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 9, 2023**

PTC THERAPEUTICS, INC.

(Exact Name of Company as Specified in Charter)

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

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

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

Not applicable

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

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

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

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

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

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

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

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

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

Emerging growth company ☐

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

Item 2.02 Results of Operations and Financial Condition.

On January 9, 2023, 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, 2022, including that the Company expects to report (i) total unaudited net revenue of approximately $710 million, (ii) total unaudited net product revenue of approximately $535 million, (iii) net product revenue for Translarna™ (ataluren) of approximately $289 million and net product revenue for Emflaza® (deflazacort) of approximately $218 million and (iv) collaboration and royalty revenue associated with Evrysdi of approximately $175 million. Final results are subject to completion of the Company's year-end audit.

Item 7.01. Regulation FD Disclosure.

On January 9, 2023, the Company also announced financial guidance for its fiscal year ending December 31, 2023 in the press release, including that the Company anticipates (i) full year total revenues to be between $940 million and $1.0 billion (ii) full year net product revenues for the Duchenne muscular dystrophy franchise to be between $545 and $565 million (iii) GAAP R&D and SG&A expense for the full year 2023 to be between $1.01 and $1.06 billion with non-GAAP R&D and SG&A expense for the full year 2023 to be between $890 and $940 million, excluding estimated non-cash, stock-based compensation expense of approximately $120 million and (iv) up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisition.

The Company announced that on Monday, January 9, 2023 at 10:30 am EST at the 41st Annual J.P. Morgan Healthcare Conference, the Company will provide an update on 2022 accomplishments and highlight upcoming 2023 potential value-creating milestones. The Company will also present its preliminary 2022 financial results and 2023 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 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 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 Current Report on Form 8-K.

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 2022 and financial guidance for fiscal year 2023. 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 2022 financial information, which is subject to completion of the Company's year-end audit; the assumptions underlying the Company's financial guidance for 2023; 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, 2021 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 9, 2023 issued by PTC Therapeutics, Inc.
99.2	Corporate Presentation – 41st 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 9, 2023

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

PTC Therapeutics Provides an Update on Commercial Progress and R&D Pipeline at 41st Annual J.P. Morgan Healthcare Conference

- ~$710 million unaudited 2022 total revenue, representing an impressive over 30% year-over-year growth –

- $940 million - $1.0 billion 2023 total revenue guidance –

- Results from three registration-directed clinical trials expected in 2023 –

- One additional registration-directed clinical trial expected to initiate in 2023 –

SOUTH PLAINFIELD, N.J., Jan. 9, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will present an update on its commercial progress and R&D pipeline at the 41st Annual J.P. Morgan Healthcare Conference today, Monday January 9, at 10:30am EST/7:30am PST. Matthew Klein, M.D., Chief Operating Officer of PTC Therapeutics, will provide an update on 2022 accomplishments and highlight upcoming 2023 potential value-creating milestones. Preliminary 2022 unaudited financial results and 2023 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 2022 Corporate Highlights:

- Upstaza™, the first direct-administered gene therapy into the brain, was approved in the EU and UK for AADC deficiency patients.
- Unaudited net product revenue of $535 million in 2022 representing 25% year-over-year growth.
  - Strong year-over-year growth for the Duchenne muscular dystrophy (DMD) franchise, with unaudited net product revenue of $289 million for Translarna™ (ataluren) and $218 million for Emflaza® (deflazacort) in 2022.
    - Translarna growth was driven by new patients in existing geographies and continued geographic expansion.
    - Emflaza growth was due to continued new prescriptions, high compliance, fewer patient discontinuations and more favorable access.
  - Upstaza™ unaudited net product revenue was $13 million driven by patients being treated through early access programs and commercial access.
- Evrysdi® (risdiplam) is now approved in more than 90 countries. It has established market leadership in all major markets and is on track to become the global market leader in treatment of spinal muscular atrophy (SMA). Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation and Roche.
- PTC successfully advanced its clinical pipeline in 2022:
  - CardinALS, a registration-directed Phase 2 clinical trial of PTC857 in amyotrophic lateral sclerosis, was initiated.
  - PIVOT-HD, a Phase 2 clinical trial of PTC518 in Huntington's disease, was initiated.
  - SunriseLMS, a registration-directed Phase 2 trial of unesbulin in leiomyosarcoma, was initiated.
  - The placebo-controlled portion of Study 041, a Phase 3 clinical trial of Translarna for nmDMD, was completed. Study 041 results supported submission of a Type II variation for conversion to standard marketing authorization in the EU.
  - FITE-19, a Phase 2/3 clinical trial of emvododstat for COVID-19, was completed.

2023 Potential Key Value-Creating Milestones:

- Results from the placebo-controlled portion of APHENITY, the Phase 3 registration-directed clinical trial of sepiapterin in patients with PKU, are expected in the first quarter of 2023.
- Results from MIT-E, the Phase 2/3 registration-directed clinical trial of vatiquinone in mitochondrial disease associated seizures, are expected in the first quarter of 2023.
- Results from MOVE-FA, the Phase 3 registration-directed clinical trial of vatiquinone in Friedreich ataxia, are expected in the second quarter of 2023.
- Results from the 12-week portion of PIVOT-HD, the Phase 2 study of PTC518 in Huntington's disease, are expected in the second quarter of 2023.
- Submission of a Biologics License Application (BLA) to the FDA for Upstaza is expected in the first half of 2023.
- A Phase 2/3 clinical trial of unesbulin in diffuse intrinsic pontine glioblastoma is expected to initiate in the fourth quarter of 2023.

Preliminary Unaudited 2022 Financial Results:

- Total unaudited net revenue for full year 2022 was approximately $710 million.
- Total unaudited net product revenue for full year 2022 was approximately $535 million.
- DMD franchise unaudited revenue for full year 2022 was approximately $507 million, including net product revenue for Translarna of approximately $289 million and for Emflaza of approximately $218 million.
- PTC expects to report approximately $175 million in 2022 collaboration and royalty revenue associated with Evrysdi.

PTC is currently in the process of finalizing its financial results for the 2022 fiscal year. The above information is based on preliminary unaudited information and management estimates for the full year 2022, subject to the completion of PTC's financial closing procedures. Evrysdi royalty revenue estimates are based on sell side analyst consensus estimates.

2023 Financial Guidance:

- PTC anticipates total revenues for the full year 2023 to be between $940 million and $1.0 billion.
- PTC anticipates net product revenues for the DMD franchise for the full year 2023 to be between $545 and $565 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2023 to be between $1.01 and $1.06 billion.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2023 to be between $890 and $940 million, excluding estimated non-cash, stock-based compensation expense of $120 million. PTC anticipates up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions.

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 2023 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 1,010,000	$ 1,060,000
Less: projected non-cash, stock-based compensation expense	120,000	120,000
Projected non-GAAP R&D and SG&A expense	$ 890,000	$ 940,000

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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kokeefe@ptcbio.com

Media
Jeanine Clemente
+1 (908) 912-9406
jclemente@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

"2023 Financial Guidance", including with respect to (i) 2023 total revenue guidance, (ii) 2023 net product revenue guidance for the DMD franchise,(iii) 2023 GAAP and non-GAAP R&D and SG&A expense guidance and (iv) 2023 acquisition related one-time 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions, commercialization and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to utilize results from Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, to support a marketing approval for Translarna for the treatment of nmDMD in the United States and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the results of PTC's clinical trial for emvododstat for COVID-19; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of its lease agreements, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

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

The forward-looking statements contained herein represent PTC's views only as of the date of this 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.

PTC 2023

Matthew Klein, M.D., COO
JP Morgan Healthcare Conference

January 9, 2023

PKU Patient

PTC THERAPEUTICS

Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this presentation, other than statements of historic fact, are forward-looking statements, including statements with respect preliminary unaudited 2022 financial information with respect to 2022 total net revenue and 2022 DMD franchise net product revenue, statements with respect to guidance relating to 2023 total net product revenue, 2023 DMD franchise net product revenue and 2023 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions, commercialization and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to complete Study 041, which is a specific obligation to continued marketing authorization in the EEA; PTC's ability to utilize results from Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, to support a marketing approval for Translarna for the treatment of nmDMD in the United States and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the results of PTC's clinical trial for emvododstat for COVID-19; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of its lease agreements, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; 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.

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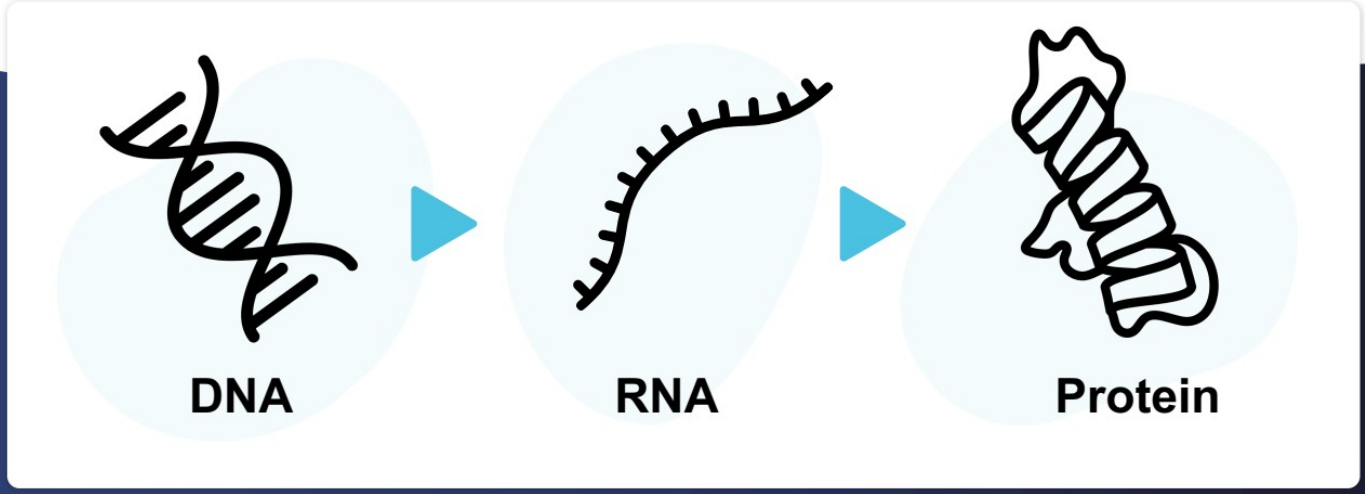
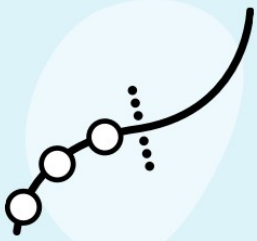
The forward-looking statements contained herein represent PTC's views only as of the date of this presentation and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this presentation except as required by law.

Treating Rare Diseases by Modulating Gene and Protein Expression



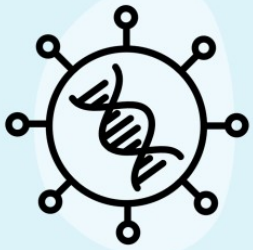
Treating Rare Diseases by Modulating Gene and Protein Expression



Nonsense Suppression

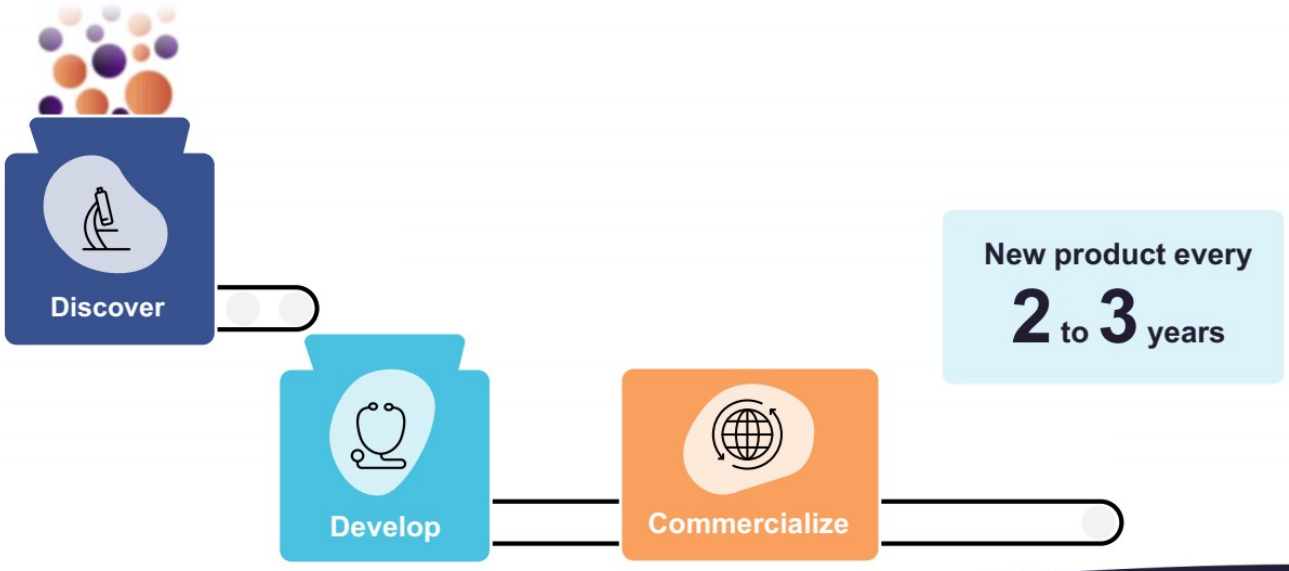
Splicing

Bio-e

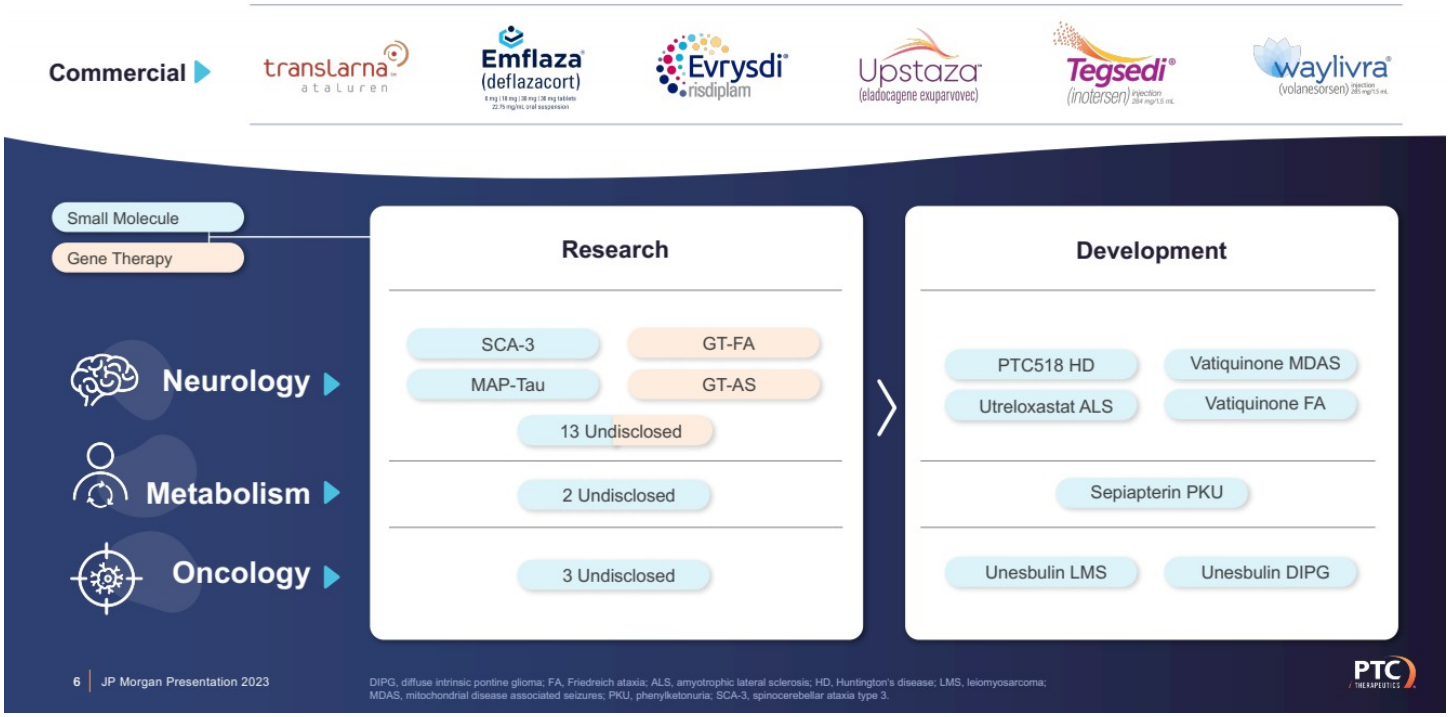


Gene Therapy

Building a Pipeline to Produce a Therapy Every Two to Three Years

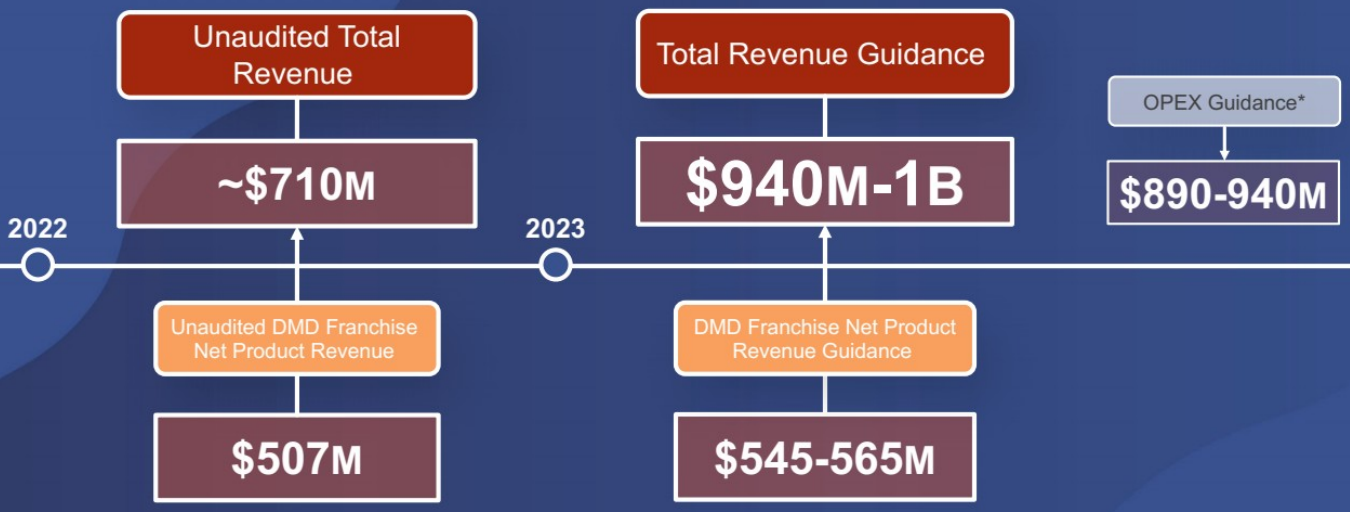


A Diverse and Robust Portfolio To Continually Create Value



DIPG, diffuse intrinsic pontine glioma; FA, Friedreich ataxia; ALS, amyotrophic lateral sclerosis; HD, Huntington's disease; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

Continuing Strong Financial Performance Expected in 2023



*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately $120 million. GAAP R&D and SG&A expense for the full year 2023 is anticipated to be between $1,010 and $1,060 million. The Company anticipates up to $80 million of one-time payments upon achievement of potential clinical and regulatory success-based milestones from previous acquisitions.

Continued Success Across Our Commercial Portfolio

translarna™ ataluren

Distributed in 50+ countries with continued growth from new patients and geographic expansion

Emflaza® (deflazacort)

First and only corticosteroid for all US DMD patients with growth from new patient starts and favorable access

Evrysdi® risdiplam

Established market leadership in all major markets with continued growth expected

Upstaza™ (eladocagene exuparvovec)

First EMA approved disease-modifying treatment for AADC deficiency for patients 18 months and older

Tegsedi® (inotersen) injection

For treatment of hATTR with LATAM patients benefiting through early-access programs

waylivra® (volanesorsen) injection

For treatment of FCS and FPL with LATAM patients benefiting through early-access programs

Building Foundation for Upstaza Success

Upstaza (eladocagene exuparvovec)

2022

Early Access

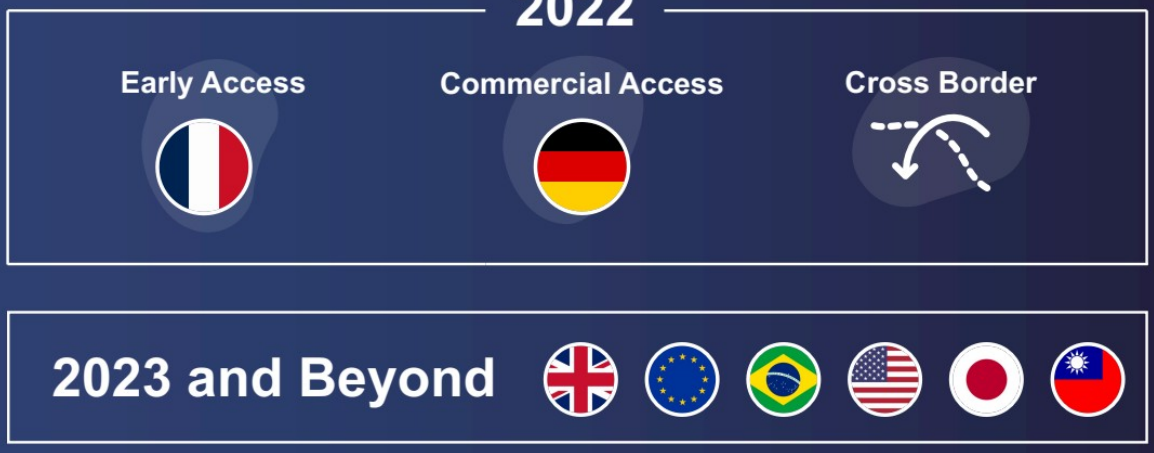
Commercial Access

Cross Border

PTC THERAPEUTICS

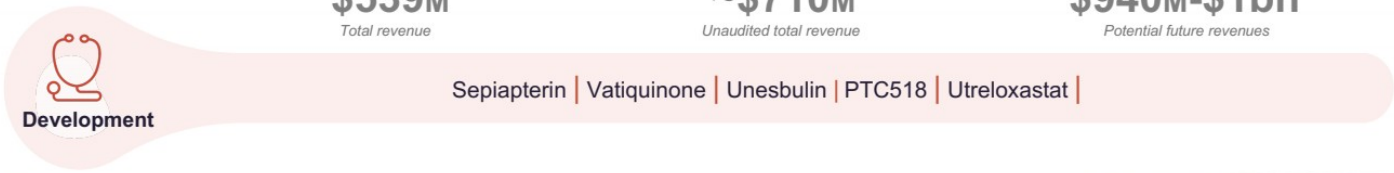
Substantial Growth in 2023 and Beyond with Additional Global Registrations

Upstaza (eladocagene exuparvovec)



PTC THERAPEUTICS

Substantial Revenue Growth from 2021 to 2023



Strong R&D Execution and Value Creation in 2022

Clinical Achievements

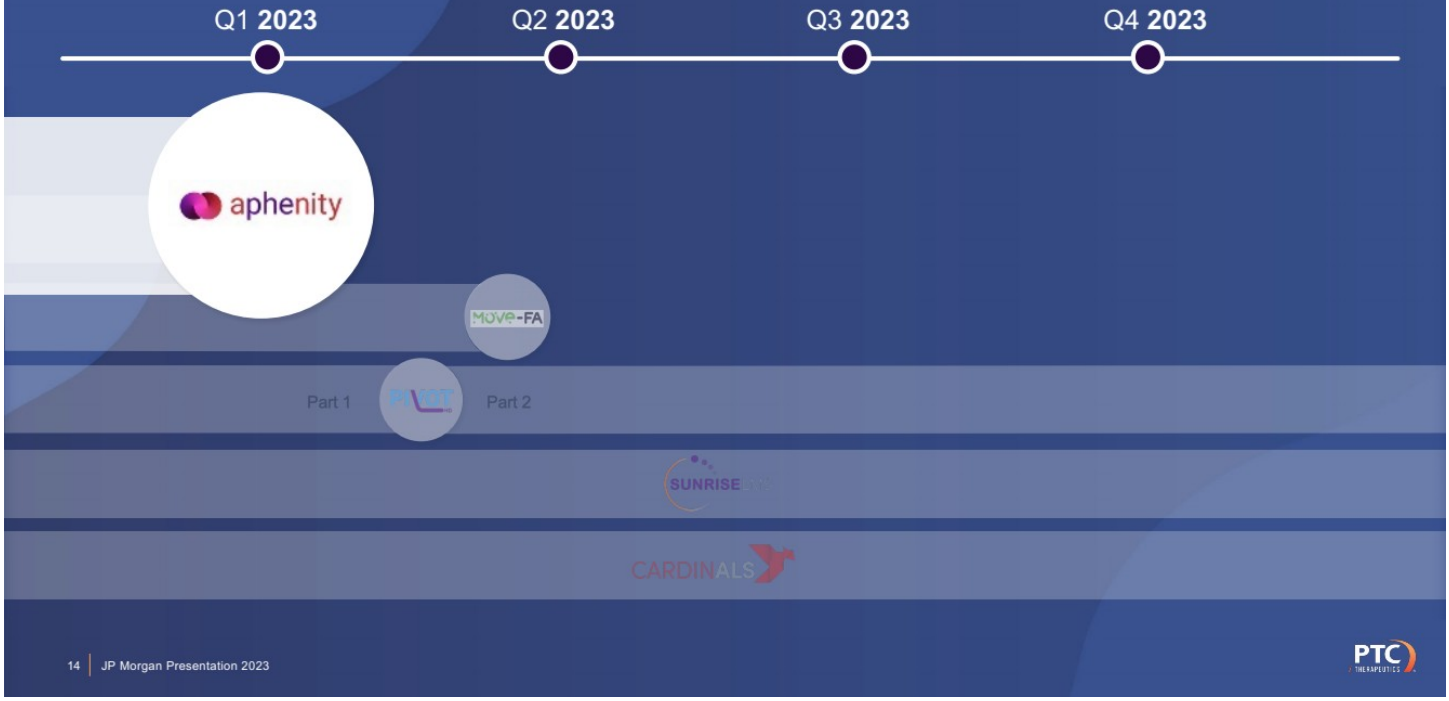
- Initiated CardinALS study for utreloxastat
- Initiated PIVOT-HD study for PTC518
- Initiated SunriseLMS trial for unesbulin
- Completed Study 041 for Translarna
- Completed FITE-19 study for emvododstat
- Completed enrollment for MIT-E

Regulatory Achievements

- Upstaza EU and UK approval
- Waylivra approved in Brazil for treatment of FPL
- Filed type II variation for MAA for Translarna in EU and additional global approvals

Substantial Pipeline Progress Planned in 2023

Substantial Pipeline Progress Planned in 2023



Sepiapterin Can Potentially Treat Broad PKU Population Including Classical PKU

aphenity

Disease

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

Mechanism of Action

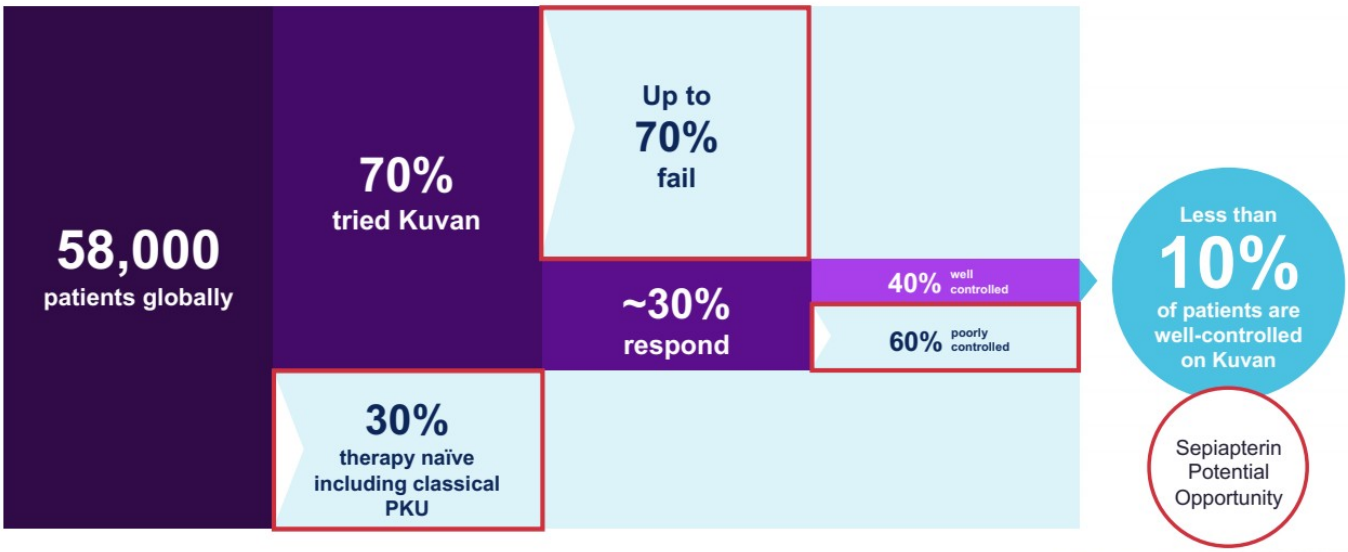
Sepiapterin is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat a broader range of PKU patients

Current Treatments

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

Large Unmet Need Remains in PKU

aphenity



1. BioMarin first quarter 2021 presentation 1Q 2021. 2. Third party reports prepared for PTC

PTC THERAPEUTICS

Established Pillars for Commercial Success

aphenity

- Newborn screening with ~58,000 patients worldwide[1,2,3]
- Well-known metabolic centers of excellence across the world
- Disease pathology well understood and documented
- Connected and coordinated patient advocacy community

1. NPKUA; https://www.npkua.org/, 2. Shoraka et. Al, Clin Exp Pediatr. 2020; 63(2): 34–43., 3. Borrajo G., Acta Ped Mex. 2012; 33(6): 279-287.

PTC THERAPEUTICS

Mechanistic Advantages of Sepiapterin Over Sapropterin: Increased Bioavailability

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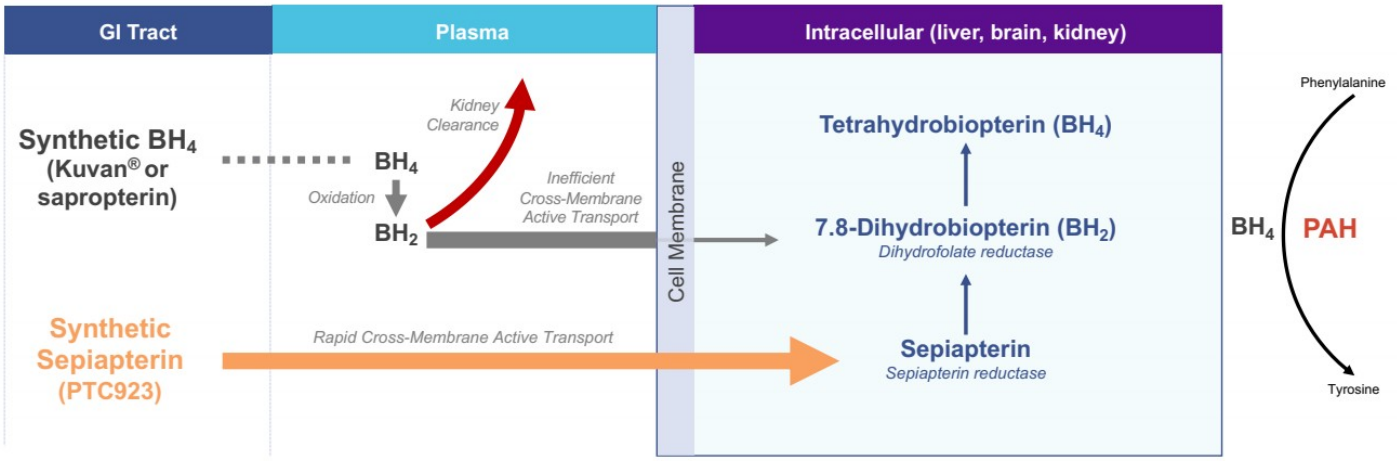

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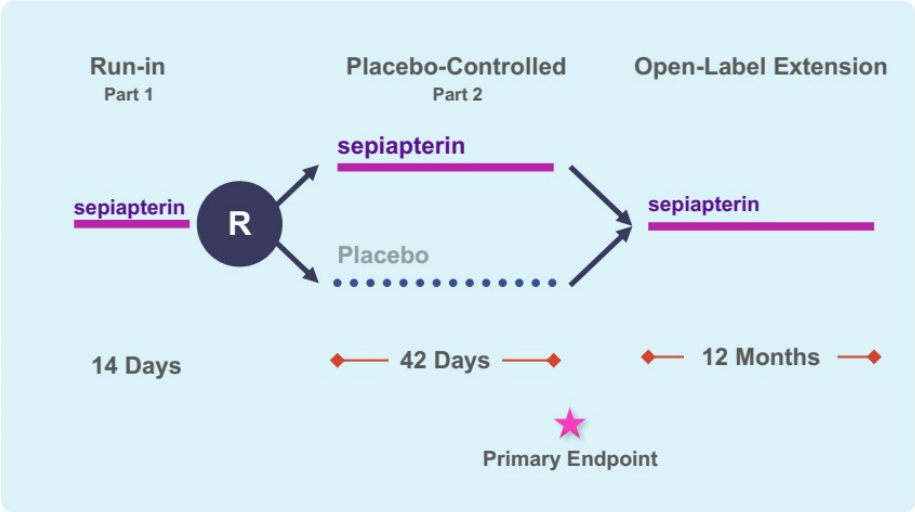
APHENITY is a Global Registration-Directed Trial of Sepiapterin for PKU

aphenity



Primary Endpoint

Reduction in blood phenylalanine levels

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APHENITY Part 1 Preliminary Data (n=104)

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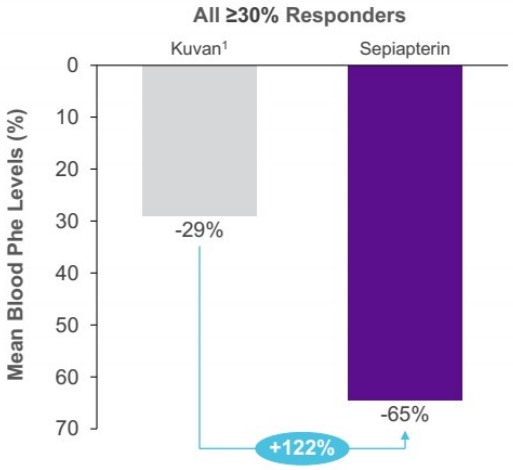
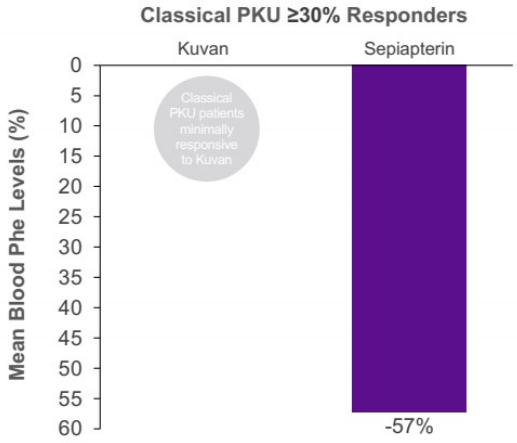
Part 1 is an open-label run-in phase to identify subjects to be randomized
The primary analysis population is those who have a ≥30% Phe reduction





1. KUVAN label (sapropterin dihydrochloride) - Accessdata.fda.gov

PTC THERAPEUTICS

Substantial Pipeline Progress Planned in 2023

Q1 **2023** | Q2 **2023** | Q3 **2023** | Q4 **2023**

MOVE-FA

Part 1 PIVOT Part 2

SUNRISE

CARDINALS

PTC THERAPEUTICS

Vatiquinone Has the Potential to Show Clinically Differentiated Improvement for MDAS Patients

Disease

Mitochondrial disease associated seizures (MDAS) is the highly morbid condition of refractory seizures in patients with inherited mitochondrial disease

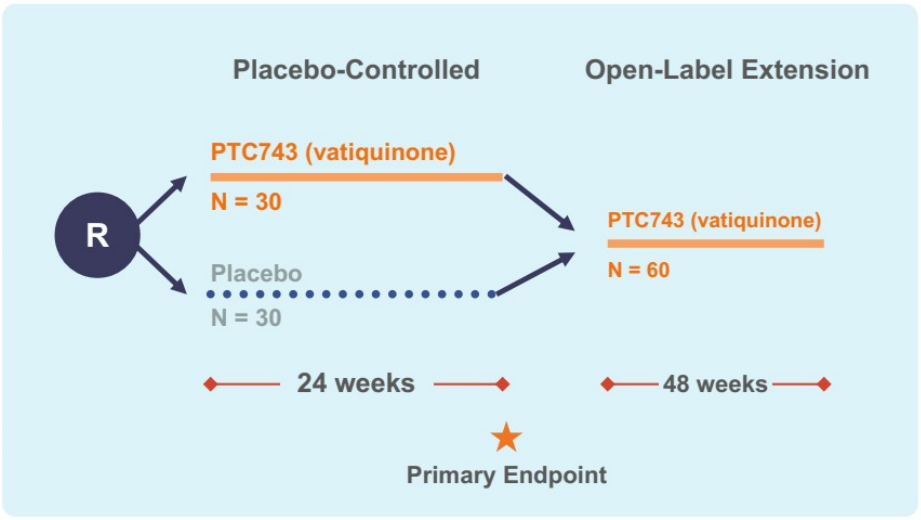
Mechanism of Action

Vatiquinone targets 15-lipoxygenase, a regulator of the key energetic and oxidative stress pathways that underpin seizures in these patients

Current Treatments

No approved disease modifying treatments

MIT-E is a Global Registration-Directed Trial of Vatiquinone for MDAS



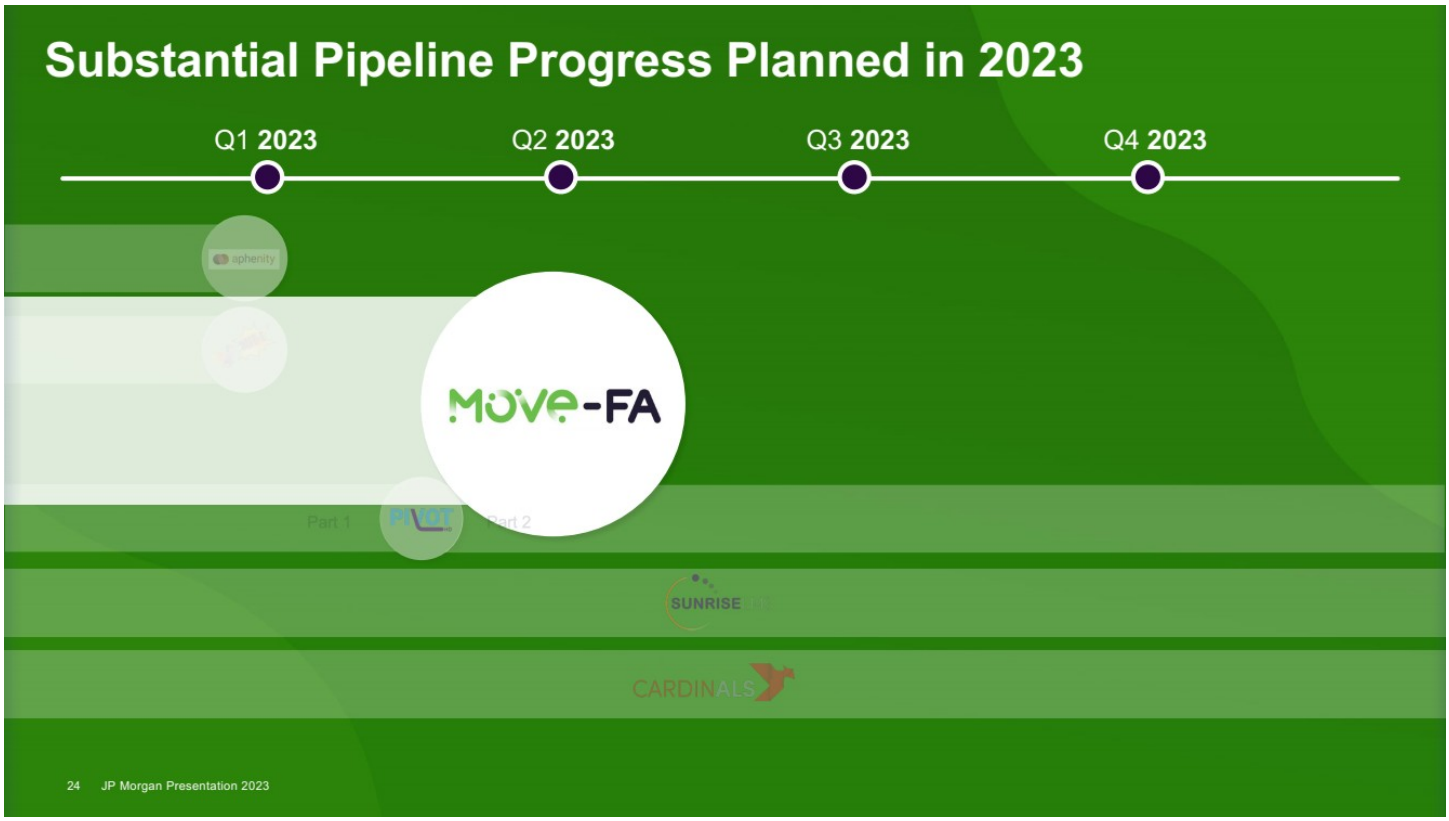
Primary Endpoint

Change from baseline in frequency of observable motor seizures

Trial Status

- Enrollment completed
- Data expected 1Q 2023

Substantial Pipeline Progress Planned in 2023



Vatiquinone Has the Potential to Provide Improvement in Neurological Function

MOVE-FA

Disease

Friedreich ataxia (FA) is a rare, inherited, progressive disease resulting from mitochondrial dysfunction

Mechanism of Action

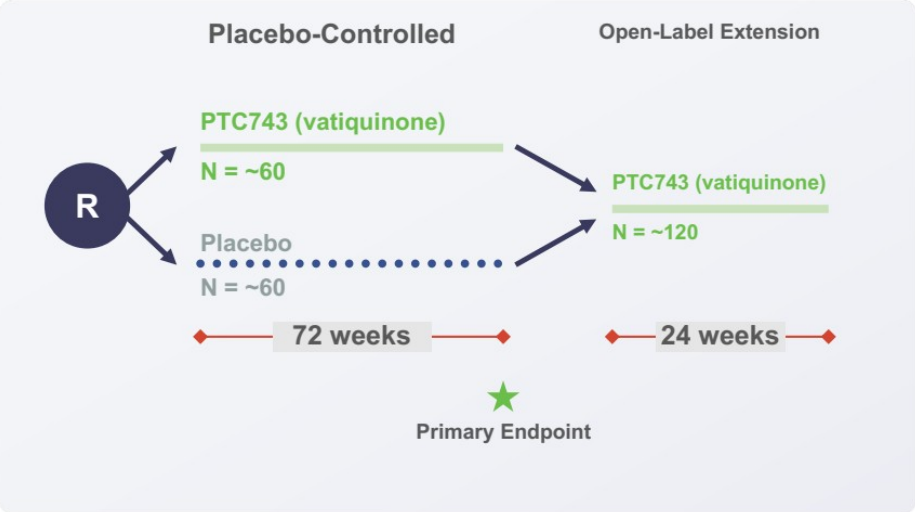
Vatiquinone targets 15-lipoxygenase, a regulator of key energetic and oxidative stress pathways that are disrupted in FA

Current Treatments

No approved disease modifying treatments

~25,000 Global Prevalence

MOVE-FA is a Global Registration-Directed Trial of Vatiquinone for FA



Primary Endpoint
Change in mFARS

Key Secondary Endpoint
Change in FA-ADL

Trial Status

- Enrollment completed
- Data expected in 2Q 2023


Substantial Pipeline Progress Planned in 2023
Q1 2023
Q2 2023
Q3 2023
Q4 2023
aphenity
Part 1
PIVOT HD
Part 2
SUNRISE EMS
CARDINALS
27 JP Morgan Presentation 2023

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

Disease

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

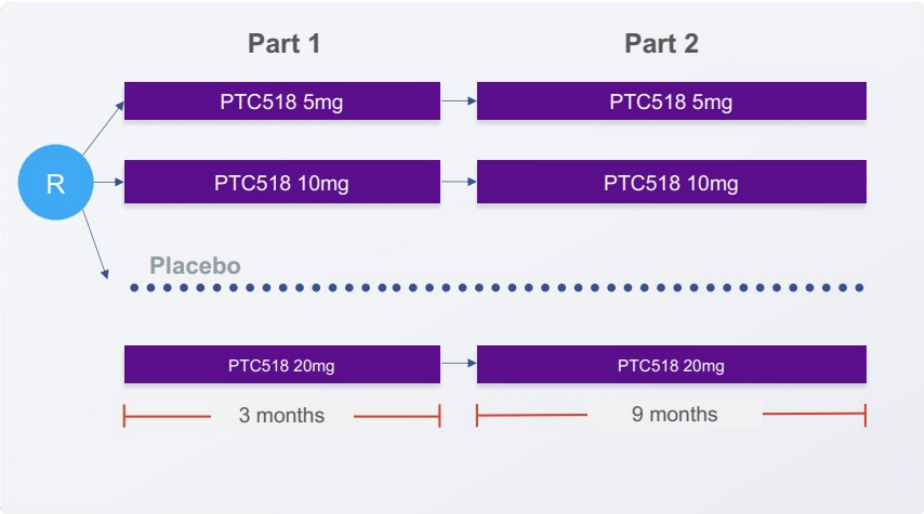
Mechanism of Action

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

Current Treatments

No approved disease modifying treatments

PIVOT-HD is a Global Phase 2 Trial of PTC518 for HD



Primary Endpoints

- Safety and tolerability of PTC518
- Percent reduction in HTT mRNA and protein in blood

Secondary Endpoints

- Percent reduction in mHTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in brain volume on volumetric MRI imaging

PIVOT-HD Expanding Trial Target Population

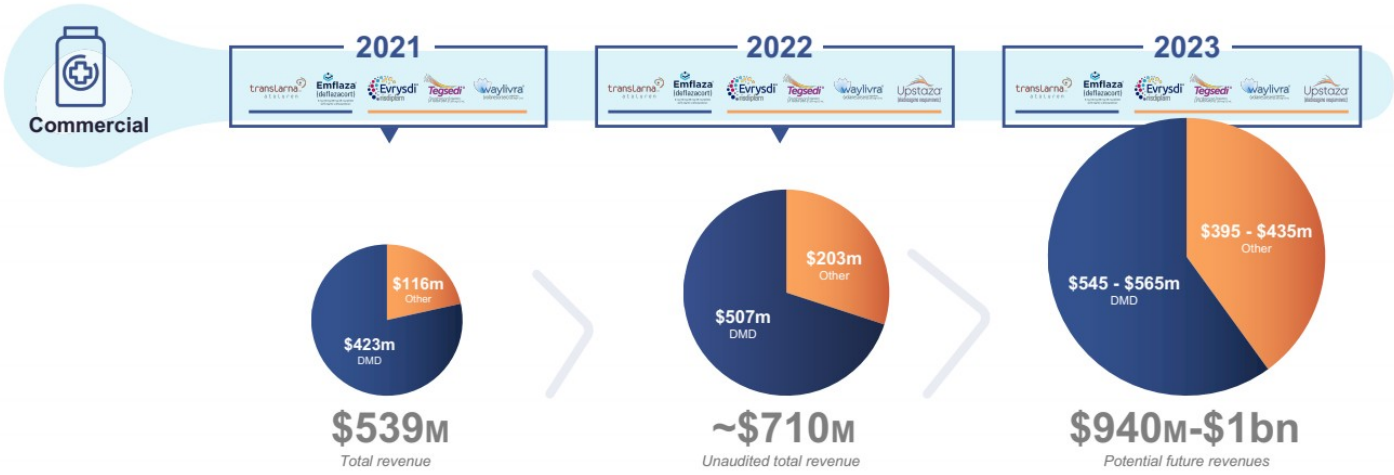
Inclusion Criteria
Stage 2

- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 40-50 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

Inclusion Criteria
Early Stage 3

- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 40-50 inclusive
- Motor and Cognitive Function:
  - UHDRS-IS score of **less than 100**
  - UHDRS TFC score of **11 or 12**
- PIN_{HD} score **0.18 - 4.93**
  - Multivariate calculation including SDMT, TMS, age, CAG

Transformational Commercial Revenue in 2023



Transformational Development Milestones in 2023

Transformational Development Milestones in 2023

PTC 2023

Matthew Klein, M.D., COO
JP Morgan Healthcare Conference

January 9, 2023

PKU Patient

PTC THERAPEUTICS