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 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** (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 10, 2022, 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, 2021, including that the Company expects to report (i) total unaudited net revenue of approximately $536 million, (ii) total unaudited net product revenue of approximately $429 million, (iii) net product revenue for Translarna™ (ataluren) of approximately $236 million, with approximately $70 million in revenue in the fourth quarter of 2021, and net product revenue for Emflaza® (deflazacort) of approximately $188 million, with approximately $48 million in revenue in the fourth quarter of 2021, (iv) collaboration and royalty revenue associated with Evrysdi of approximately $107 million and (v) ending cash, cash equivalents and marketable securities of approximately $773 million. Final results are subject to completion of the Company's year-end audit.

Item 5.02. Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.

On January 7, 2022 the Board of Directors of the Company promoted Matthew Klein, the Company's Chief Development Officer, to the role of Chief Operating Officer, effective immediately. Dr. Klein, 50, has been the Company's Chief Development Officer since April 2020. Dr. Klein joined the Company in October 2019 as Global Head Gene and Mitochondrial Therapies and became Global Head Clinical Development in March 2020. Prior to joining the Company, Dr. Klein was Chief Executive Officer of BioElectron Technology Corporation ("BioElectron") from 2018 to 2019, and served as a board member of BioElectron from 2018 to 2020. Dr. Klein served as the Chief Medical Officer of BioElectron from 2013 to 2019 and was Senior Vice President, Clinical Science at BioElectron from 2012 to 2013. Dr. Klein has also served as a member of the board of directors of ClearPoint Neuro, Inc., a Nasdaq-listed company, since 2020 as our director designee. Dr. Klein has a BA from the University of Pennsylvania, an MD from Yale University School of Medicine and an MS in epidemiology from the University of Washington School of Public Health.

In connection with Dr. Klein's promotion, his base salary was increased to $600,000 annually, with a target bonus of 50% of annual salaried earnings in accordance with the terms of the Company's annual incentive compensation plan. Dr. Klein also received, pursuant to the Company's 2013 Long Term Incentive Plan, a one-time grant of 50,000 stock options (the "Options") to purchase shares of the Company's common stock and 20,000 restricted stock units (the "RSUs"), each representing the right to receive one share of the Company's common stock upon vesting. The Options and RSUs will each vest over two years with 50% of each award vesting on January 7, 2023 and 50% of each award vesting on January 7, 2024.

As previously disclosed, on October 25, 2019, the Company completed the acquisition (the "Acquisition") of substantially all of the assets of BioElectron pursuant to an Asset Purchase Agreement by and between the Company and BioElectron, dated October 1, 2019 (the "Asset Purchase Agreement").

Upon the closing of the Acquisition, the Company paid to BioElectron total upfront consideration of $10.0 million, funded with cash on hand, less (i) transaction expenses incurred by BioElectron, (ii) the amount of outstanding indebtedness of BioElectron and (iii) $1.5 million to be held in an escrow account to secure potential indemnification obligations owed to the Company. Subject to the terms and conditions of the Asset Purchase Agreement, BioElectron may become entitled to receive contingent milestone payments of up to $200.0 million (in cash or in shares of our common stock, as determined by the Company) from the Company based on the achievement of certain regulatory and net sales milestones. Subject to the terms and conditions of the Asset Purchase Agreement, BioElectron may also become entitled to receive contingent payments of a low single digit percentage of net sales of certain products.

At the time of the Acquisition, Dr. Klein was the Chief Executive Officer and director of BioElectron, and was, and remains as of the date of this Current Report on Form 8-K, a shareholder of BioElectron, owning approximately six percent of its outstanding shares. As a shareholder of BioElectron, Dr. Klein is entitled to receive a portion of any payments made to BioElectron pursuant to the Asset Purchase Agreement. Dr. Klein was not a related party at the time of the Acquisition.

Dr. Klein does not have a family relationship with any of the Company's officers or directors.

Item 7.01. Regulation FD Disclosure.

On January 10, 2022, the Company also announced financial guidance for its fiscal year ending December 31, 2022 in the press release, including that the Company anticipates (i) full-year total revenues to be between $700 and $750 million (ii) full-year net product revenues for the Duchenne muscular dystrophy franchise to be between $475 and $495 million and (iii) GAAP R&D and SG&A expense for the full year 2022 to be between $915 and $965 million with non-GAAP R&D and SG&A expense for the full year 2022 to be between $800 and $850 million, excluding estimated non-cash, stock-based compensation expense of approximately $115 million.

The Company announced that on Monday, January 10, 2022 at 7:30 am EST at the 40th Annual J.P. Morgan Virtual Healthcare Conference, the Company will present its 2022 strategic priorities, preliminary 2021 financial results, and 2022 financial guidance. The presentation will be webcast live on the Events and Presentations page under the Investors section of the Company's website.

A copy of the press release, which also announced the appointment of Dr. Klein, is attached to this Current Report on Form 8-K as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

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 SG&A 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 the Company and the Company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP.

The information set forth in or incorporated by reference into Item 2.02 or this Item 7.01, 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. 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

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 2021 and financial guidance for fiscal year 2022. 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 2021 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2022; 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, 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.

Item 9.01. Financial Statements and Exhibits.

(d) Exhibits

Exhibit No.	Description
99.1	Press Release, dated January 10, 2022 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – 40th Annual J.P. Morgan Virtual 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 10, 2022

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

PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 40th Annual J.P. Morgan Healthcare Conference

- $536 million unaudited 2021 total revenue representing impressive 41% year-over-year growth -

- $700 - 750 million 2022 total revenue guidance -

- Results are expected in four registration-directed trials this year -

- Three additional registration-directed clinical trials expected to initiate in 2022 –

- Dr. Matthew Klein promoted to Chief Operating Officer and will continue to oversee the Development organization -

SOUTH PLAINFIELD, N.J., Jan. 10, 2022 – PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 40th Annual J.P. Morgan Healthcare Conference today, Monday Jan. 10th at 7:30am EST. Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, will provide an update on 2021 accomplishments and highlight upcoming potential value-creating milestones. Preliminary 2021 unaudited financial results and 2022 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 2021 Corporate Highlights:

- Unaudited net product revenue of $429 million in 2021 representing 29% year-over-year growth.
- Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $424 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in 2021. Cumulative net product revenue for Translarna exceeds $1 billion and Emflaza exceeds $500 million, since respective launches.
  - Translarna growth was driven by new patients and high compliance in existing geographies and continued geographic expansion.
  - Emflaza growth was due to continued new prescriptions, high compliance, less patient discontinuations and more favorable access.
- Evrysdi® (risdiplam) has shown continued rapid uptake in the United States and is now approved in all major markets including the European Union and Japan. Evrysdi is a product of the Spinal Muscular Atrophy (SMA) collaboration between PTC, the SMA Foundation and Roche.
- Waylivra® (volanesorsen), the only treatment for familial chylomicronemia syndrome was approved by Brazilian Health Regulatory Agency, Agência Nacional de Vigilância Sanitária (ANVISA), and both Waylivra and Tegsedi® (inotersen) received Category 1 classification from Câmara de Regulação do Mercado de Medicamentos - CMED (Drug Market Regulation Chamber) in Brazil. Category 1 classification is given to innovative treatments that provide greater efficacy than the standard of care and allows for pricing in line with international markets.
- PTC successfully advanced its clinical pipeline in 2021:
  - APHENITY, a Phase 3 registration-directed trial of PTC923 in phenylketonuria (PKU) was initiated.

- Phase 1 healthy volunteer trials of PTC518 and PTC857 were completed.
- Advanced the oncology platform with the completion of Phase 1b studies of unesbulin in leiomyosarcoma and diffuse intrinsic pontine glioma (DIPG).
- Completed enrollment for the vatiquinone MOVE-FA registration-directed trial with results expected in the second quarter of 2023.

2022 Potential Key Value-Creating Milestones:

- Results from Study 041 for Translarna are expected mid-year 2022 and if positive could support re-submission of a New Drug Application (NDA) to the Food and Drug Administration (FDA).
- Results for MIT-E, the registration-directed study of vatiquinone in mitochondrial disease associated seizures, are expected in the fourth quarter of 2022.
- Results are expected by year end 2022 for the Phase 3 registration-directed study, APHENITY, for PTC923 in patients with PKU.
- From the splicing platform, the PIVOT-HD Phase 2 study of PTC518 in Huntington's disease patients is planned to initiate in the first quarter of 2022.
- From the Bio-e platform, the registration-directed CardinALS study of PTC857 in amyotrophic lateral sclerosis (ALS) patients is expected to be initiated in the second quarter of 2022.
- Progress in the gene therapy platform is anticipated in 2022:
  - PTC expects an opinion from the European Medicine Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) for PTC-AADC, the first gene therapy for AADC deficiency, in April 2022.
  - Submission of a Biologics License Application (BLA) to the FDA for PTC-AADC is expected in the second quarter of 2022.

Preliminary Unaudited 2021 Financial Results:

- Total unaudited net revenue for full year 2021 was approximately $536 million.
- Total unaudited net product revenue for full year 2021 was approximately $429 million.
- DMD franchise revenue for year end 2021 included net product revenue for Translarna of approximately $236 million, with $70 million in revenue in the fourth quarter, and Emflaza of approximately $188 million, with $48 million in revenue in the fourth quarter.
- PTC expects to report approximately $107 million in 2021 collaboration and royalty revenue associated with Evrysdi.
- PTC expects to report 2021 year-end cash, cash equivalents and marketable securities of approximately $773 million.

PTC is currently in the process of finalizing its financial results for the 2021 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2021, subject to the completion of PTC's financial closing procedures.

2022 Financial Guidance:

- PTC anticipates total revenues for the full year 2022 to be between $700 and $750 million.
- PTC anticipates net product revenues for the DMD franchise for the full year 2022 to be between $475 and $495 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2022 to be between $915 and $965 million.

- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2022 to be between $800 and $850 million, excluding estimated non-cash, stock-based compensation expense of $115 million.

Non-GAAP Financial Measures:

In this press release, the financial results and financial guidance of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP financial measures exclude non-cash, stock-based compensation expense. These non-GAAP financial measures are provided as a complement to financial measures reported in GAAP because management uses these non-GAAP financial measures when assessing and identifying operational trends. In management's opinion, these non-GAAP financial measures are 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. Quantitative reconciliations of the non-GAAP financial measures to their respective closest equivalent GAAP financial measures are included in the table below.

PTC Therapeutics, Inc.

Reconciliation of GAAP to Non-GAAP Projected Full Year 2022 R&D and SG&A Expense

(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 915,000	$ 965,000
Less: projected non-cash, stock-based compensation expense	115,000	115,000
Projected non-GAAP R&D and SG&A expense	**$ 800,000**	**$ 850,000**

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 innovate to identify new therapies and to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines. PTC's mission is to provide access to best-in-class treatments for patients who have little to no treatment options. PTC's strategy is to leverage its strong scientific and clinical expertise and global commercial infrastructure to bring therapies to patients. PTC believes this allows it to maximize value for all its stakeholders. To learn more about PTC, please visit us at www.ptcbio.com and follow us on Instagram, Facebook, Twitter, and LinkedIn.

For More Information:

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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 "2022 Financial Guidance", including with respect to (i) 2022 total revenue guidance, (ii) 2022 net product revenue guidance for the DMD franchise and (iii) 2022 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 regulatory submissions 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 PTC's gene therapy platform, including any regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; the enrollment, conduct, and results of ongoing studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any regulatory submissions and commercialization with respect to Evrysdi; PTC's ability to utilize results from Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, to support a marketing approval for Translarna for the treatment of nmDMD in the United States; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA) and other regions, including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to fund, complete and timely submit to the EMA the results of Study 041, which is a specific obligation to continued marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the enrollment, conduct and results of PTC's clinical trial for emvododstat for COVID-19; significant business effects, including the effects of industry, market, economic,

political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the lease agreement for its leased biologics manufacturing facility; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, Evrysdi, Tegsedi, Waylivra or 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 2022
J.P. Morgan Healthcare Conference
Stuart W. Peltz, Ph.D., CEO

Forward Looking Statements

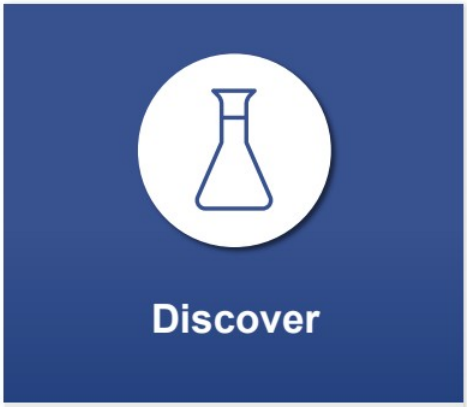
This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements o historic fact, are forward-looking statements, including statements with respect to preliminary unaudited 2021 financial information with respect to 2021 total net revenue and 2021 DMD franchise net product revenue, statements with respect to guidance relating to 2022 total net product revenue, 2022 DMD franchise net product revenue, 2022 operating expenditure guidance and future revenue guidance and statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and manufacturing capabilities; advancement 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, regula 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 manage 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, inc 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 appro and PTC's collaborators, contract research organizations, suppliers and manufacturers; the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to PTC's gene therapy platform, including any regulatory submissions and potential approv 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 a 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 cou delay or prevent further development under the program, including any regulatory submissions and commercialization with respect to Evrysdi; PTC's ability to utilize results from Study 041, a randomi 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, to support a marketing approval for Translarna for the treatment of nmDMD in the United States; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA) and other regions, including wheth 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 fun complete and timely submit to the EMA the results of Study 041, which is a specific obligation to continued marketing authorization in the EEA; expectations with respect to the commercialization of T and Waylivra; the enrollment, conduct and results of PTC's clinical trial for emvododstat for COVID-19; significant business effects, including the effects of industry, market, economic, political or regul 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 g 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 abilit 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 recen 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 v 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 presentation and PTC does not undertake or plan to update or revise any such forward-looking state 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

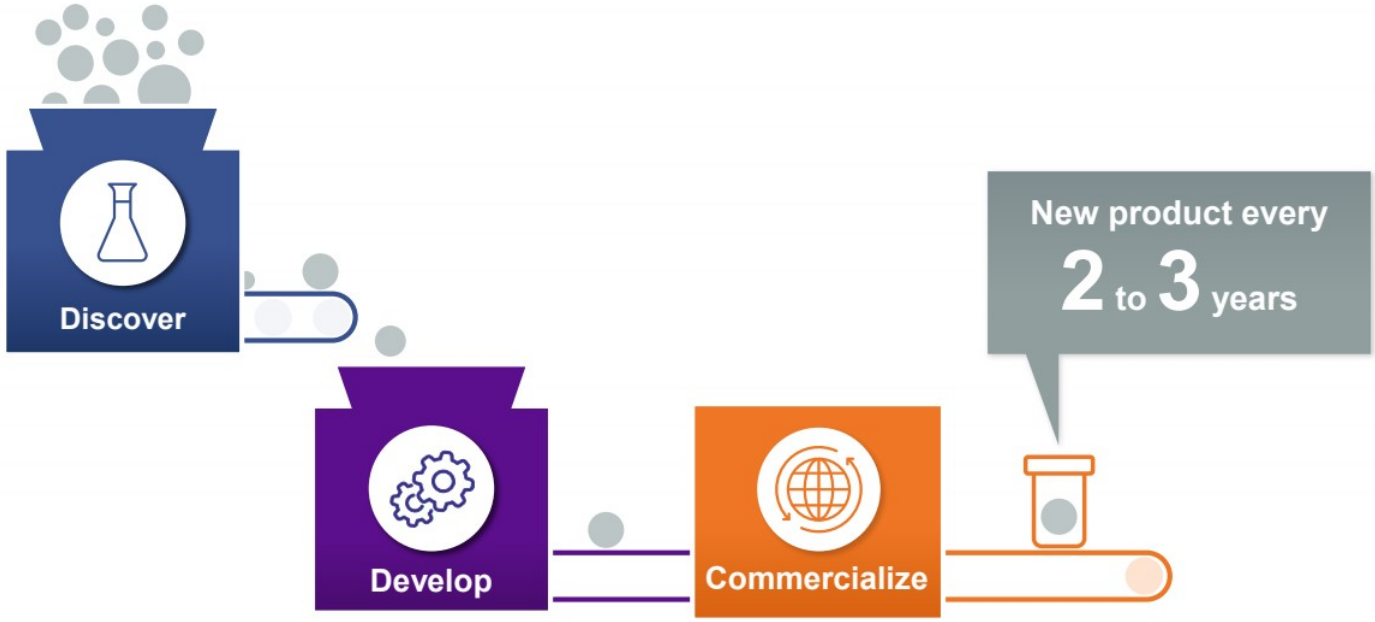


Proven groundbreaking science

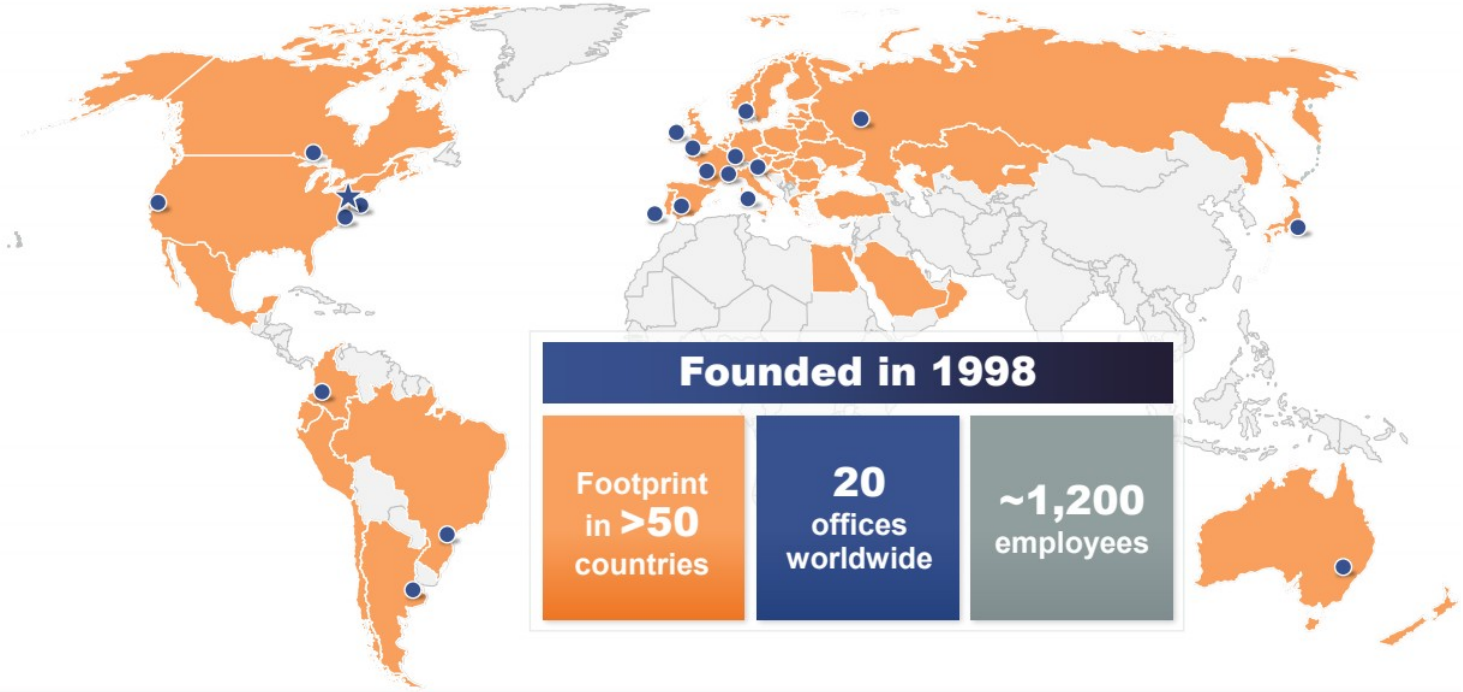
Enduring innovation engine

Providing patients with access to transformative treatments

Building a Pipeline to Produce a Therapy Every Three Years



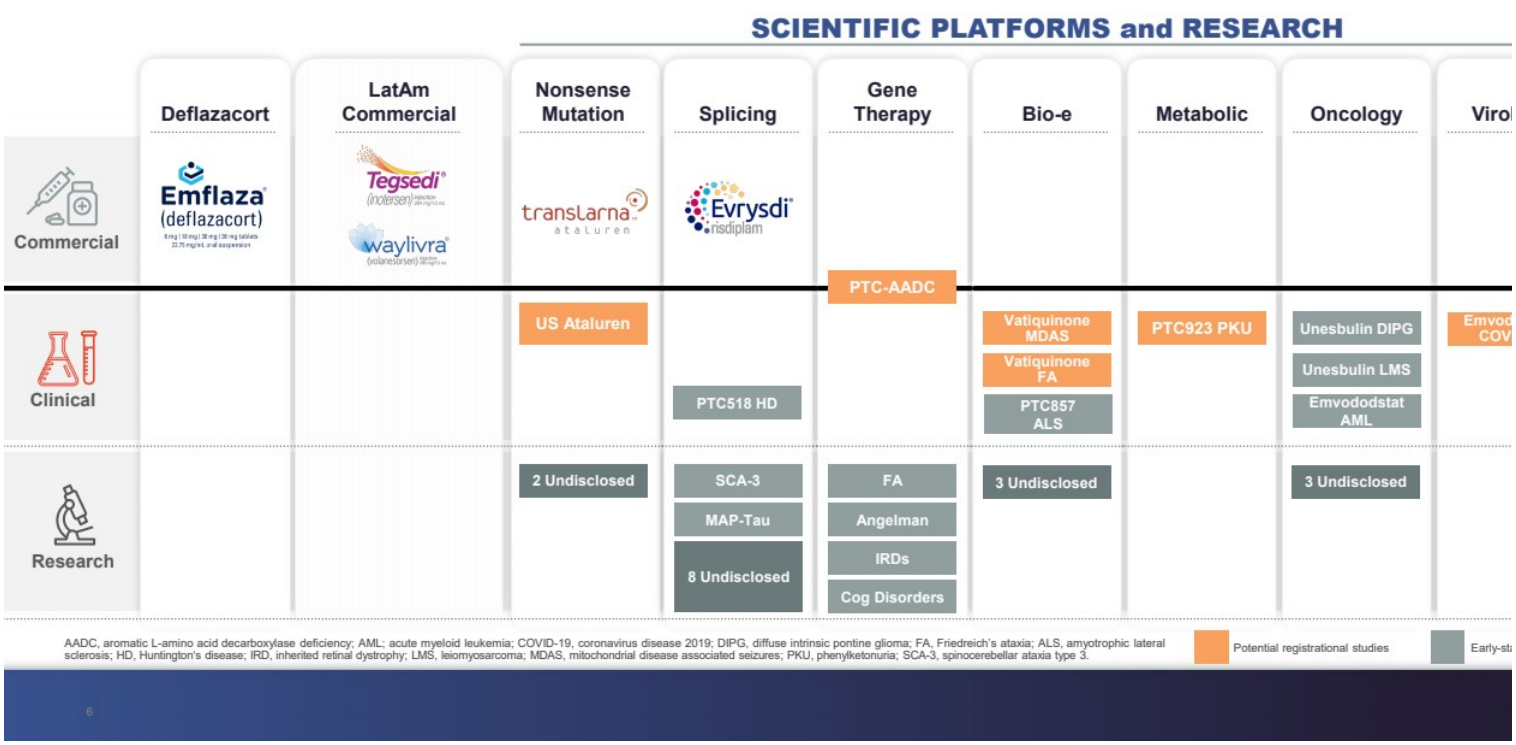
PTC has a Growing Global Footprint



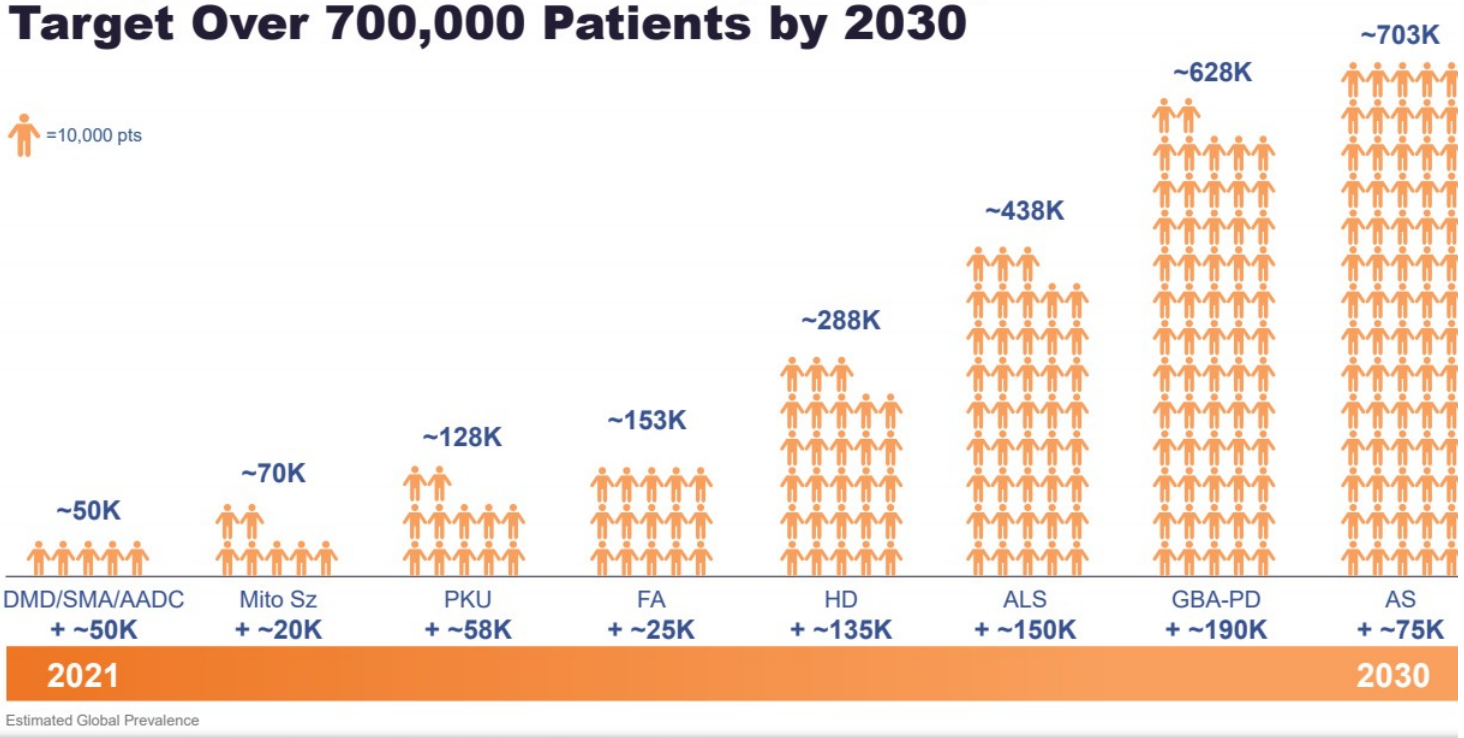
Founded in 1998

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

Diversified Platform Drives Strong Portfolio



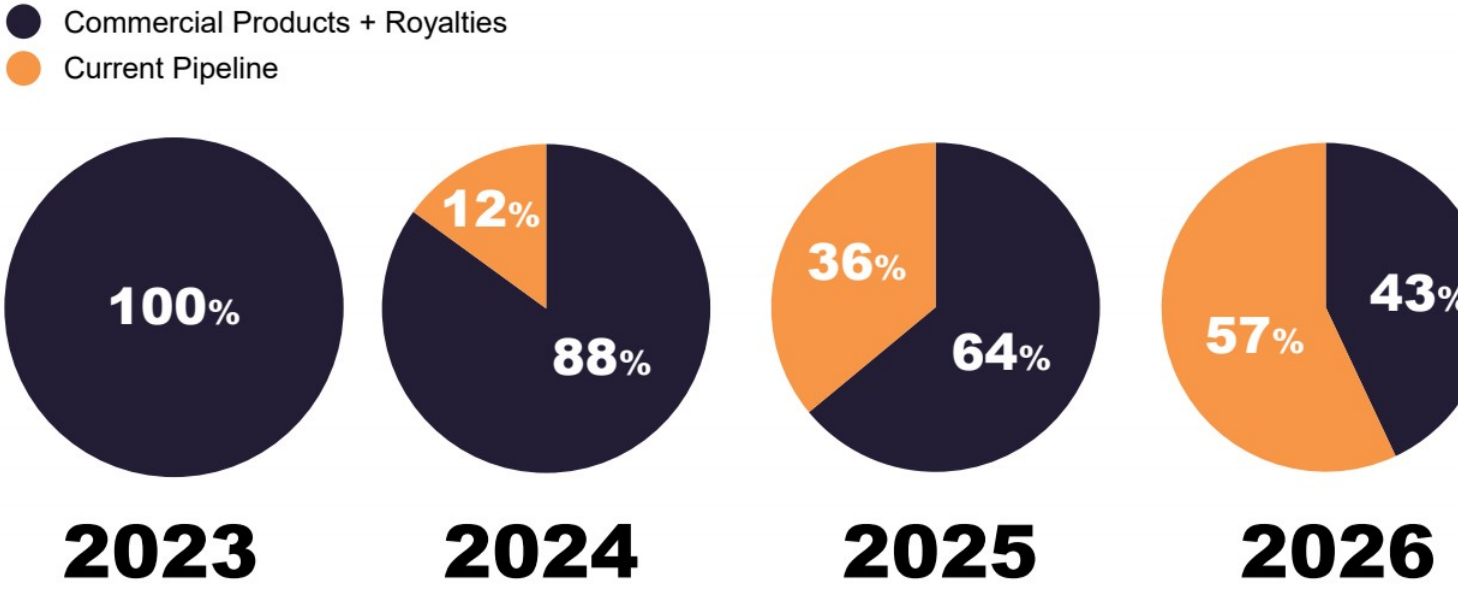
Multiple Platforms Provide Opportunity to Target Over 700,000 Patients by 2030



Estimated Global Prevalence

Enduring Innovation Drives Value Creation

Revenue Contribution of our Pipeline Grows



Potential future revenues

Significant Execution and Value Creation in 2021

Clinical Achievements

- Completed Phase 1 healthy volunteer trial of PTC518
- Completed Phase 1 healthy volunteer trial of PTC857
- Completed enrollment of vatiquinone MOVE-FA registration-directed trial
- Initiation of registration-directed APHENITY Phase 3 trial for PTC923 in PKU
- Completion of unesbulin Phase 1b LMS trial
- Completion of unesbulin Phase 1b DIPG trial

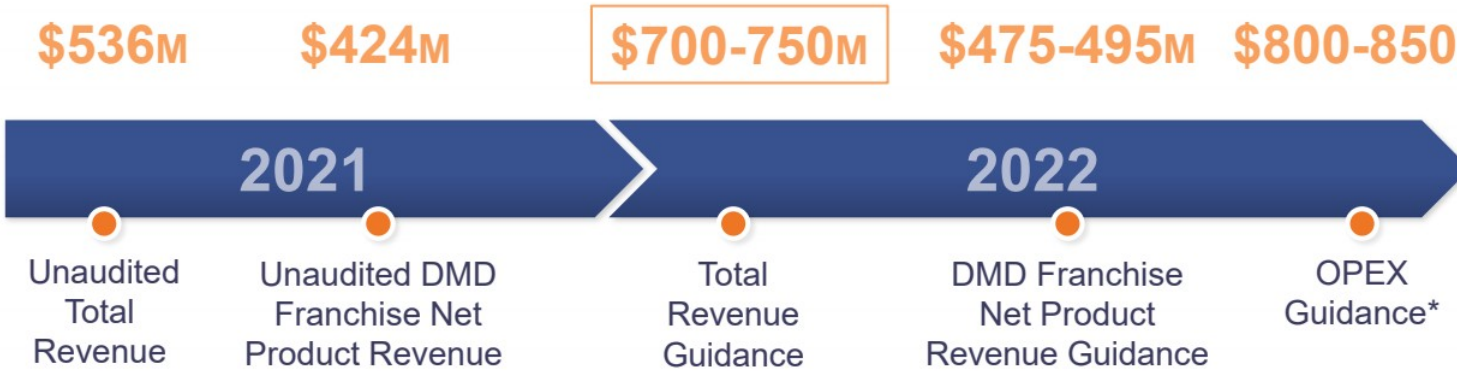
Regulatory Achievements

- Evrysdi now approved in >65 countries including the EU and Japan
- Waylivra approved in Brazil for treatment of FCS
- Translarna label expansion in Brazil to include patients 2 years of age and up
- 2 Rare Pediatric Disorder Designations
- 6 Orphan Drug Designations

Commercial Achievements

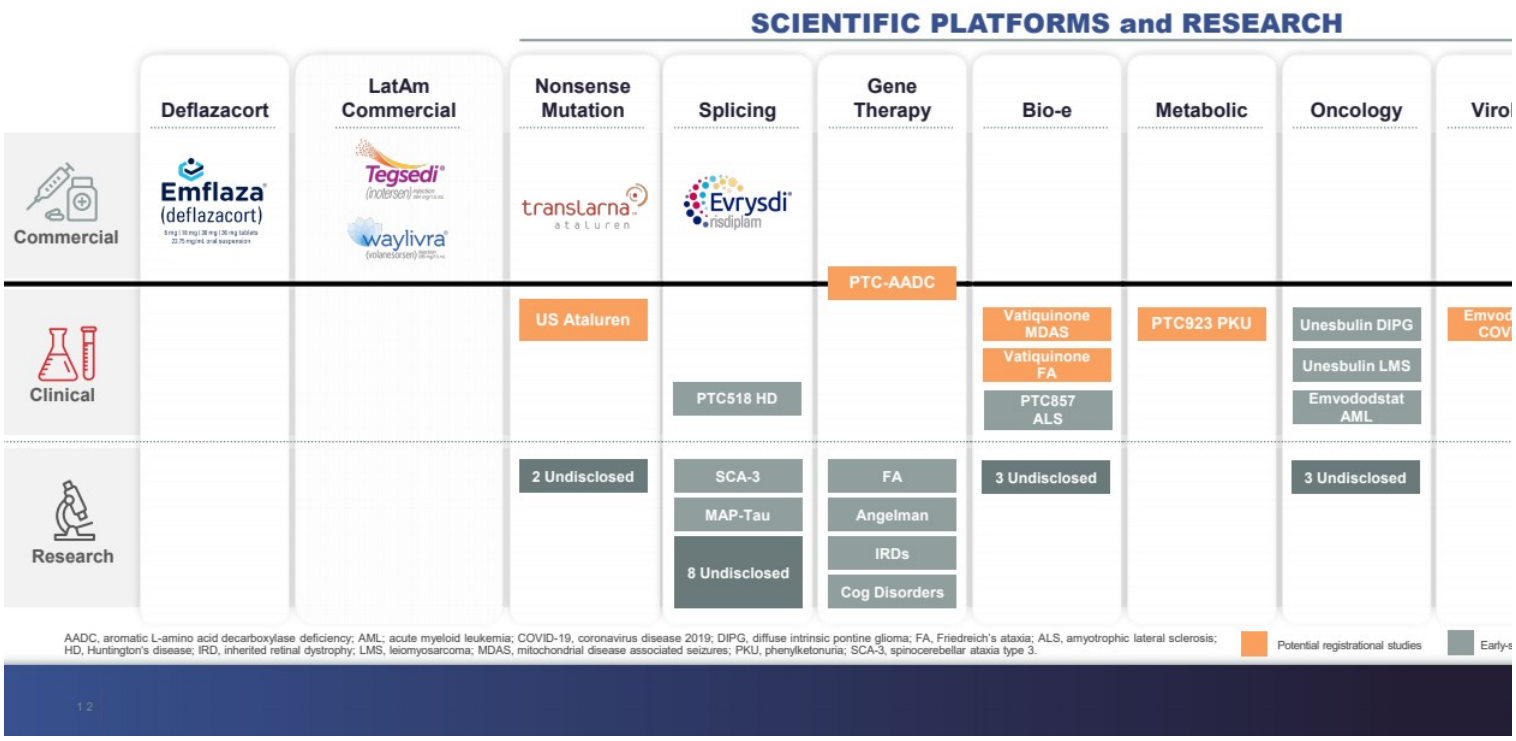
- DMD franchise continues to grow with new patients in existing geographies and geographic expansion for Translarna and new patients and increased compliance for Emflaza
- Evrysdi is most prescribed SMA product and reached ~20% market share in the US
- Tegsedi Category 1 pricing
- Waylivra Category 1 pricing

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



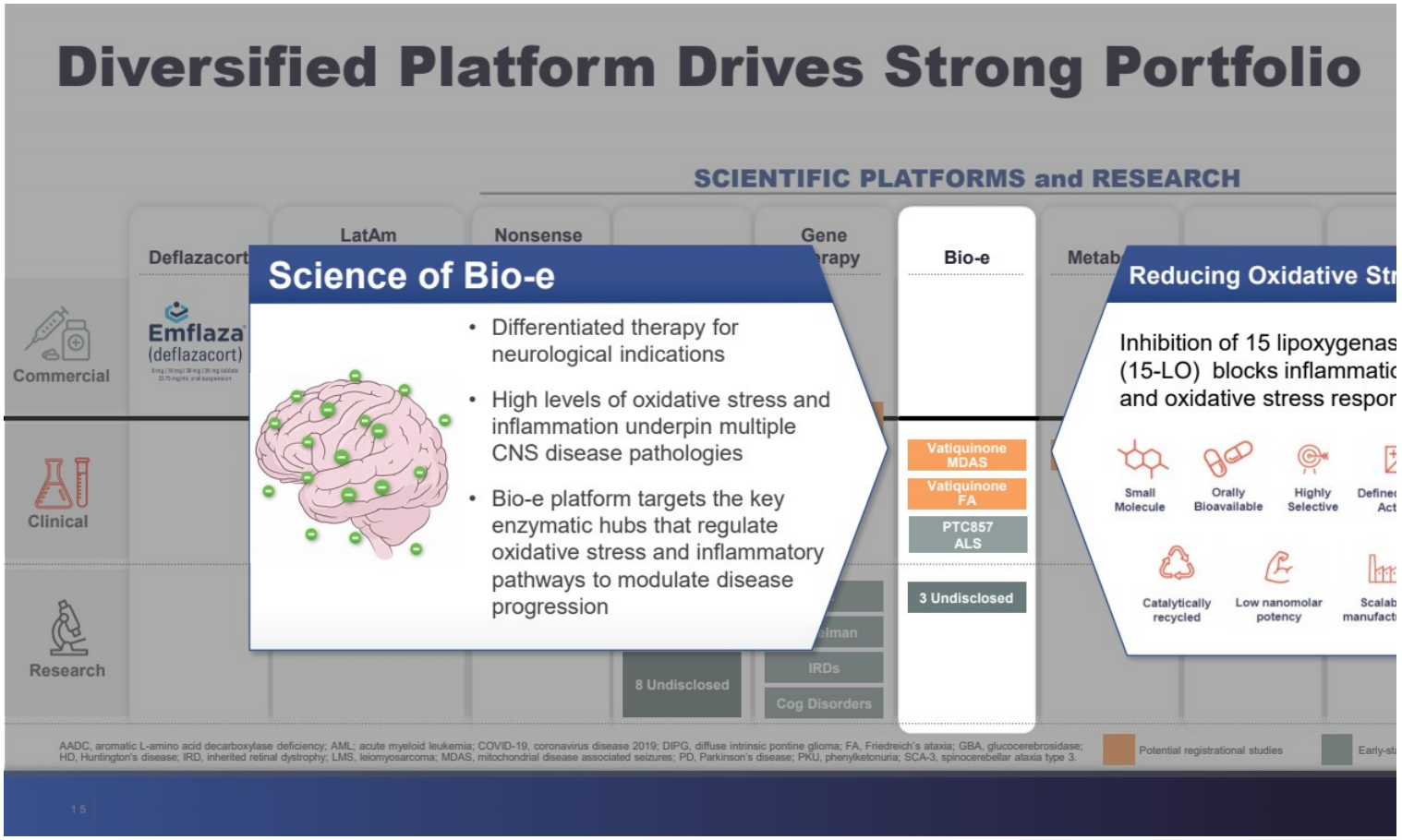
AADC, aromatic L-amino acid decarboxylase deficiency; AML; acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedreich's ataxia; ALS, amyotrophic lateral sclerosis; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma;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

Five Registration-Directed Clinical Trials Drives Near-Term Value

Emvododstat	Translarna (Ataluren)	Vatiquinone	PTC923	Vatiquinone
COVID-19	nmDMD	Mitochondrial Disease Associated Seizures	PKU	Friedreich Ataxia
FITE19	Study 041	MIT-E epilepsy	aphenity	MOVE-FA
Data Expected **1H 2022**	Data Expected **Mid Year 2022**	Data Expected **4Q 2022**	Data Expected **YE 2022**	Data Expected **2Q 2023**

Substantial Pipeline Progress Planned in 202

Diversified Platform Drives Strong Portfolio



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

Vatiquinone has 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

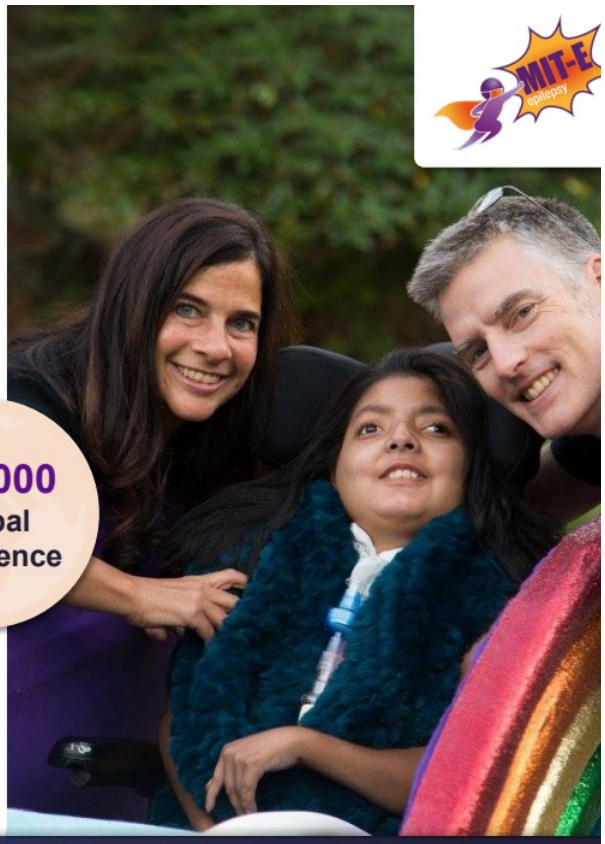
Current Treatments

No approved disease modifying treatments

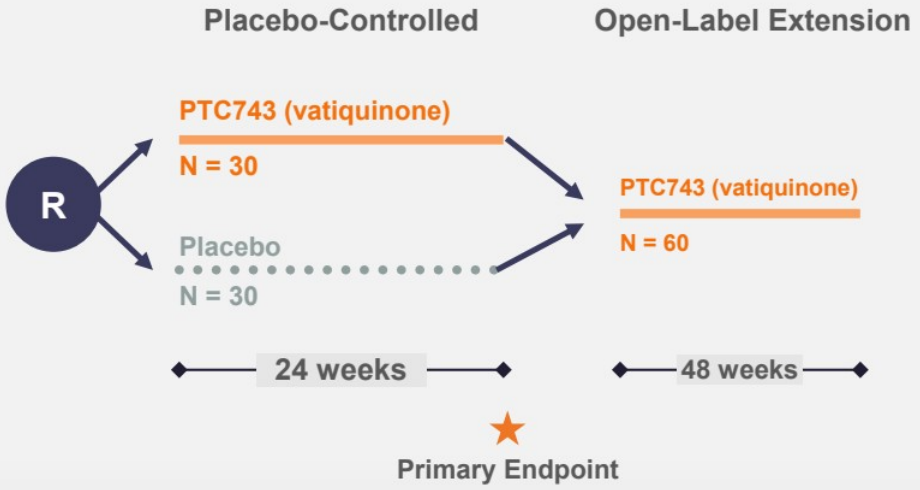
~20,000
Global Prevalence

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 Potential to Show Clinically Differentiated Improvement for MDAS Patients



Primary Endpoint

Change from baseline in frequency of observable motor seizures

Trial Status

- Enrolling
- Data expected 4Q 2022


MOVE-FA
MOVE-FA:
Registration-directed trial
of vatiquinone for
Friedreich Ataxia
19

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

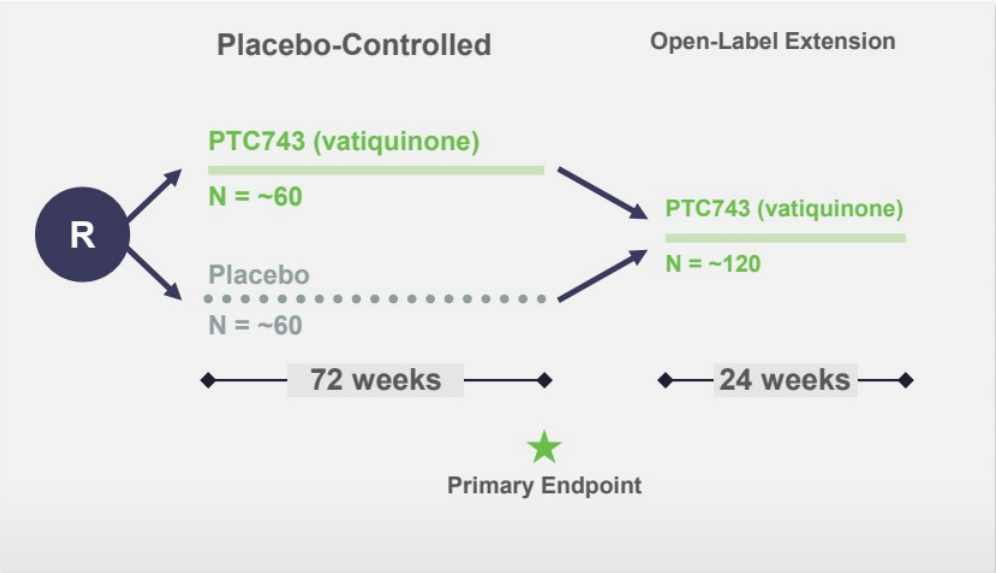
Current Treatments

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



Primary Endpoint

Change in mFARS

Key Secondary Endpoint

Change in FA-ADL

Trial Status

Enrollment complete

- Data expected in 2Q 2023

CardinALS:
Phase 2 trial of PTC857 for Amyotrophic Lateral Sclerosis

PTC857 has 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

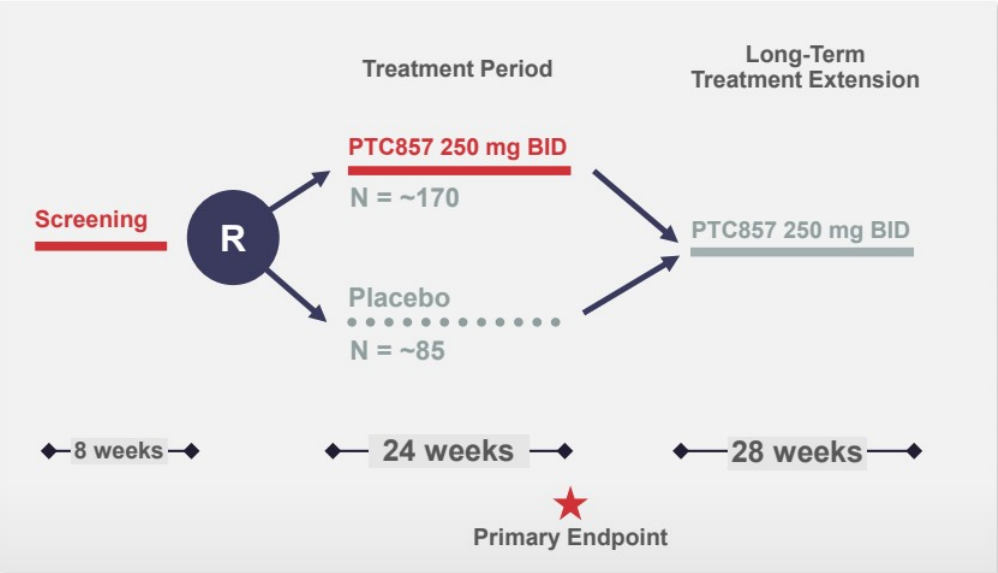
No approved disease modifying therapies

Mechanism of Action

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

CARDINALS

PTC857 has Potential to Slow Disease Progression in ALS



Primary Endpoints

Change in ALSFRS-R

Secondary Endpoints

Safety and PK

Trial Status

- Initiation in 2Q22

Diversified Platform Drives Strong Portfolio

SCIENTIFIC PLATFORMS and RESEARCH

LatAm

Nonsense tation

Splicing

Gene Thera

Comm

Clin

Research

Evrysdi risdiplam

PTC518 HD

SCA-3

MAP-Tau

8 Undisclosed

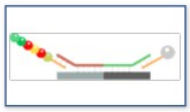
closed

IRDs

Cog Disorders

Leaders in splicing technology

Databases of Splicing Targets



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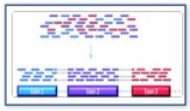
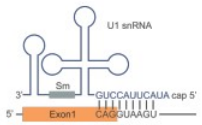


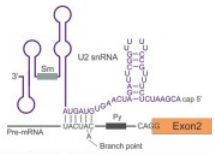
HTSpliceseq

Splicing

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



5' Splice Site



3' Splice Site

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

Potential registrational studies

Early-st


PIVOT
HD
PIVOT HD:
PTC518 for Huntington's Disease
26

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

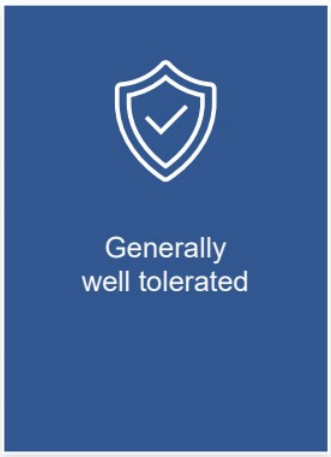
Current Treatments

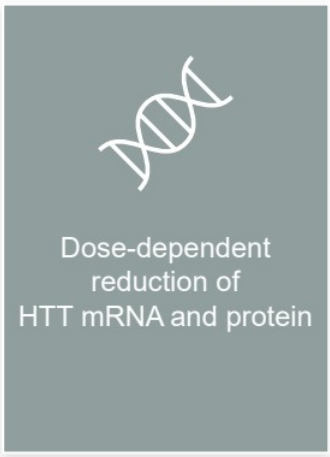
No approved disease modifying therapies

Mechanism of Action

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

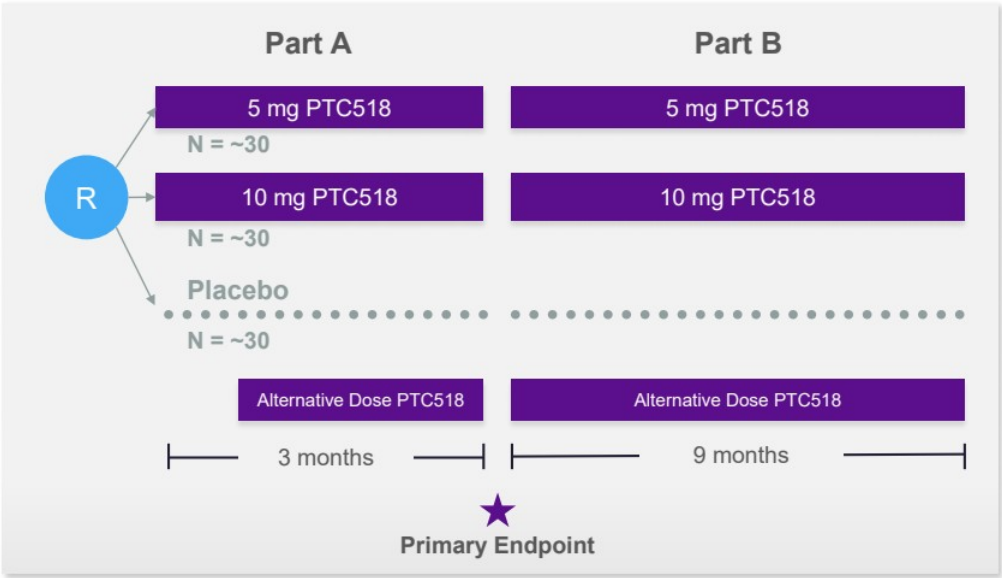
PTC518 Reduces HTT mRNA and Protein to Target the Proximal Cause of HD





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

- Trial to initiate in first quarte

PIVOT HD Trial Will Target the "Goldilocks" Population

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 ligh chain (NfL) in plasma and CS
- Change in caudate, putamena 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

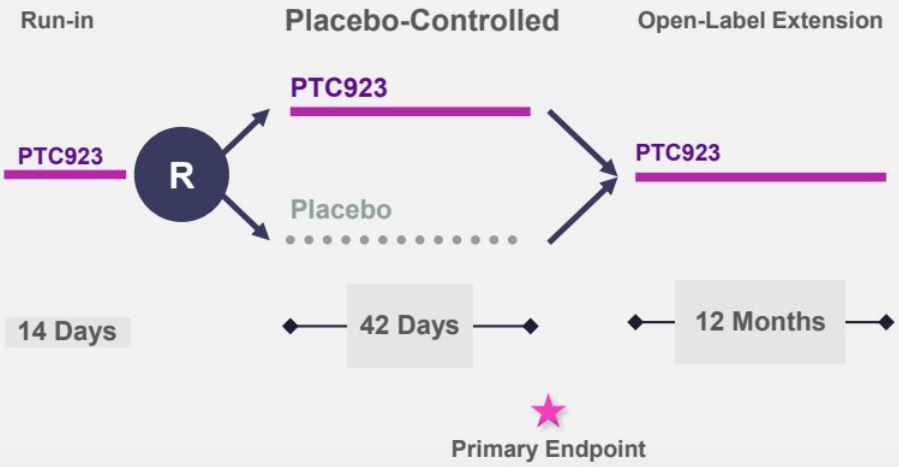
Majority of patients do not initially respond or are not well controlled by standard of care

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



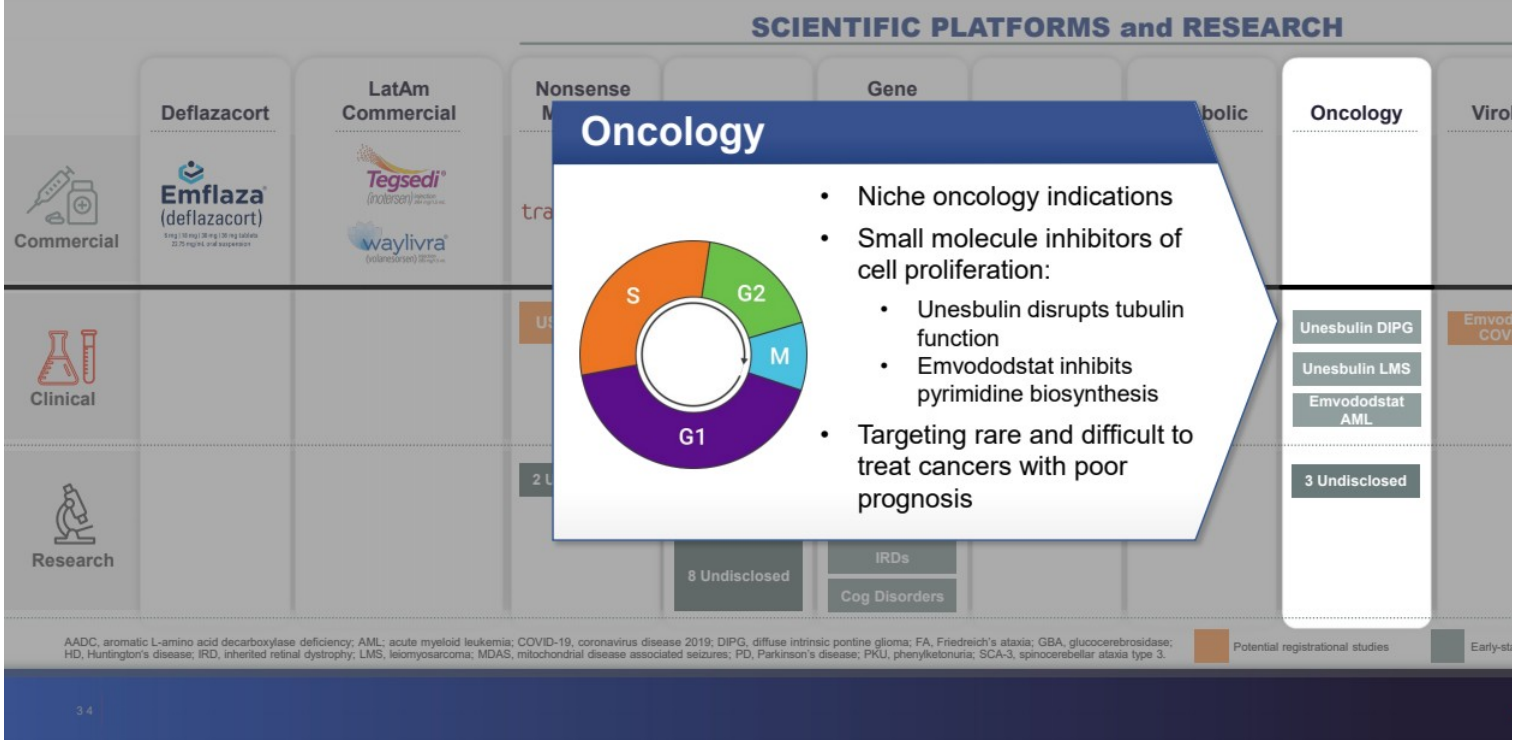
Primary Endpoint

Reduction in blood phenylalanine levels

Trial Status

- Initiated in 3Q21
- Data expected YE 2022

Diversified Platform Drives Strong Portfolio



Unesbulin has 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

Unesbulin has Opportunity to Provide Additional Progression Free Survival in LMS

21 Day Treatment Cycles

Phase 1b Study Design

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

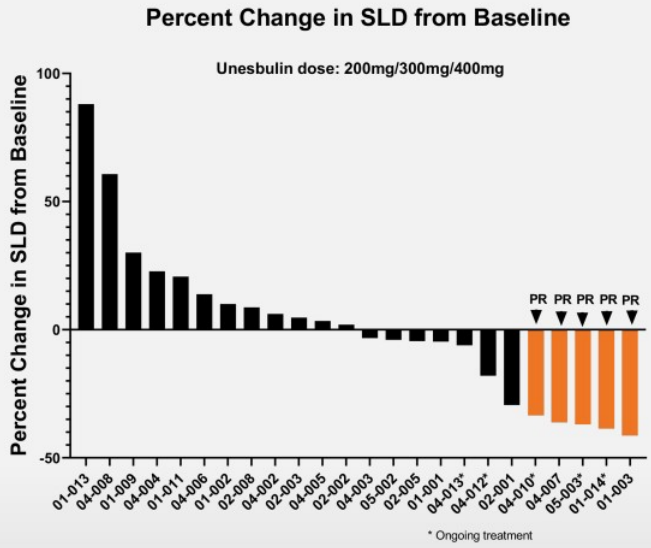
N = 29

Inclusion Criteria

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

Phase 1b study results

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



Unesbulin has Opportunity to Provide Additional Progression Free Survival in LMS

Primary Endpoint
PFS as determined by RECIST

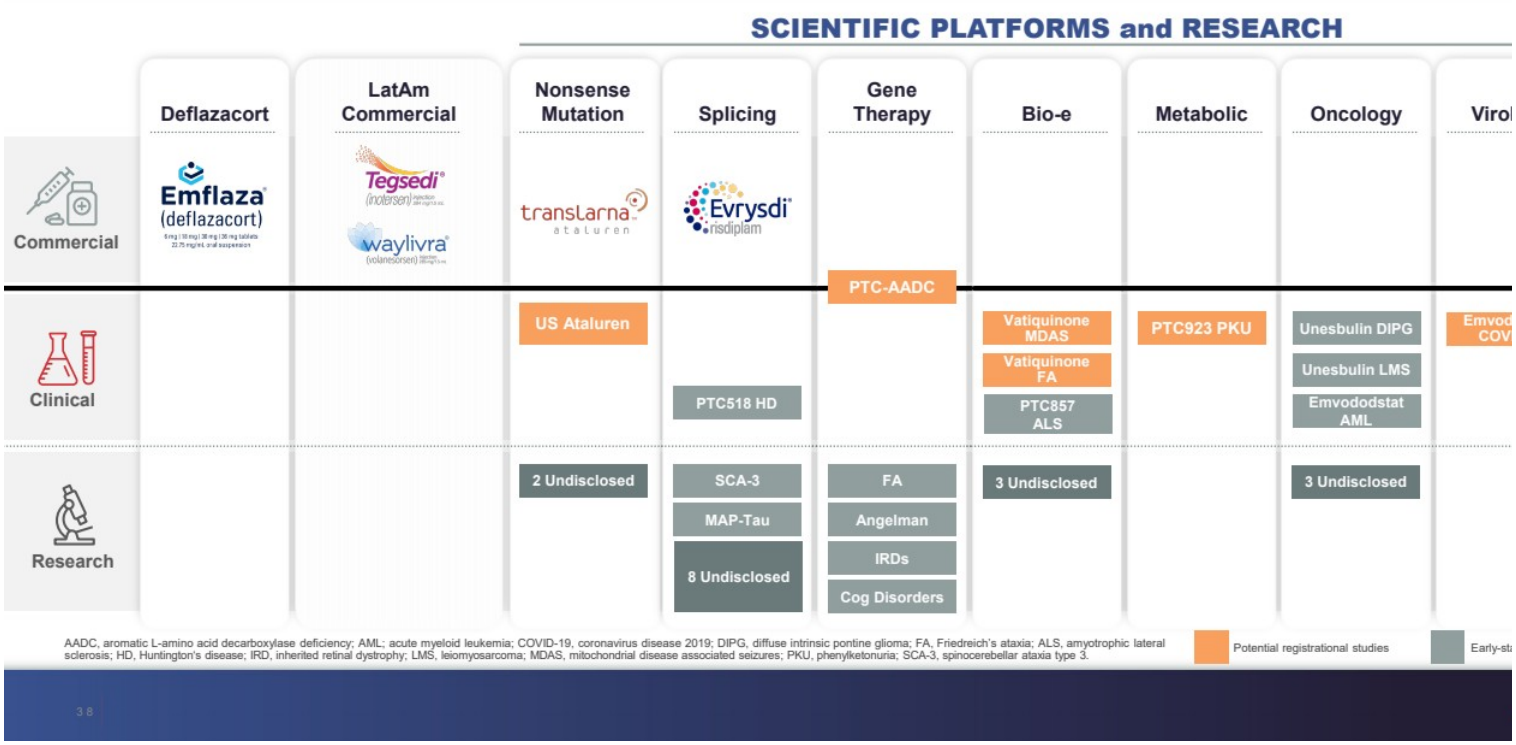
Secondary Endpoints
OS, ORR, DCR, DOR

Interim Analysis

Trial Status

- Initiating in 2Q 2022

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
- Expanding patient base, high compliance, better access, strong geographic expansion

Emflaza® (deflazacort)

6 mg | 18 mg | 30 mg | 36 mg tablets
22.75 mg/mL oral suspension

- First and only corticosteroid approved for DMD; approved for all US DMD patients >2yrs
- Data show clinical benefit over prednisone
- New patients, high adherence and fewer discontinuations

Evrysdi® risdiplam

- Evrysdi now approved in >65 countries
- Continued strong uptake in the U.S. ~20% market share just over one year after launch
- Potential for $325M in sales-based milestones

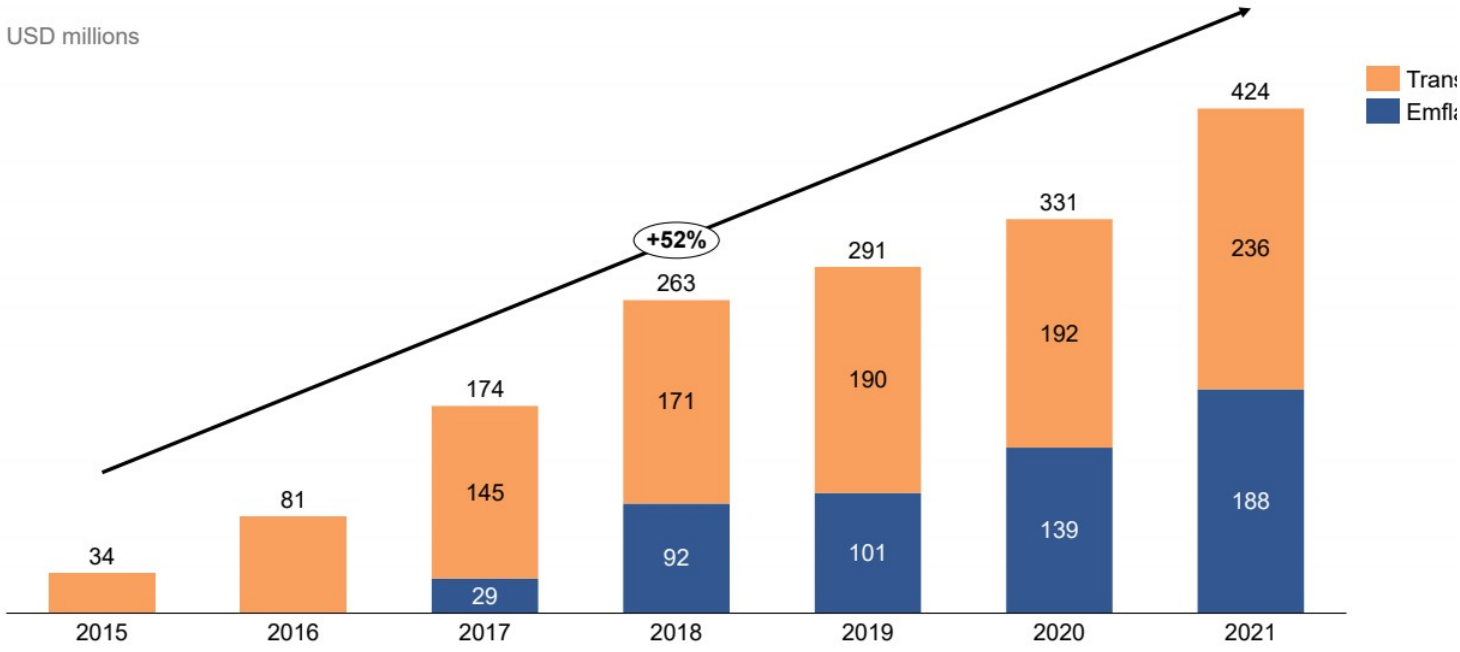
Tegsedi® (inotersen) injection 284 mg/1.5 mL

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

waylivra® (volanesorsen) injection 285 mg/1.5 mL

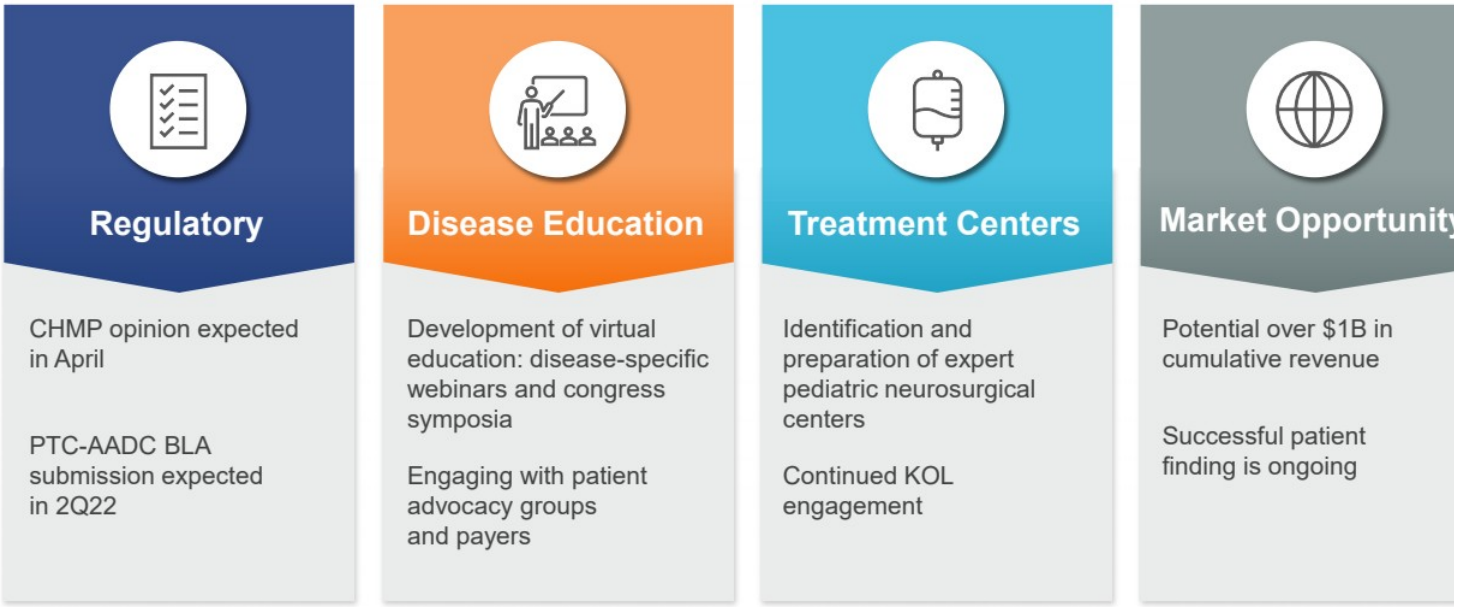
- For treatment of familial chylomicronaemia syndrome (FCS)
- LATAM patients benefiting through early-access programs
- Received Category pricing, in final pricing negotiations

Continued Strong DMD Franchise Growth



2021 unaudited net product revenue

PTC-AADC Gene Therapy Opportunity and Launch Preparation



Enduring Innovation Drives Value Creation

Clinical

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