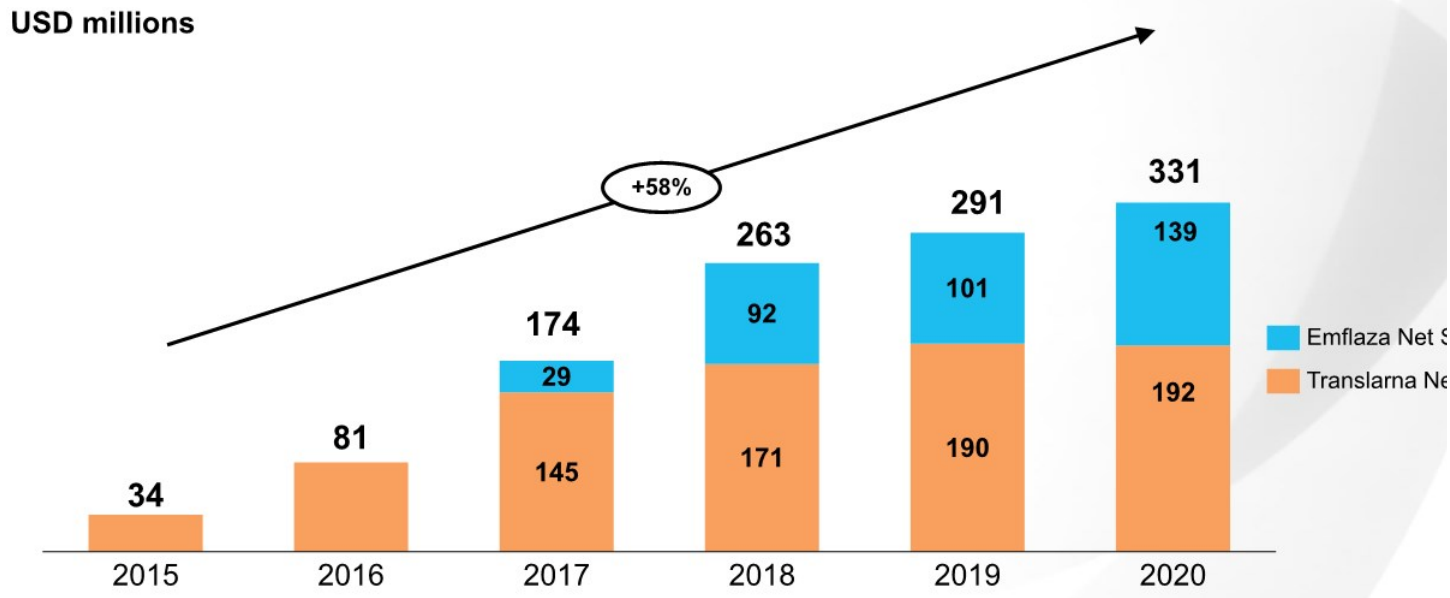


Emflaza®
(deflazacort)
6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

- YE 2020 unaudited revenue of $139M
- First and only corticosteroid approved for DMD
- Approved for all DMD patients in the US >2yrs
- Data from multiple publications demonstrate Emflaza's clinical benefit over prednisone

Continued Strong DMD Franchise Growth



Evrysdi's Strong Global Launch Brings Therapy to SMA Patients with High Unmet Need

Patients treated across all SMA types

Patients are treatment-naïve or switching from both Spinraza and Zolgensma

Broadest range of age treated

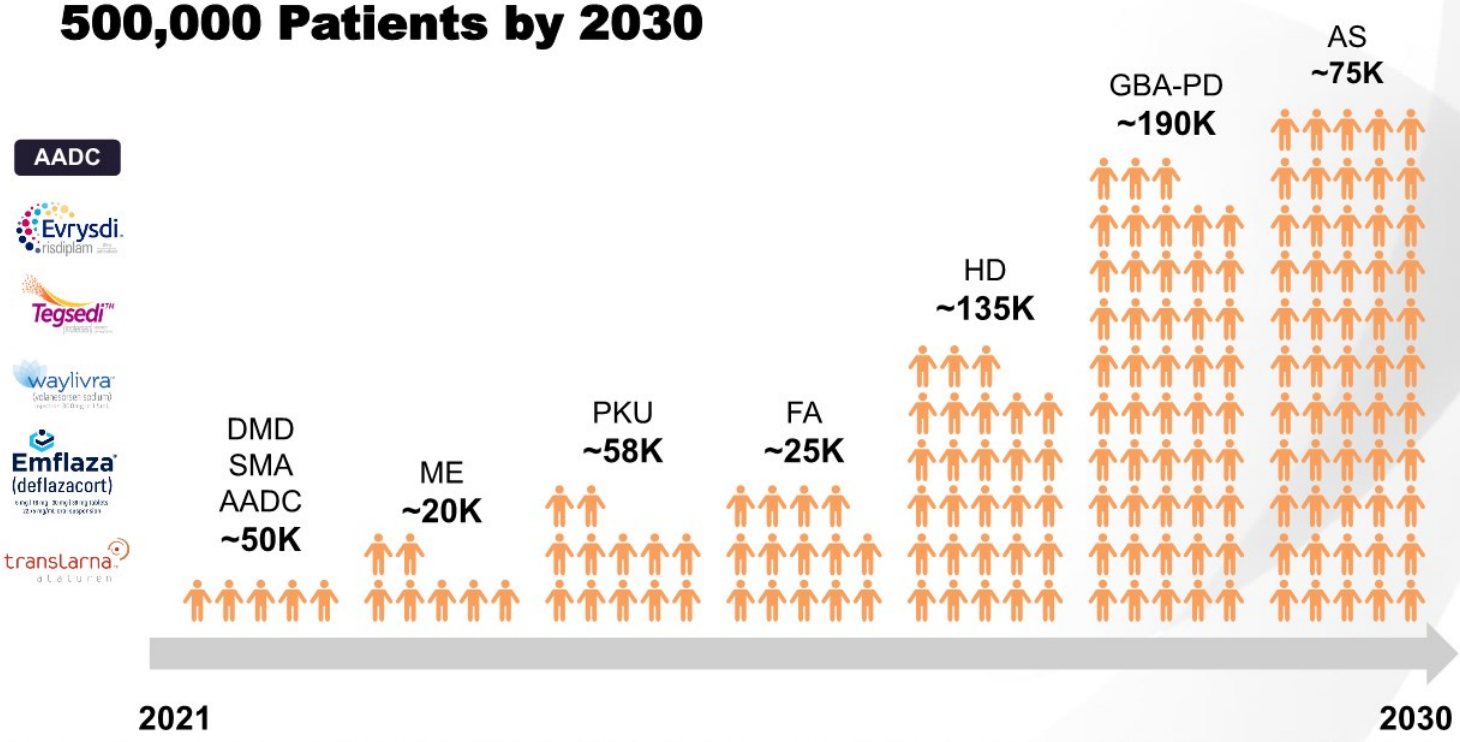
Differentiated product for SMA patients

- The first at-home, oral treatment for SMA
- Global approvals and regulatory submissions
- CHMP opinion expected in 1H 2021
- Under priority review in Japan

Significant milestones ahead

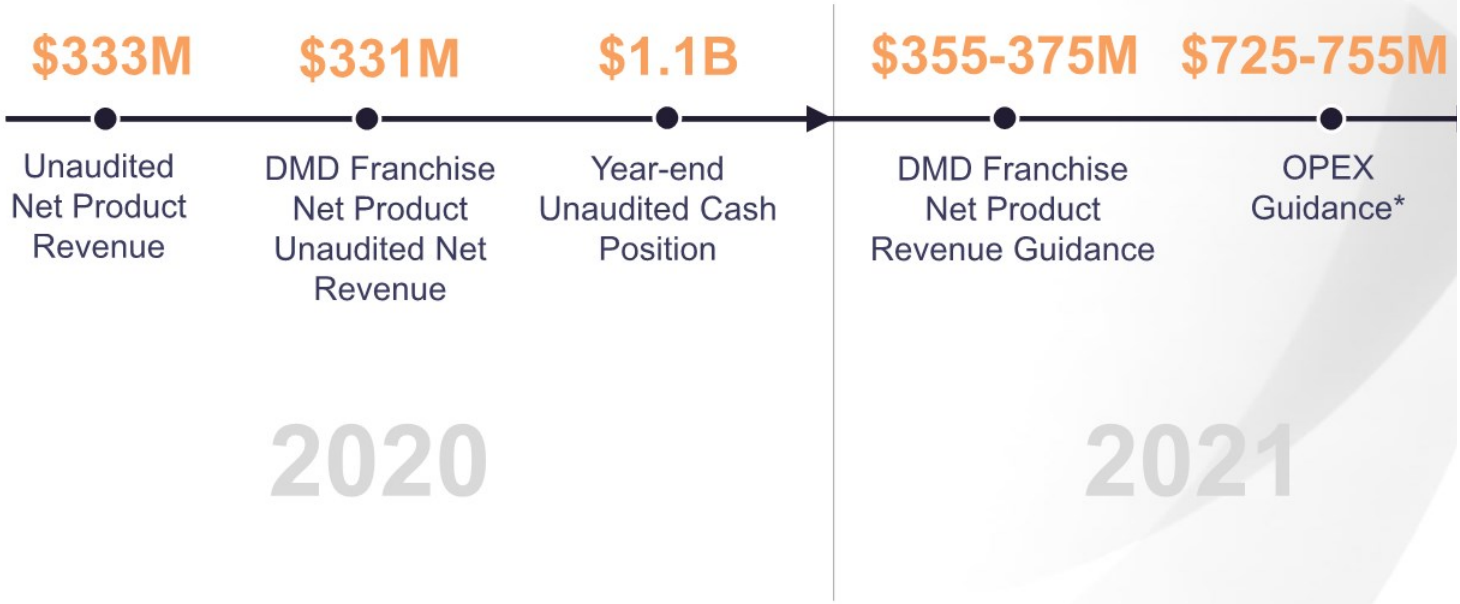
- Potential $355 million in sales and regulatory milestones
- ~15% tiered royalty on global sales

Multiple Platforms Provide Opportunity to Treat Over 500,000 Patients by 2030



Strong Financial Performance Supports Future Growth

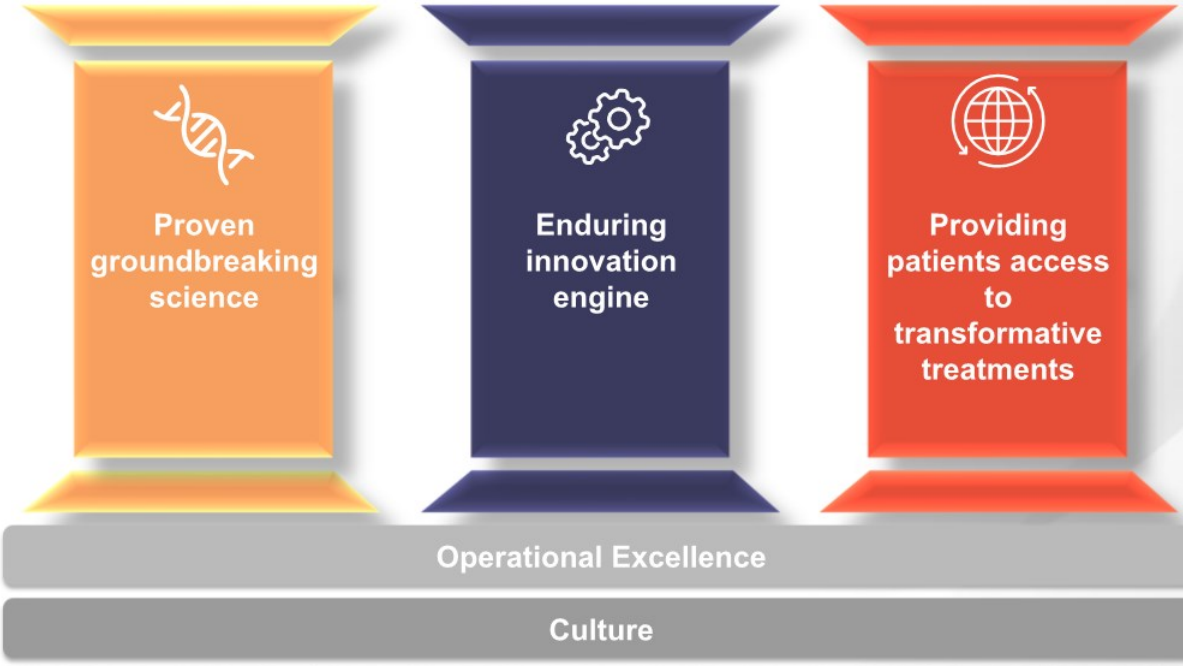
Strong Financial Performance Supports Future Growth



*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $100 million. GAAP R&D and SG&A expense for the full year 2021 is anticipated to be between $825 and $855 million.

2021 Potential Milestones to Generate Value

PTC has Built a Strong, Sustainable Company to Execute on our Mission



Translating Science to Transform Lives

PTC THERAPEUTICS