

**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): **June 8, 2022**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-35969**  
(Commission  
File Number)

**04-3416587**  
(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 5.02. Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.**

PTC Therapeutics, Inc. (the “Company”) held its Annual Meeting on June 8, 2022 (the “Annual Meeting”). At the Annual Meeting, the stockholders of the Company approved an amendment and restatement of the Company’s 2013 Long-Term Incentive Plan (the “Plan,” and the Plan as so amended, the “Restated Plan”). The Restated Plan includes the following material changes to the Plan: (i) the Restated Plan eliminates the evergreen feature that historically provided for an annual increase in the Plan’s share reserve, (ii) the Restated Plan increases the number of shares of our common stock available for issuance under the Plan by 8,475,000 shares, subject to adjustment in the event of stock splits and other similar events, (iii) the Restated Plan newly prohibits the repricing of options and stock appreciation rights (“SARs”) without stockholder approval, (iv) the Restated Plan newly permits the recycling of shares withheld to cover the exercise price or withholding taxes with respect to awards and would deplete the share pool only by shares actually used to settle SARs, (v) under the Restated Plan, the sum of all cash retainers and the reported value of equity compensation provided to the pool of all incumbent non-employee directors for their Board of Directors (the “Board”) service in a given year may not exceed an amount equal to one tenth of one percent (0.1%) of the Company’s weighted average market capitalization for the prior three years (measured on an annual basis) times the number of incumbent non-employee directors; notwithstanding the foregoing, during their first year of service on the Board, newly-appointed or elected non-employee directors will not be included in the foregoing calculation in the year of their appointment and may receive a combination of cash retainers and equity compensation in connection with their initial appointment or election to the Board of up to two (2) times the average incumbent director compensation, (vi) the Restated Plan newly requires recipients of awards from and after June 8, 2022 to agree, upon receipt of an award, to be bound by any clawback policy that we have in effect or may adopt in the future, (vii) the Restated Plan newly requires that awards to non-employee directors must be granted and administered by a committee of the Board, all of the members of which are independent directors as defined by Nasdaq Marketplace Rules, (viii) the Restated Plan newly prohibits the grant of options and SARs with a provision entitling the award holder to the automatic grant of additional options or SARs in connection with any exercise of the original option or SAR, (ix) the Restated Plan newly prohibits the grant of options or SARs that provide for the payment or accrual of dividend equivalents, (x) the Restated Plan newly requires that any dividends or dividend equivalents paid with respect to restricted stock, restricted stock units or other stock-based awards must be subject to the same restrictions on transfer and forfeitability as the award with respect to which it is paid and (xi) the term of the Restated Plan will expire on June 7, 2032, unless extended by stockholder approval in the future, while the Plan was set to expire on June 3, 2023.

The foregoing summary of the Restated Plan is qualified in its entirety by reference to the full text of the Restated Plan, which is attached as Exhibit 10.1 hereto and is incorporated herein by reference.

**Item 5.07. Submission of Matters to a Vote of Security Holders.**

The final results for the votes regarding each proposal at the Annual Meeting are set forth below. As of the record date of the Annual Meeting, April 14, 2022, there were 71,360,039 shares of the Company’s common stock outstanding. Each share of common stock entitled its holder to one vote per share.

The stockholders of the Company elected four Class III directors, each to hold office until the Company’s 2025 annual meeting of stockholders or until his or her successor has been duly elected and qualified, as follows:

<b>Director</b>	<b>For</b>	<b>Withheld</b>	<b>Broker Non-Votes</b>
Allan Jacobson	60,959,512	4,030,869	2,387,625
David P. Southwell	54,623,562	10,366,819	2,387,625
Dawn Svoronos	45,974,645	19,015,736	2,387,625
Alethia Young	64,875,171	115,210	2,387,625

An amendment and restatement to the Company’s 2013 Long-Term Incentive Plan was approved by the Company’s stockholders with 52,087,644 votes “For,” 12,841,178 votes “Against,” 61,559 votes “Abstained,” and 2,387,625 broker non-votes.

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The appointment of Ernst & Young LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2022 was ratified by the Company's stockholders with 66,988,350 votes "For," 373,566 votes "Against," and 16,090 votes "Abstained."

The non-binding advisory proposal on named executive officer compensation was approved by the Company's stockholders with 63,004,106 votes "For," 1,917,418 votes "Against," 68,857 votes "Abstained," and 2,387,625 broker non-votes.

The non-binding advisory proposal on the frequency of the advisory vote to approve named executive officer compensation was selected to be "1 Year" by the Company's stockholders with 64,938,771 votes for "1 Year," 1,848 votes for "2 Years," 11,623 votes for "3 Years," 38,139 votes "Abstained," and 2,387,625 broker non-votes.

After taking into consideration the foregoing voting results and the Board's prior recommendation in favor of an annual advisory vote by stockholders on the compensation of the Company's named executive officers, the Board has determined to hold future advisory votes on the compensation of the Company's named executive officers every year.

#### **Item 7.01. Regulation FD Disclosure.**

The Company will present a company overview at the William Blair 42<sup>nd</sup> Annual Growth Stock Conference (the "Conference") on June 9, 2022 at 2:20 PM eastern time. The Company's corporate presentation will be webcast live on the Events and Presentations page under the Investors section of the Company's website and the presentation slide deck will be posted in the same section of the Company's website. A copy of the slide deck is also attached to this Current Report on Form 8-K (this "Report") as Exhibit 99.1 and is incorporated by reference into this Item 7.01.

The information set forth in or incorporated by reference into Item 7.01, including Exhibit 99.1, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

#### **Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits

<u>Exhibit No.</u>	<u>Description</u>
10.1	<a href="#">PTC Therapeutics, Inc. Amended and Restated 2013 Long-Term Incentive Plan</a>
99.1	<a href="#">Corporate Presentation – William Blair 42<sup>nd</sup> Annual Growth Stock Conference</a>
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: June 9, 2022

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

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## PTC Therapeutics, Inc.

Amended and Restated 2013 Long-Term Incentive Plan1. Purpose

The purpose of this Amended and Restated 2013 Long-Term Incentive Plan (the “**Plan**”) of PTC Therapeutics, Inc., a Delaware corporation (the “**Company**”), is to advance the interests of the Company’s stockholders by enhancing the Company’s ability to attract, retain and motivate persons who are expected to make important contributions to the Company and by providing such persons with equity ownership opportunities and performance-based incentives that are intended to better align the interests of such persons with those of the Company’s stockholders. Except where the context otherwise requires, the term “**Company**” shall include any of the Company’s present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Internal Revenue Code of 1986, as amended, and any regulations thereunder (the “**Code**”) and any other business venture (including, without limitation, joint venture or limited liability company) in which the Company has a controlling interest, as determined by the Board of Directors of the Company (the “**Board**”).

2. Eligibility

All of the Company’s employees, officers and directors, as well as consultants and advisors to the Company (as the terms consultants and advisors are defined and interpreted for purposes of Form S-8 under the Securities Act of 1933, as amended (the “**Securities Act**”), or any successor form) are eligible to be granted Awards under the Plan. Each person who is granted an Award under the Plan is deemed a “**Participant**.” “**Award**” means Options (as defined in Section 5), SARs (as defined in Section 6), Restricted Stock (as defined in Section 7), Restricted Stock Units (as defined in Section 7) and Other Stock-Based Awards (as defined in Section 8).

3. Administration and Delegation

(a) Administration by Board of Directors. The Plan will be administered by the Board. The Board shall have authority to grant Awards and to adopt, amend and repeal such administrative rules, guidelines and practices relating to the Plan as it shall deem advisable. The Board may construe and interpret the terms of the Plan and any Award agreements entered into under the Plan. The Board may correct any defect, supply any omission or reconcile any inconsistency in the Plan or any Award in the manner and to the extent it shall deem expedient and it shall be the sole and final judge of such expediency. All decisions by the Board shall be made in the Board’s sole discretion and shall be final and binding on all persons having or claiming any interest in the Plan or in any Award.

(b) Appointment of Committees. To the extent permitted by applicable law, the Board may delegate any or all of its powers under the Plan to one or more committees or subcommittees of the Board (a “**Committee**”). All references in the Plan to the “**Board**” shall mean the Board or a Committee of the Board or the officers referred to in Section 3(c) to the extent that the Board’s powers or authority under the Plan have been delegated to such Committee or officers.

(c) Delegation to Officers. Subject to any requirements of applicable law (including as applicable Sections 152 and 157(c) of the General Corporation Law of the State of Delaware), the Board may delegate to one or more officers of the Company the power to grant Awards (subject to any limitations under the Plan) to employees or officers of the Company and to exercise such other powers under the Plan as the Board may determine, *provided* that the Board shall fix the terms of Awards to be granted by such officers, the maximum number of shares subject to Awards that the officers may grant, and the time period in which such Awards may be granted; and *provided further*, that no officer shall be authorized to grant such Awards to any “executive officer” of the Company (as defined by Rule 3b-7 under the Securities Exchange Act of 1934, as amended (the “**Exchange Act**”) or to any “officer” of the Company (as defined by Rule 16a-1(f) under the Exchange Act).

(d) Awards to Non-Employee Directors. Awards to non-employee directors will be granted and administered by a Committee, all of the members of which are independent directors as defined by Section 5605(a)(2) of the Nasdaq Marketplace Rules.

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4. Stock Available for Awards

(a) Number of Shares; Share Counting.

(1) Authorized Number of Shares. Subject to adjustment under Section 9, Awards may be made under the Plan (any or all of which Awards may be in the form of Incentive Stock Options, as defined in Section 5(b)) for up to such number of shares of common stock, \$0.001 par value per share, of the Company (the “**Common Stock**”) as is equal to the sum of (A) a number of shares of Common Stock (up to 16,724,212 shares) as is equal to the sum of (x) the number of shares of Common Stock issued under the Plan prior to the Restatement Effective Date, (y) the number of shares of Common Stock that remain available for issuance under the Plan immediately prior to the Restatement Effective Date, and (z) the number of shares of Common Stock subject to Awards granted prior to the Restatement Effective Date that are outstanding as of the Restatement Effective Date plus, (B) from and after the Restatement Effective Date, an additional 8,475,000 shares of Common Stock. Shares issued under the Plan may consist in whole or in part of authorized but unissued shares or treasury shares.

(2) Share Counting. For purposes of counting the number of shares available for the grant of Awards under the Plan:

(A) all shares of Common Stock covered by SARs shall be counted against the number of shares available for the grant of Awards under the Plan; *provided, however*, that (i) SARs that may be settled only in cash shall not be so counted and (ii) if the Company grants an SAR in tandem with an Option for the same number of shares of Common Stock and provides that only one such Award may be exercised (a “**Tandem SAR**”), only the shares covered by the Option, and not the shares covered by the Tandem SAR, shall be so counted, and the expiration of one in connection with the other’s exercise will not restore shares to the Plan;

(B) if any Award expires or is terminated, surrendered or canceled without having been fully exercised, is forfeited in whole or in part (including as the result of shares of Common Stock subject to such Award being repurchased by the Company at the original issuance price pursuant to a contractual repurchase right), is settled in cash or otherwise results in any Common Stock not being issued, the unused Common Stock covered by such Award shall again be available for the grant of Awards under the Plan. Further, shares of Common Stock delivered (either by actual delivery, attestation or net exercise) to the Company by a Participant to exercise an Award or to satisfy tax withholding obligations (including shares retained from the Award creating the tax obligation) shall be added to the number of shares of Common Stock available for the grant of Awards under the Plan. However, in the case of Incentive Stock Options (as hereinafter defined), the foregoing provisions shall be subject to any limitations under the Code.

(b) Substitute Awards. In connection with a merger or consolidation of an entity with the Company or the acquisition by the Company of property or stock of an entity, the Board may grant Awards (“**Substitute Awards**”) in substitution for any options or other stock or stock-based awards granted by such entity or an affiliate thereof. Substitute Awards may be granted on such terms as the Board deems appropriate in the circumstances, notwithstanding any limitations on Awards contained in the Plan. Substitute Awards shall not count against the overall share limit set forth in Section 4(a)(1) or any sublimit contained in the Plan, except as may be required by reason of Section 422 and related provisions of the Code.

(c) Limit on Awards to Non-Employee Directors. The sum of all cash retainers and the reported value of equity compensation provided to the pool of all incumbent non-employee directors for their Board service in a given year shall not exceed an amount equal to one tenth of one percent (0.1%) of the Company’s weighted average market capitalization for the prior three years (measured on an annual basis) times the number of incumbent non-employee directors.

Notwithstanding the foregoing provision, during their first year of service on the Board, newly-appointed or elected non-employee directors will not be included in the foregoing calculation in the year of their appointment

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and may receive a combination of cash retainers and equity compensation in connection with their initial appointment or election to the Board of up to two (2) times the average incumbent director compensation.

5. Stock Options

(a) General. The Board may grant options to purchase Common Stock (each, an “**Option**”) and determine the number of shares of Common Stock to be covered by each Option, the exercise price of each Option and the conditions and limitations applicable to the exercise of each Option, including conditions relating to applicable federal or state securities laws, as it considers necessary or advisable.

(b) Incentive Stock Options. An Option that the Board intends to be an “incentive stock option” as defined in Section 422 of the Code (an “**Incentive Stock Option**”) shall only be granted to employees of PTC Therapeutics, Inc., any of PTC Therapeutics, Inc.’s present or future parent or subsidiary corporations as defined in Sections 424(e) or (f) of the Code, and any other entities the employees of which are eligible to receive Incentive Stock Options under the Code, and shall be subject to and shall be construed consistently with the requirements of Section 422 of the Code. An Option that is not intended to be an Incentive Stock Option shall be designated a “**Nonstatutory Stock Option.**” The Company shall have no liability to a Participant, or any other party, if an Option (or any part thereof) that is intended to be an Incentive Stock Option is not an Incentive Stock Option or if the Company converts an Incentive Stock Option to a Nonstatutory Stock Option.

(c) Exercise Price. The Board shall establish the exercise price of each Option or the formula by which such exercise price will be determined. The exercise price shall be specified in the applicable Option agreement. The exercise price shall be not less than 100% of the Grant Date Fair Market Value (as defined below) of the Common Stock on the date the Option is granted; *provided* that if the Board approves the grant of an Option with an exercise price to be determined on a future date, the exercise price shall be not less than 100% of the Grant Date Fair Market Value on such future date. “**Grant Date Fair Market Value**” of a share of Common Stock for purposes of the Plan will be determined as follows:

(1) if the Common Stock trades on a national securities exchange, the closing sale price (for the primary trading session) on the date of grant; or

(2) if the Common Stock does not trade on any such exchange, the average of the closing bid and asked prices on the date of grant as reported by an over-the-counter marketplace designated by the Board; or

(3) if the Common Stock is not publicly traded, the Board will determine the Grant Date Fair Market Value for purposes of the Plan using any measure of value it determines to be appropriate (including, as it considers appropriate, relying on appraisals) in a manner consistent with the valuation principles under Section 409A of the Code or any successor provision thereto, and the regulations thereunder, except as the Board may expressly determine otherwise.

For any date that is not a trading day, the Grant Date Fair Market Value of a share of Common Stock for such date will be determined by using the closing sale price or average of the bid and asked prices, as appropriate, for the immediately preceding trading day and with the timing in the formulas above adjusted accordingly. The Board can substitute a particular time of day or other measure of “closing sale price” or “bid and asked prices” if appropriate because of exchange or market procedures or can, use weighted averages either on a daily basis or such longer period, in each case to the extent permitted by Section 409A of the Code.

The Board shall determine the Grant Date Fair Market Value for purposes of the Plan, and all Awards are conditioned on the Participant’s agreement that the Board’s determination is conclusive and binding even though others might make a different determination.

(d) Duration of Options. Each Option shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable option agreement; *provided, however*, that no Option will be granted with a term in excess of 10 years.

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(e) Exercise of Options. Options may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with payment in full (in the manner specified in Section 5(f)) of the exercise price for the number of shares for which the Option is exercised. Shares of Common Stock subject to the Option will be delivered by the Company as soon as practicable following exercise.

(f) Payment Upon Exercise. Common Stock purchased upon the exercise of an Option granted under the Plan shall be paid for as follows:

(1) in cash or by check, payable to the order of the Company;

(2) except as may otherwise be provided in the applicable Option agreement or approved by the Board, in its sole discretion, by (i) delivery of an irrevocable and unconditional undertaking by a creditworthy broker to deliver promptly to the Company sufficient funds to pay the exercise price and any required tax withholding or (ii) delivery by the Participant to the Company of a copy of irrevocable and unconditional instructions to a creditworthy broker to deliver promptly to the Company cash or a check sufficient to pay the exercise price and any required tax withholding;

(3) to the extent provided for in the applicable Option agreement or approved by the Board, in its sole discretion, by delivery (either by actual delivery or attestation) of shares of Common Stock owned by the Participant valued at their fair market value (valued in the manner determined or approved by the Board), provided (i) such method of payment is then permitted under applicable law, (ii) such Common Stock, if acquired directly from the Company, was owned by the Participant for such minimum period of time, if any, as may be established by the Board in its discretion and (iii) such Common Stock is not subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements;

(4) to the extent provided for in the applicable Nonstatutory Stock Option agreement or approved by the Board in its sole discretion, by delivery of a notice of “net exercise” to the Company, as a result of which the Participant would receive (i) the number of shares underlying the portion of the Option being exercised, less (ii) such number of shares as is equal to (A) the aggregate exercise price for the portion of the Option being exercised divided by (B) the fair market value of the Common Stock (valued in the manner determined or approved by the Board) on the date of exercise;

(5) to the extent permitted by applicable law and provided for in the applicable Option agreement or approved by the Board, in its sole discretion, by payment of such other lawful consideration as the Board may determine; or

(6) by any combination of the above permitted forms of payment.

(g) Limitation on Repricing. Unless such action is approved by the Company’s stockholders, the Company may not (except as provided for under Section 9): (1) amend any outstanding Option granted under the Plan to provide an exercise price per share that is lower than the then-current exercise price per share of such outstanding Option; (2) cancel any outstanding option (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan (other than Awards granted pursuant to Section 4(b)) covering the same or a different number of shares of Common Stock and having an exercise or measurement price per share lower than the then-current exercise price per share of the canceled option; (3) cancel in exchange for a cash payment any outstanding Option with an exercise price per share above the then-current fair market value of the Common Stock (valued in the manner determined or approved by the Board); or (4) take any other action under the Plan that constitutes a “repricing” within the meaning of the rules of the Nasdaq Stock Market or any other exchange or marketplace on which the Company’s stock is listed or traded (the “*Exchange*”).

(h) No Reload Options. No Option granted under the Plan shall contain any provision entitling the Participant to the automatic grant of additional Options in connection with any exercise of the original Option.

(i) No Dividend Equivalents. No Option shall provide for the payment or accrual of dividend equivalents.

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6. Stock Appreciation Rights

(a) General. The Board may grant Awards consisting of stock appreciation rights (“**SARs**”) entitling the holder, upon exercise, to receive an amount of Common Stock or cash or a combination thereof (such form to be determined by the Board) determined by reference to appreciation, from and after the date of grant, in the fair market value of a share of Common Stock (valued in the manner determined or approved by the Board) over the measurement price established pursuant to Section 6(b). The date as of which such appreciation is determined shall be the exercise date.

(b) Measurement Price. The Board shall establish the measurement price of each SAR and specify it in the applicable SAR agreement. The measurement price shall not be less than 100% of the Grant Date Fair Market Value of the Common Stock on the date the SAR is granted; *provided* that if the Board approves the grant of an SAR effective as of a future date, the measurement price shall be not less than 100% of the Grant Date Fair Market Value on such future date.

(c) Duration of SARs. Each SAR shall be exercisable at such times and subject to such terms and conditions as the Board may specify in the applicable SAR agreement; *provided, however*, that no SAR will be granted with a term in excess of 10 years.

(d) Exercise of SARs. SARs may be exercised by delivery to the Company of a notice of exercise in a form (which may be electronic) approved by the Company, together with any other documents required by the Board.

(e) Limitation on Repricing. Unless such action is approved by the Company’s stockholders, the Company may not (except as provided for under Section 9): (1) amend any outstanding SAR granted under the Plan to provide a measurement price per share that is lower than the then-current measurement price per share of such outstanding SAR; (2) cancel any outstanding SAR (whether or not granted under the Plan) and grant in substitution therefor new Awards under the Plan (other than Awards granted pursuant to Section 4(b)) covering the same or a different number of shares of Common Stock and having an exercise or measurement price per share lower than the then-current measurement price per share of the canceled SAR; (3) cancel in exchange for a cash payment any outstanding SAR with a measurement price per share above the then-current fair market value of the Common Stock (valued in the manner determined or approved by the Board); or (4) take any other action under the Plan that constitutes a “repricing” within the meaning of the rules of the Exchange.

(f) No Reload SARs. No SAR granted under the Plan shall contain any provision entitling the Participant to the automatic grant of additional SARs in connection with any exercise of the original SAR.

(g) No Dividend Equivalents. No SAR shall provide for the payment or accrual of dividend equivalents.

7. Restricted Stock; Restricted Stock Units

(a) General. The Board may grant Awards entitling recipients to acquire shares of Common Stock (“**Restricted Stock**”), subject to the right of the Company to repurchase all or part of such shares at their issue price or other stated or formula price (or to require forfeiture of such shares if issued at no cost) from the recipient in the event that conditions specified by the Board in the applicable Award are not satisfied prior to the end of the applicable restriction period or periods established by the Board for such Award. The Board may also grant Awards entitling the recipient to receive shares of Common Stock or cash to be delivered at the time such Award vests (“**Restricted Stock Units**”) (Restricted Stock and Restricted Stock Units are each referred to herein as a “**Restricted Stock Award**”).

(b) Terms and Conditions for All Restricted Stock Awards. The Board shall determine the terms and conditions of a Restricted Stock Award, including the conditions for vesting and repurchase (or forfeiture) and the issue price, if any.

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(c) Additional Provisions Relating to Restricted Stock.

(1) Dividends. Any dividends (whether paid in cash, stock or property) declared and paid by the Company with respect to shares of Restricted Stock ("**Accrued Dividends**") shall be paid to the Participant only if and when such shares become free from the restrictions on transferability and forfeitability that apply to such shares. Each payment of Accrued Dividends will be made no later than the end of the calendar year in which the dividends are paid to stockholders of that class of stock or, if later, the 15th day of the third month following the lapsing of the restrictions on transferability and the forfeitability provisions applicable to the underlying shares of Restricted Stock. No interest will be paid on Accrued Dividends.

(2) Stock Certificates/Issuance. The Company may require that any stock certificates issued in respect of shares of Restricted Stock, as well as dividends or distributions paid on such Restricted Stock, shall be deposited in escrow by the Participant, together with a stock power endorsed in blank, with the Company (or its designee) or, alternatively, that such shares be issued in book entry only, in the name of the Participant with appropriate transfer and forfeiture restrictions. At the expiration of the applicable restriction periods, the Company (or such designee) shall deliver the certificates no longer subject to such restrictions (or, to the extent the Restricted Stock was issued in book entry, remove the restrictions) to the Participant or if the Participant has died, to his or her Designated Beneficiary. "**Designated Beneficiary**" means (i) the beneficiary designated, in a manner determined by the Board, by a Participant to receive amounts due or exercise rights of the Participant in the event of the Participant's death or (ii) in the absence of an effective designation by a Participant, the Participant's estate.

(d) Additional Provisions Relating to Restricted Stock Units.

(1) Settlement. Upon the vesting of and/or lapsing of any other restrictions (i.e., settlement) with respect to each Restricted Stock Unit, the Participant shall be entitled to receive from the Company the number of shares of Common Stock set forth in the applicable Award agreement or (if so provided in the applicable Award agreement) an amount of cash equal to the fair market value (valued in the manner determined or approved by the Board) of one of such number of shares of Common Stock. The Board may, in its discretion, provide that settlement of Restricted Stock Units shall be deferred, on a mandatory basis or at the election of the Participant in a manner that complies with Section 409A of the Code.

(2) Voting Rights. A Participant shall have no voting rights with respect to any Restricted Stock Units.

(3) Dividend Equivalents. The Award agreement for Restricted Stock Units may provide Participants with the right to receive an amount equal to any dividends or other distributions declared and paid on an equal number of outstanding shares of Common Stock ("**Dividend Equivalents**"). Dividend Equivalents may be credited to an account for the Participant and may be settled in cash and/or shares of Common Stock, in each case to the extent provided in the applicable Award agreement. Dividend Equivalents with respect to Restricted Stock Units will be subject to the same restrictions on transfer and forfeitability as the Restricted Stock Units with respect to which paid. No interest will be paid on Dividend Equivalents.

8. Other Stock-Based Awards

(a) General. Other Awards of shares of Common Stock, and other Awards that are valued in whole or in part by reference to, or are otherwise based on, shares of Common Stock or other property, may be granted hereunder to Participants ("**Other Stock-Based Awards**"). Such Other Stock-Based Awards shall also be available as a form of payment in the settlement of other Awards granted under the Plan or as payment in lieu of compensation to which a Participant is otherwise entitled. Other Stock-Based Awards may be paid in shares of Common Stock or cash, as the Board shall determine.

(b) Terms and Conditions. Subject to the provisions of the Plan, the Board shall determine the terms and conditions of each Other Stock-Based Award, including any purchase price applicable thereto.

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(c) Dividend Equivalents. The Award agreement for an Other Stock-Based Award may provide Participants with the right to receive Dividend Equivalents. Dividend Equivalents may be credited to an account for the Participant and may be settled in cash and/or shares of Common Stock, in each case to the extent provided in the applicable Award agreement. Dividend Equivalents with respect to Other Stock-Based Awards will be subject to the same restrictions on transfer and forfeitability as the Other Stock-Based Award with respect to which paid. No interest will be paid on Dividend Equivalents.

9. Adjustments for Changes in Common Stock and Certain Other Events

(a) Changes in Capitalization. In the event of any stock split, reverse stock split, stock dividend, recapitalization, combination of shares, reclassification of shares, spin-off or other similar change in capitalization or event, or any dividend or distribution to holders of Common Stock other than an ordinary cash dividend, (i) the number and class of securities available under the Plan, (ii) the share counting rules set forth in Section 4(a), (iii) the number and class of securities and exercise price per share of each outstanding Option, (iv) the share and per-share provisions and the measurement price of each outstanding SAR, (v) the number of shares subject to and the repurchase price per share subject to each outstanding Restricted Stock Award and (vi) the share and per-share-related provisions and the purchase price, if any, of each outstanding Other Stock-Based Award, shall be equitably adjusted by the Company (or substituted Awards may be made, if applicable) in the manner determined by the Board. Without limiting the generality of the foregoing, in the event the Company effects a split of the Common Stock by means of a stock dividend and the exercise price of and the number of shares subject to an outstanding Option are adjusted as of the date of the distribution of the dividend (rather than as of the record date for such dividend), then an optionee who exercises an Option between the record date and the distribution date for such stock dividend shall be entitled to receive, on the distribution date, the stock dividend with respect to the shares of Common Stock acquired upon such Option exercise, notwithstanding the fact that such shares were not outstanding as of the close of business on the record date for such stock dividend.

(b) Reorganization Events.

(1) Definition. A “**Reorganization Event**” shall mean: (a) any merger or consolidation of the Company with or into another entity as a result of which all of the Common Stock of the Company is converted into or exchanged for the right to receive cash, securities or other property or is cancelled, (b) any transfer or disposition of all of the Common Stock of the Company for cash, securities or other property pursuant to a share exchange or other transaction or (c) any liquidation or dissolution of the Company.

(2) Consequences of a Reorganization Event on Awards Other than Restricted Stock.

(A) In connection with a Reorganization Event, the Board may take any one or more of the following actions as to all or any (or any portion of) outstanding Awards other than Restricted Stock on such terms as the Board determines (except to the extent specifically provided otherwise in an applicable Award agreement or another agreement between the Company and the Participant): (i) provide that such Awards shall be assumed, or substantially equivalent Awards shall be substituted, by the acquiring or succeeding corporation (or an affiliate thereof), (ii) upon written notice to a Participant, provide that all of the Participant’s unexercised Awards will terminate immediately prior to the consummation of such Reorganization Event unless exercised by the Participant (to the extent then exercisable) within a specified period following the date of such notice, (iii) provide that outstanding Awards shall become exercisable, realizable, or deliverable, or restrictions applicable to an Award shall lapse, in whole or in part prior to or upon such Reorganization Event, (iv) in the event of a Reorganization Event under the terms of which holders of Common Stock will receive upon consummation thereof a cash payment for each share surrendered in the Reorganization Event (the “**Acquisition Price**”), make or provide for a cash payment to Participants with respect to each Award held by a Participant equal to (A) the number of shares of Common Stock subject to the vested portion of the Award (after giving effect to any acceleration of vesting that occurs upon or immediately prior to such Reorganization Event) multiplied by (B) the excess, if any, of (I) the Acquisition Price over (II) the exercise, measurement or purchase price of such Award and any applicable tax withholdings, in exchange for the termination of such Award, (v) provide that, in connection with a liquidation or dissolution of the Company, Awards shall convert into the right to receive liquidation proceeds (if applicable, net of the exercise, measurement or purchase price thereof and any applicable tax withholdings) and (vi) any combination

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of the foregoing. In taking any of the actions permitted under this Section 9(b)(2), the Board shall not be obligated by the Plan to treat all Awards, all Awards held by a Participant, or all Awards of the same type, identically.

(B) Notwithstanding the terms of Section 9(b)(2)(A), in the case of outstanding Restricted Stock Units that are subject to Section 409A of the Code: (i) if the applicable Restricted Stock Unit agreement provides that the Restricted Stock Units shall be settled upon a “change in control event” within the meaning of Treasury Regulation Section 1.409A-3(i)(5)(i), and the Reorganization Event constitutes such a “change in control event”, then no assumption or substitution shall be permitted pursuant to Section 9(b)(2)(A)(i) and the Restricted Stock Units shall instead be settled in accordance with the terms of the applicable Restricted Stock Unit agreement; and (ii) the Board may only undertake the actions set forth in clauses (iii), (iv) or (v) of Section 9(b)(2)(A) if the Reorganization Event constitutes a “change in control event” as defined under Treasury Regulation Section 1.409A-3(i)(5)(i) and such action is permitted or required by Section 409A of the Code; if the Reorganization Event is not a “change in control event” as so defined or such action is not permitted or required by Section 409A of the Code, and the acquiring or succeeding corporation does not assume or substitute the Restricted Stock Units pursuant to clause (i) of Section 9(b)(2)(A), then the unvested Restricted Stock Units shall terminate immediately prior to the consummation of the Reorganization Event without any payment in exchange therefor.

(C) For purposes of Section 9(b)(2)(A)(i), an Award (other than Restricted Stock) shall be considered assumed if, following consummation of the Reorganization Event, such Award confers the right to purchase or receive pursuant to the terms of such Award, for each share of Common Stock subject to the Award immediately prior to the consummation of the Reorganization Event, the consideration (whether cash, securities or other property) received as a result of the Reorganization Event by holders of Common Stock for each share of Common Stock held immediately prior to the consummation of the Reorganization Event (and if holders were offered a choice of consideration, the type of consideration chosen by the holders of a majority of the outstanding shares of Common Stock); *provided, however*, that if the consideration received as a result of the Reorganization Event is not solely common stock of the acquiring or succeeding corporation (or an affiliate thereof), the Company may, with the consent of the acquiring or succeeding corporation, provide for the consideration to be received upon the exercise or settlement of the Award to consist solely of such number of shares of common stock of the acquiring or succeeding corporation (or an affiliate thereof) that the Board determined to be equivalent in value (as of the date of such determination or another date specified by the Board) to the per share consideration received by holders of outstanding shares of Common Stock as a result of the Reorganization Event.

(3) Consequences of a Reorganization Event on Restricted Stock. Upon the occurrence of a Reorganization Event other than a liquidation or dissolution of the Company, the repurchase and other rights of the Company with respect to outstanding Restricted Stock shall inure to the benefit of the Company’s successor and shall, unless the Board determines otherwise, apply to the cash, securities or other property which the Common Stock was converted into or exchanged for pursuant to such Reorganization Event in the same manner and to the same extent as they applied to such Restricted Stock; *provided, however*, that the Board may provide for termination or deemed satisfaction of such repurchase or other rights under the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, either initially or by amendment. Upon the occurrence of a Reorganization Event involving the liquidation or dissolution of the Company, except to the extent specifically provided to the contrary in the instrument evidencing any Restricted Stock or any other agreement between a Participant and the Company, all restrictions and conditions on all Restricted Stock then outstanding shall automatically be deemed terminated or satisfied.

#### 10. General Provisions Applicable to Awards

(a) Transferability of Awards. Awards shall not be sold, assigned, transferred, pledged or otherwise encumbered by the person to whom they are granted, either voluntarily or by operation of law, except by will or the laws of descent and distribution or, other than in the case of an Incentive Stock Option, pursuant to a qualified domestic relations order, and, during the life of the Participant, shall be exercisable only by the Participant; *provided, however*, that the Board may permit or provide in an Award for the gratuitous transfer of the Award by the Participant to or for the benefit of any immediate family member, family trust or other entity established for the benefit of the Participant and/or an immediate family member thereof if the Company would be eligible to use a Form S-8 under the Securities Act for the registration of the sale of the Common Stock subject to such Award to such proposed transferee; *provided further*, that the Company shall not be required to recognize any such permitted

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transfer until such time as such permitted transferee shall, as a condition to such transfer, deliver to the Company a written instrument in form and substance satisfactory to the Company confirming that such transferee shall be bound by all of the terms and conditions of the Award. References to a Participant, to the extent relevant in the context, shall include references to authorized transferees. For the avoidance of doubt, nothing contained in this Section 10(a) shall be deemed to restrict a transfer to the Company.

(b) Documentation. Each Award shall be evidenced in such form (written, electronic or otherwise) as the Board shall determine. Each Award may contain terms and conditions in addition to those set forth in the Plan.

(c) Board Discretion. Except as otherwise provided by the Plan, each Award may be made alone or in addition or in relation to any other Award. The terms of each Award need not be identical, and the Board need not treat Participants uniformly.

(d) Termination of Status. The Board shall determine the effect on an Award of the disability, death, termination or other cessation of employment, authorized leave of absence or other change in the employment or other status of a Participant and the extent to which, and the period during which, the Participant, or the Participant's legal representative, conservator, guardian or Designated Beneficiary, may exercise rights under the Award.

(e) Withholding. The Participant must satisfy all applicable federal, state, and local or other income and employment tax withholding obligations before the Company will deliver stock certificates or otherwise recognize ownership of Common Stock under an Award. The Company may elect to satisfy the withholding obligations through additional withholding on salary or wages. If the Company elects not to or cannot withhold from other compensation, the Participant must pay the Company the full amount, if any, required for withholding or have a broker tender to the Company cash equal to the withholding obligations. Payment of withholding obligations is due before the Company will issue any shares on exercise, vesting or release from forfeiture of an Award or at the same time as payment of the exercise or purchase price, unless the Company determines otherwise. If provided for in an Award or approved by the Board, a Participant may satisfy such tax obligations in whole or in part by delivery (either by actual delivery or attestation) of shares of Common Stock, including shares retained from the Award creating the tax obligation, valued at their fair market value (valued in the manner determined or approved by the Company); *provided, however*, except as otherwise provided by the Board, that the total tax withholding where stock is being used to satisfy such tax obligations cannot exceed the Company's minimum statutory withholding obligations (based on minimum statutory withholding rates for federal and state tax purposes, including payroll taxes, that are applicable to such supplemental taxable income), except that, to the extent that the Company is able to retain shares of Common Stock having a fair market value (determined or approved by the Company) that exceeds the statutory minimum applicable withholding tax without financial accounting implications or the Company is withholding in a jurisdiction that does not have a statutory minimum withholding tax, the Company may retain such number of shares of Common Stock (up to the number of shares having a fair market value equal to the maximum individual statutory rate of tax (determined or approved by, the Company)) as the Company shall determine to be necessary to satisfy the tax liability associated with any Award. Shares used to satisfy tax withholding requirements cannot be subject to any repurchase, forfeiture, unfulfilled vesting or other similar requirements.

(f) Amendment of Award. Subject to Sections 5(g) and 6(e), the Board may amend, modify or terminate any outstanding Award, including but not limited to, substituting therefor another Award of the same or a different type, changing the date of exercise or realization, and converting an Incentive Stock Option to a Nonstatutory Stock Option. The Participant's consent to such action shall be required unless (i) the Board determines that the action, taking into account any related action, does not materially and adversely affect the Participant's rights under the Plan or (ii) the change is permitted under Section 9.

(g) Conditions on Delivery of Stock. The Company will not be obligated to deliver any shares of Common Stock pursuant to the Plan or to remove restrictions from shares previously issued or delivered under the Plan until (i) all conditions of the Award have been met or removed to the satisfaction of the Company, (ii) in the opinion of the Company's counsel, all other legal matters in connection with the issuance and delivery of such shares have been satisfied, including any applicable securities laws and regulations and any applicable stock exchange or stock market rules and regulations, and (iii) the Participant has executed and delivered to the Company

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such representations or agreements as the Company may consider appropriate to satisfy the requirements of any applicable laws, rules or regulations.

(h) Acceleration. The Board may at any time provide that any Award shall become immediately exercisable in whole or in part, free of some or all restrictions or conditions, or otherwise realizable in whole or in part, as the case may be.

#### 11. Miscellaneous

(a) No Right To Employment or Other Status. No person shall have any claim or right to be granted an Award by virtue of the adoption of the Plan, and the grant of an Award shall not be construed as giving a Participant the right to continued employment or any other relationship with the Company. The Company expressly reserves the right at any time to dismiss or otherwise terminate its relationship with a Participant free from any liability or claim under the Plan, except as expressly provided in the applicable Award.

(b) No Rights As Stockholder; Clawback. Subject to the provisions of the applicable Award, no Participant or Designated Beneficiary shall have any rights as a stockholder with respect to any shares of Common Stock to be distributed with respect to an Award until becoming the record holder of such shares. In accepting an Award under the Plan granted on or after the Restatement Effective Date, the Participant agrees to be bound by any clawback policy that the Company has in effect or may adopt in the future.

(c) Effective Date and Term of Plan. The Plan originally became effective on June 25, 2013. This amendment and restatement of the Plan shall become effective on the date the Plan is approved by the Company's stockholders at the 2022 Annual Meeting of Stockholders (the "**Restatement Effective Date**"). No Awards shall be granted under the Plan after the expiration of 10 years from the Restatement Effective Date, but Awards previously granted may extend beyond that date.

(d) Amendment of Plan. The Board may amend, suspend or terminate the Plan or any portion thereof at any time provided that (i) neither Section 5(g) nor Section 6(e) requiring stockholder approval of an Option or SAR repricing may be amended without stockholder approval and (ii) no amendment that would require stockholder approval under the rules of the national securities exchange on which the Company then maintains its primary listing will be effective unless and until the Company's stockholders approve such amendment. In addition, if at any time the approval of the Company's stockholders is required as to any other modification or amendment under Section 422 of the Code or any successor provision with respect to Incentive Stock Options, the Board may not effect such modification or amendment without such approval. Unless otherwise specified in the amendment, any amendment to the Plan adopted in accordance with this Section 11(d) shall apply to, and be binding on the holders of, all Awards outstanding under the Plan at the time the amendment is adopted, *provided* the Board determines that such amendment, taking into account any related action, does not materially and adversely affect the rights of Participants under the Plan. No Award shall be made that is conditioned upon stockholder approval of any amendment to the Plan unless the Award provides that (i) it will terminate or be forfeited if stockholder approval of such amendment is not obtained within no more than 12 months from the date of grant and (ii) it may not be exercised or settled (or otherwise result in the issuance of Common Stock) prior to such stockholder approval.

(e) Authorization of Sub-Plans (including for Grants to non-U.S. Employees). The Board may from time to time establish one or more sub-plans under the Plan for purposes of satisfying applicable securities, tax or other laws of various jurisdictions. The Board shall establish such sub-plans by adopting supplements to the Plan containing (i) such limitations on the Board's discretion under the Plan as the Board deems necessary or desirable or (ii) such additional terms and conditions not otherwise inconsistent with the Plan as the Board shall deem necessary or desirable. All supplements adopted by the Board shall be deemed to be part of the Plan, but each supplement shall apply only to Participants within the affected jurisdiction and the Company shall not be required to provide copies of any supplement to Participants in any jurisdiction which is not the subject of such supplement.

(f) Compliance with Section 409A of the Code. If and to the extent (i) any portion of any payment, compensation or other benefit provided to a Participant pursuant to the Plan in connection with his or her employment termination constitutes "nonqualified deferred compensation" within the meaning of Section 409A of the Code and (ii) the Participant is a specified employee as defined in Section 409A(a)(2)(B)(i) of the Code, in each

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case as determined by the Company in accordance with its procedures, by which determinations the Participant (through accepting the Award) agrees to be bound, such portion of the payment, compensation or other benefit shall not be paid before the day that is six months plus one day after the date of "separation from service" (as determined under Section 409A of the Code) (the "***New Payment Date***"), except as Section 409A of the Code may then permit. The aggregate of any payments that otherwise would have been paid to the Participant during the period between the date of separation from service and the New Payment Date shall be paid to the Participant in a lump sum on such New Payment Date, and any remaining payments will be paid on their original schedule. The Company makes no representations or warranty and shall have no liability to the Participant or any other person if any provisions of or payments, compensation or other benefits under the Plan are determined to constitute nonqualified deferred compensation subject to Section 409A of the Code but do not to satisfy the conditions of that section.

(g) Limitations on Liability. Notwithstanding any other provisions of the Plan, no individual acting as a director, officer, employee or agent of the Company will be liable to any Participant, former Participant, spouse, beneficiary, or any other person for any claim, loss, liability, or expense incurred in connection with the Plan, nor will such individual be personally liable with respect to the Plan because of any contract or other instrument he or she executes in his or her capacity as a director, officer, employee or agent of the Company. The Company will indemnify and hold harmless each director, officer, employee or agent of the Company to whom any duty or power relating to the administration or interpretation of the Plan has been or will be delegated, against any cost or expense (including attorneys' fees) or liability (including any sum paid in settlement of a claim with the Board's approval) arising out of any act or omission to act concerning the Plan unless arising out of such person's own fraud or bad faith.

(h) Governing Law. The provisions of the Plan and all Awards made hereunder shall be governed by and interpreted in accordance with the laws of the State of Delaware, excluding choice-of-law principles of the law of such state that would require the application of the laws of a jurisdiction other than the State of Delaware.

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# PTC Therapeutics

## William Blair Growth Stock Conference

Matthew Klein, M.D. *Chief Operating Officer*

Kylie O'Keefe, *SVP Global Commercial and  
Corporate Strategy*

June 9, 2022



# Forward Looking Statements

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

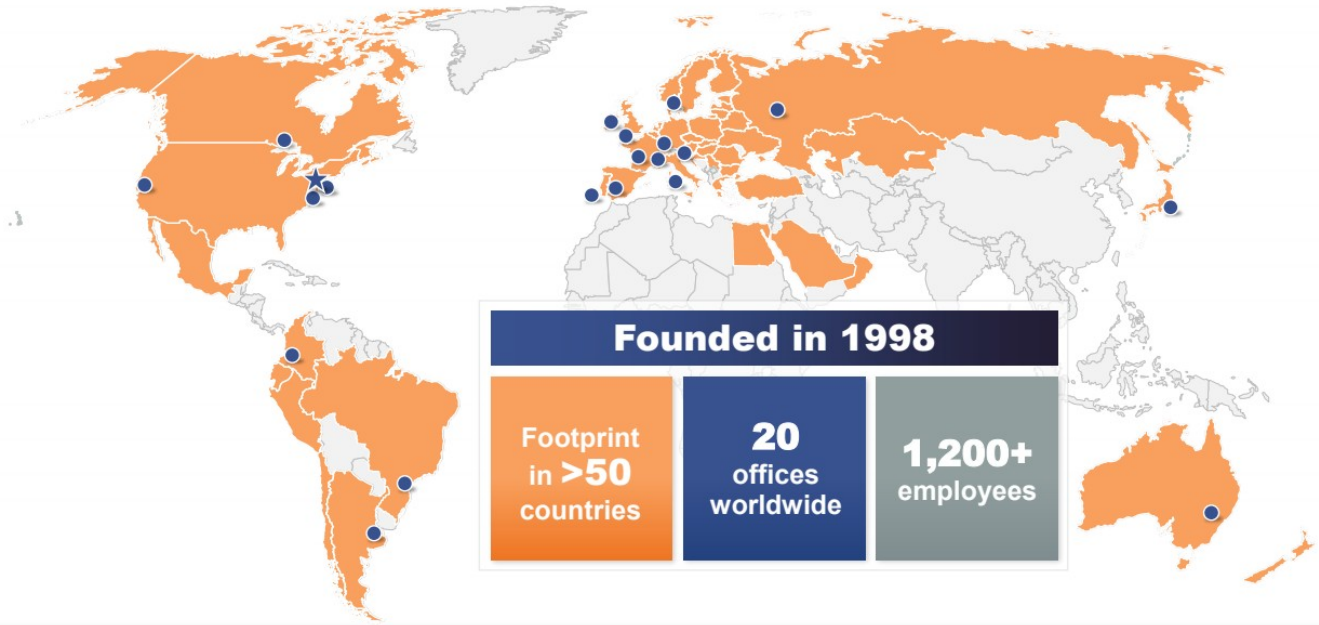
PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: expectations with respect to the COVID-19 pandemic and related response measures 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 Upstaza.

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



# PTC Has a Growing Global Footprint



# Diversified Platform Drives Strong Portfolio

## SCIENTIFIC PLATFORMS and RESEARCH

	Deflazacort	LatAm Commercial	Nonsense Mutation	Splicing	Gene Therapy	Bio-e	Metabolic	Oncology	Virology	
Commercial	 Emflaza <sup>®</sup> (deflazacort) 6mg, 18mg, 30mg, 50mg tablets 20mg/5mL oral suspension	 Tegsedi <sup>®</sup> (inotersen) waylivra <sup>®</sup> (waylivra)	 transLarna <sup>®</sup> aluren	 Evrysdi <sup>®</sup> risdiplam	 Upstaza <sup>®</sup> (etabocagene exuparvovec)					
Clinical			US Ataluren	PTC518 HD		Vatiquinone MDAS Vatiquinone FA PTC857 ALS	PTC923 PKU	Unesbulin DIPG Unesbulin LMS Emvododstat AML	Emvododstat COVID-19	
Research			2 Undisclosed	SCA-3 MAP-Tau 8 Undisclosed	FA Angelman IRDs Cog Disorders	3 Undisclosed		3 Undisclosed		

AADC, aromatic L-amino acid decarboxylase deficiency; AML, acute myeloid leukemia; COVID-19, coronavirus disease 2019; DIPG, diffuse intrinsic pontine glioma; FA, Friedrich'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. \* Positive CHMP opinion, EMA ratification expected in July

Potential registrational studies

Early-stage programs

# Strong Financial Performance Driven by Innovation



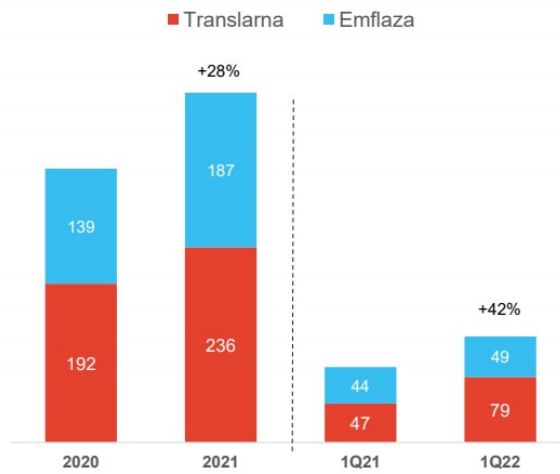
\*Non-GAAP measure which excludes estimated non-cash, stock-based compensation expense of approximately \$115 million. GAAP R&D and SG&A expense for the full year 2022 is anticipated to be between \$915 and \$965 million.

# Strong DMD Franchise Growth Expected to Continue



- Treatment for nonsense mutation DMD for ages 2 and older
- Distributed in 50+ countries
- New patients in existing geographies and geographic expansion

DMD Franchise Revenue



- First and only corticosteroid approved for DMD; approved for all US DMD patients >2yrs
- Data show clinical benefit over prednisone
- New patients starts, continued high compliance and operational excellence

# Evrysdi Revenue Demonstrates Benefits of Safe and Effective Therapy for SMA



## Benefits of Evrysdi for SMA Patients

- First at-home, oral treatment for SMA
- Patients treated across all SMA types
- Patients are treatment-naïve or previously treated with Spinraza, Zolgensma
- Broadest range of ages treated

## Newly Approved Indication in US for Pre-Symptomatic Infants



Based on interim data from the RAINBOWFISH study



Showed 80 percent of pre-symptomatic infants with SMA treated with Evrysdi for at least 12 months achieved motor milestones



Including sitting without support, rolling, crawling, standing unaided, and walking independently

# Leveraging Regulatory Expertise and Commercial Infrastructure to Support Patients in Latin America



- 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

Commercial efforts ongoing

Application submitted for additional indication of FPL



- For treatment of familial chylomicronaemia syndrome (FCS)
- LATAM patients benefiting through early-access programs
- Received Category 1 pricing, in final pricing negotiations

# Upstaza™ Has the Potential to Provide Significant Benefit to AADC Patients

Upstaza™  
(eladocagene exuparvovec)

## 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 approved disease-modifying treatments

## Mechanism of Action



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

~5,000  
Global  
Prevalence



Subject 011-311

**Baseline (pre-gene therapy):  
1 year and 7 months of age**



# Upstaza™ Gene Therapy Opportunity and Launch Preparation



## Regulatory

Positive CHMP opinion received in May, ratification expected in July

Upstaza™ BLA submission expected in 4Q22



## Disease Education

Development of virtual education: disease-specific webinars and congress symposia

Engaging with patient advocacy groups and payers



## Treatment Centers

Identification and preparation of expert pediatric neurosurgical centers

Continued KOL engagement



## Market Opportunity

Potential over \$1B in cumulative revenue

Successful patient finding is ongoing

# Pipeline Progress Planned in 2022

## Study Initiations



2022 Q1

2022 Q2

2022 Q3

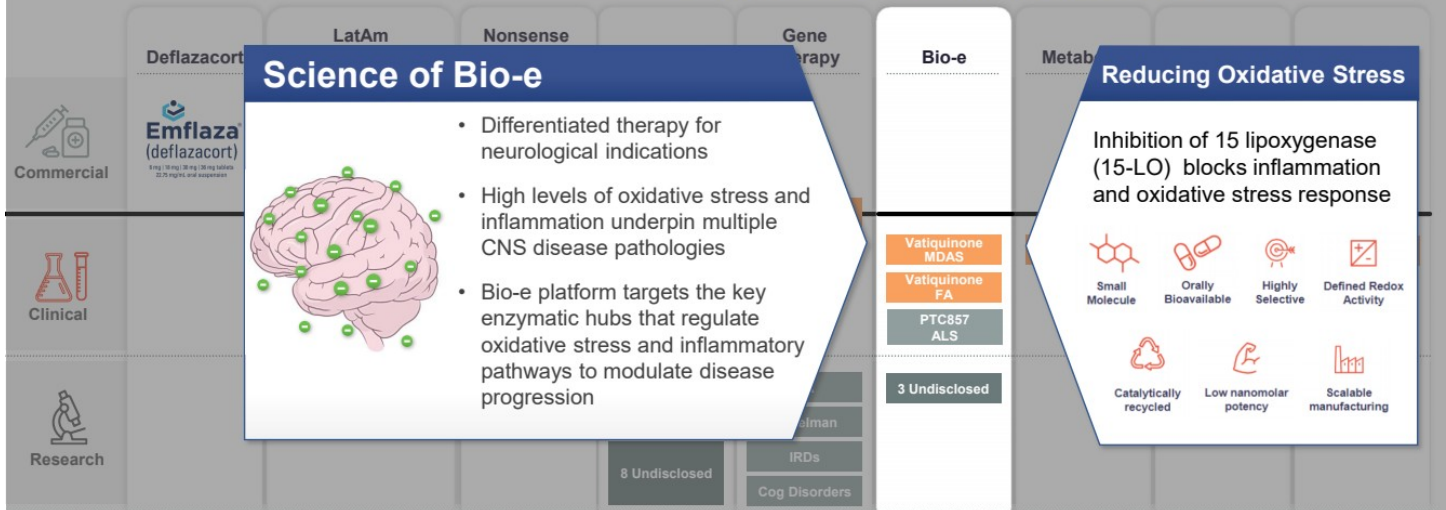
2022 Q4



## Study Results

# Diversified Platform Drives Strong Portfolio

## SCIENTIFIC PLATFORMS and RESEARCH



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

Potential registrational studies

Early-stage programs

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



## Disease



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

## Current Treatments



No approved disease-modifying treatments

## Mechanism of Action

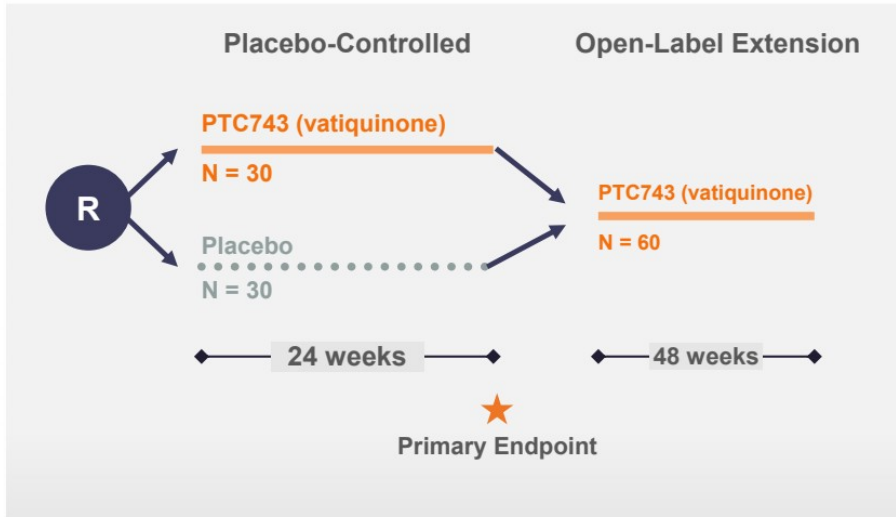


Vatiquinone targets 15-lipoxygenase, a regulator of the key energetic and oxidative stress pathways that underpin seizures in these patients

**~20,000**  
Global  
Prevalence



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



**Primary Endpoint**

Change from baseline in frequency of observable motor seizures

**Trial Status**

- Enrolling
- Data expected 4Q 2022

# 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

~25,000  
Global  
Prevalence

## Current Treatments



No approved disease-modifying therapies

## Opportunity

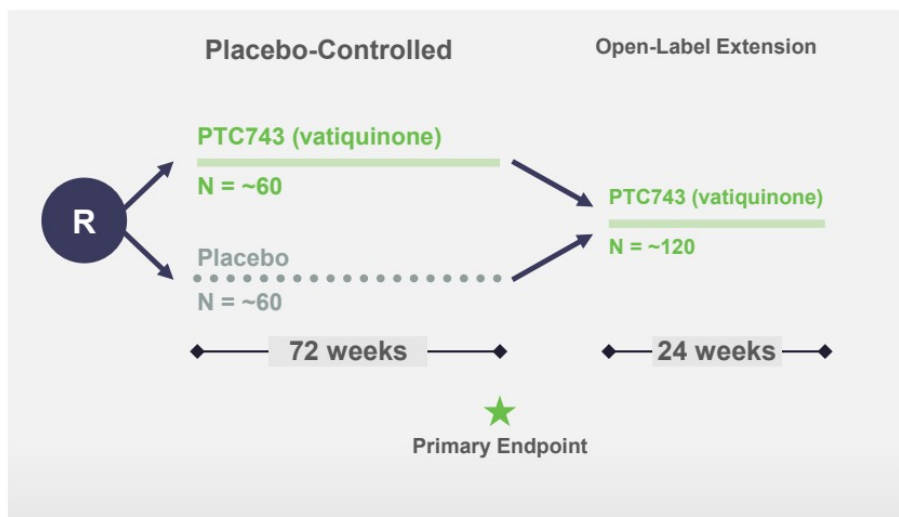


Vatiquinone targets 15-lipoxygenase, a regulator of key energetic and oxidative stress pathways that are disrupted in FA



# Vatiquinone Has the Potential to Provide Improvement in Neurological Function

MOVE-FA



**Primary Endpoint**  
Change in mFARS

**Key Secondary Endpoint**  
Change in FA-ADL

### Trial Status

✓ Enrollment complete

• Data expected in 2Q 2023

# PTC857 Has the Potential to Slow Disease Progression in ALS

## Disease



Amyotrophic lateral sclerosis (ALS) is a rapidly progressing neurodegenerative disease caused by oxidative damage, which leads to neuronal cell death and muscular atrophy

## Current Treatments



No approved disease-modifying therapies

## Mechanism of Action



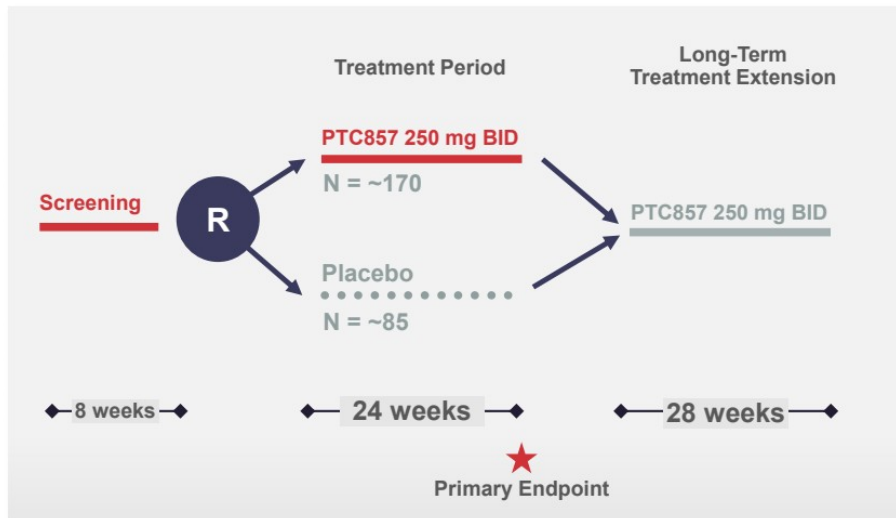
PTC857 inhibits pathways leading to oxidative damage and ferroptosis, resulting in protection of motor neurons

  
~150,000  
Global  
Prevalence





# PTC857 Has the Potential to Slow Disease Progression in ALS



## Primary Endpoints

Change in ALSFRS-R

## Secondary Endpoints

Safety and PK

## Trial Status

- Enrolling
- Trial initiated in Q1 2022

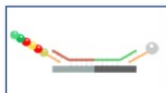
# Diversified Platform Drives Strong Portfolio

## SCIENTIFIC PLATFORMS and RESEARCH

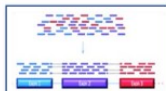
### Leaders in Splicing Technology



Databases of Splicing Targets



Isoform plex



HTSpliceseq

### Splicing



PTC518 HD

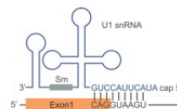
SCA-3

MAP-Tau

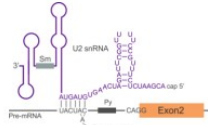
8 Undisclosed

### 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, Friedrich's ataxia; GBA, glucocerebrosidase; HD, Huntington's disease; IRD, inherited retinal dystrophy; LMS, leiomyosarcoma; MDAS, mitochondrial disease associated seizures; PD, Parkinson's disease; PKU, phenylketonuria; SCA-3, spinocerebellar ataxia type 3.

Potential registrational studies

Early-stage programs

# PTC518 Reduces HTT mRNA and Protein to Target HD

PIVOT<sup>HD</sup>

## 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

~135,000  
Global  
Prevalence



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



Generally well tolerated



Consistent pharmacology



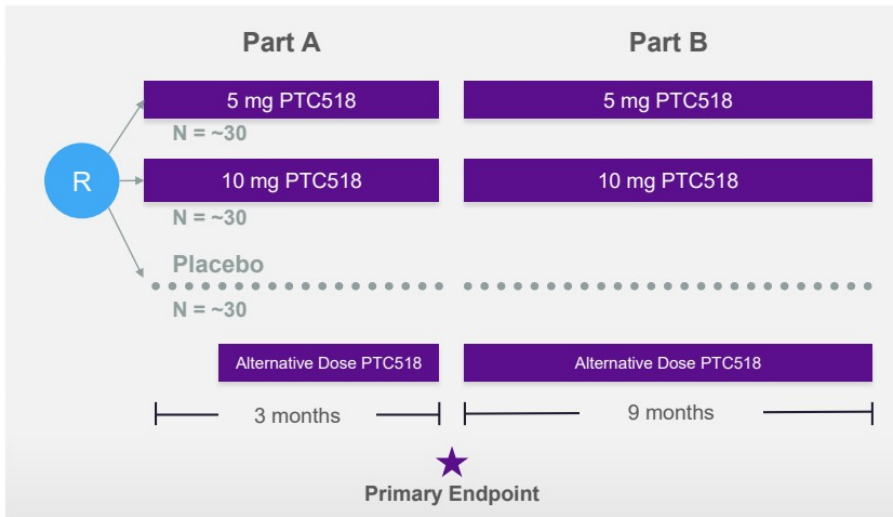
Dose-dependent reduction of HTT mRNA and protein



Crosses blood-brain barrier and is not effluxed

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

- Enrolling
- Trial initiated in Q1 2022

# PTC518 Has the Potential to Reduce HTT Protein in Huntington Patients



## Inclusion Criteria

- Ambulatory Huntington's patients ages 25 and older
- CAG repeats 42-50 inclusive
- Motor and Cognitive Function:
  - UHDRS-IS score of 100
  - UHDRS TFC score of 13
- $PIN_{HD}$  score 0.18 - 4.93
  - Multivariate calculation including SDMT, TMS, age, CAG

## Primary endpoints

- Safety and tolerability of PTC518 in Huntington's disease patients
- Percent reduction in HTT mRNA and protein in blood

## Secondary endpoints

- Percent reduction in HTT protein in CSF
- Changes in neurofilament light chain (NfL) in plasma and CSF
- Change in caudate, putamenal, ventricular volume on volumetric MRI imaging
- Changes in clinical scales of motor and cognitive function

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

## Disease



Phenylketonuria (PKU) is a metabolic condition caused by mutations to phenylalanine hydroxylase, which 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



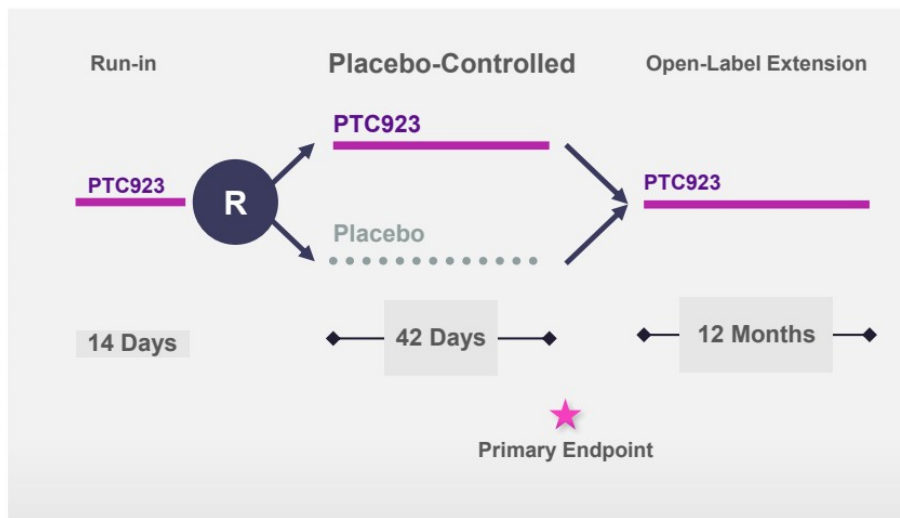
PTC923 is a more bioavailable precursor than exogenously administered synthetic BH4 and has the potential to treat a broader range of PKU patients

**~58,000**  
Global  
Prevalence

 aphenity



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



**Primary Endpoint**  
Reduction in blood phenylalanine levels

**Trial Status**

- Enrolling
- Data expected YE 2022



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



## Disease



Leiomyosarcoma (LMS) is a rare and aggressive cancer with tumors found in smooth muscle

## Current Treatments



Several chemotherapeutics are utilized but offer minimal meaningful clinical benefit

## Mechanism of Action



Unesbulin is an oral small molecule tubulin inhibitor that arrests tumor cells, including cancer stem cells, in G2/M phase by inhibiting tubulin polymerization

~4,000  
diagnosed  
annually  
in US



Not an actual LMS patient.

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

Percentage Change for SLD from Baseline

◆ 21 Day Treatment Cycles ◆

## Phase 1b Study Design

Ascending doses 200, 300 and 400 mg unesbulin + 1000mg/m<sup>2</sup> dacarbazine

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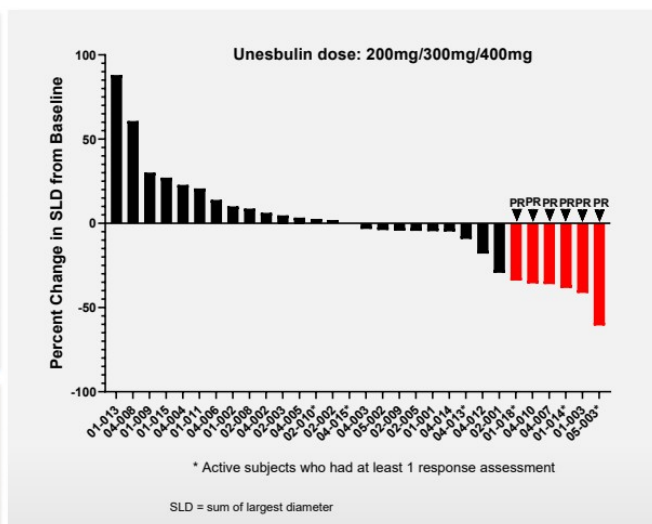
N = 29

**Inclusion Criteria**

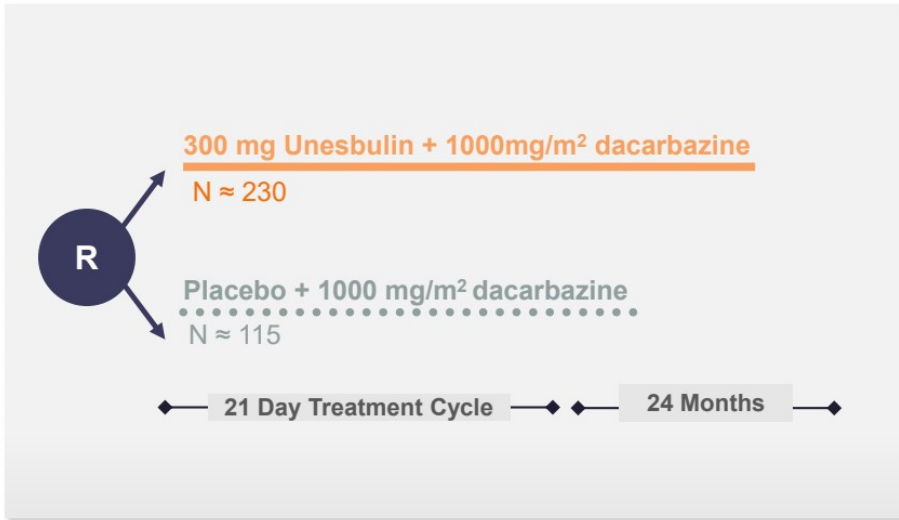
- Patients with locally advanced or metastatic LMS intolerant or refractory to standard therapy
- Any number of previous lines of treatments allowed

### 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

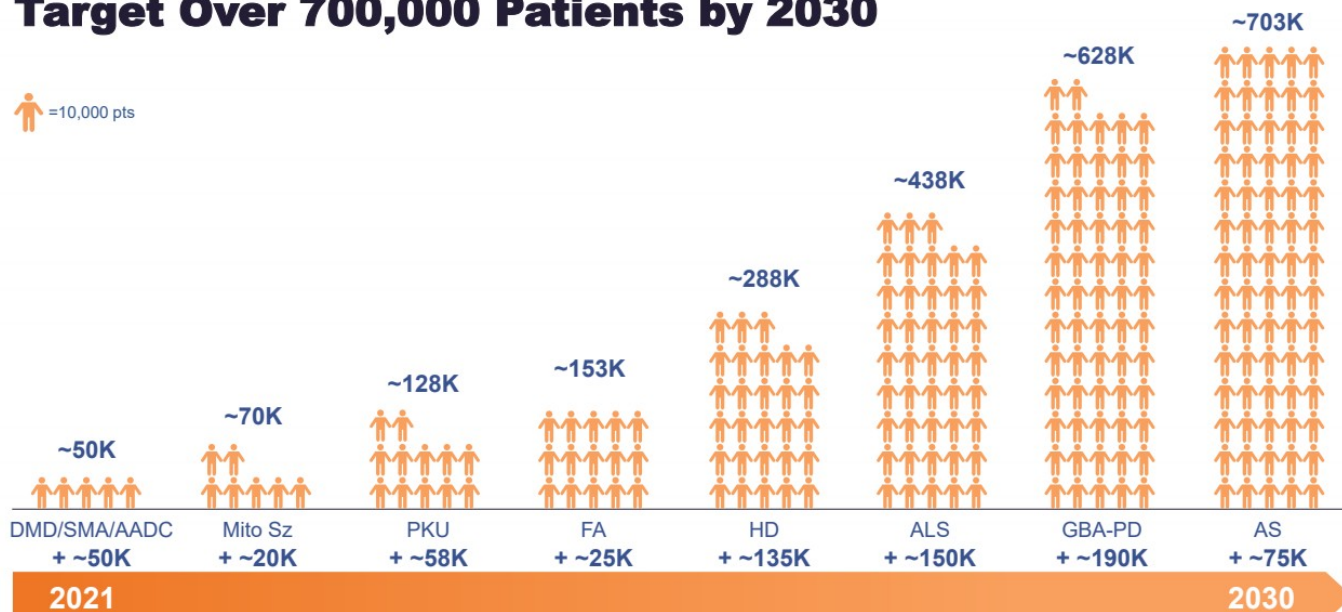
**Interim Analysis**

**Trial Status**

- Enrolling
- Initiated in Q1 2022

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

 = 10,000 pts



Estimated Global Prevalence

# Enduring Innovation Drives Value Creation

