# 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 17, 2024**

# 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.) |

| **500 Warren Corporate Center Drive** | |
|---|---|
| **Warren, NJ** | **07059** |
| (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 1.01. Entry into a Material Definitive Agreement.**

*Amendment No. 1 to Amended and Restated Royalty Purchase Agreement and First Put Option Exercise Agreement*

On June 17, 2024, PTC Therapeutics, Inc. (the "Company"), Royalty Pharma Investments 2019 ICAV ("RPI"), and, for the limited purposes set forth in the Purchase Agreement (as defined below), Royalty Pharma plc, entered into an Amendment No. 1 to Amended and Restated Royalty Purchase Agreement and First Put Option Exercise Agreement (the "Amendment"), which amends that certain Amended and Restated Royalty Purchase Agreement dated as of October 18, 2023 (the "Purchase Agreement").

Under the Purchase Agreement, the Company sold to RPI a certain portion of the Company's right to receive sales-based royalty payments on worldwide net sales of Roche's Evrysdi® (risdiplam) product and any other product developed pursuant to the License and Collaboration Agreement, dated as of November 23, 2011, by and among the Company, F. Hoffman-La Roche Ltd., Hoffman-La Roche Inc. (together with F. Hoffman-La Roche Ltd, "Roche"), and, for the limited purposes set forth therein, the Spinal Muscular Atrophy Foundation (such payments, the "Royalty"). The Purchase Agreement provided, among other things, the Company with five $100.0 million put options pursuant to which the Company could sell to RPI, after the closing of the Purchase Agreement and before December 31, 2025, its retained portion of the Royalty in exchange for total cash consideration of up to $500.0 million, less royalties received through the applicable put closing date in respect of the put portion of the Royalty for net sales occurring on or after October 1, 2023.

The Amendment modifies the size and number of put options available to be exercised by the Company under the Purchase Agreement, without changing the total cash consideration payable to the Company or the total amount of the Royalty to be sold to RPI in connection with the exercise of all the put options. The Amendment also removes from the Purchase Agreement the call options previously held by RPI to purchase up to 9.5112% of the Royalty. The Amendment provides that the Company has the option to sell its retained portions of the Royalty to RPI in up to four tranches for the following payments: (1) $250.0 million for the first tranche in exchange for 9.5112% of the Royalty, which increases to 16.6667% after the 2020 Assigned Royalty Cap (as defined in the Purchase Agreement) has been met, (2) $100.0 million for the second tranche in exchange for 3.8045% of the Royalty, which increases to 6.6667% after the 2020 Assigned Royalty Cap has been met, (3) $100.0 million for the third tranche in exchange for 3.8045% of the Royalty, which increases to 6.6667% after the 2020 Assigned Royalty Cap has been met, and (4) $50.0 million for the fourth tranche in exchange for 1.9021% of the Royalty, which increases to 3.3332% after the 2020 Assigned Royalty Cap has been met, in each case less royalties received through the applicable put closing date in respect of the put portion of the Royalty for net sales occurring on or after October 1, 2023. In connection with the closing of the Amendment, the Company exercised its put option to sell the first tranche to RPI, resulting in the Company receiving $250.0 million in cash consideration, less royalties received through June 17, 2024 in respect of the put portion of the Royalty for net sales occurring on or after October 1, 2023, in exchange for 9.5112% of the Royalty with respect to net sales arising after April 1, 2024, which increases to 16.6667% of the Royalty after the 2020 Assigned Royalty Cap has been met. As of the closing of the first tranche, the Company retains 9.5111% of the Royalty, which increases to 16.6666% after the 2020 Assigned Royalty Cap has been met.

The foregoing description of the material terms of the Amendment is qualified in its entirety by reference to the full text of the Amendment, which will be filed as an exhibit to the Company's Quarterly Report on Form 10-Q for the fiscal quarter ending June 30, 2024.

*Amendment and Restatement of Hopewell Lease*

In connection with the disposition of certain assets related to early-stage gene therapy programs, on June 17, 2024, the Company and Hopewell Campus Owner, LLC, as successor-in-interest to Bristol-Myers Squibb Company (the "Landlord"), entered into an amendment and restatement of the lease of office, production and laboratory space at a facility located in Hopewell Township, New Jersey filed as Exhibit 10.24 to the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2023 (the "Lease"). The amendments to the Lease significantly reduce the space and corresponding rent subject to the Lease.

As a result of the amendments to the Lease, the Company no longer considers the Lease to be material.

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

The Company held its Annual Meeting on June 18, 2024 (the "Annual Meeting"). The 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 22, 2024, there were 76,696,655 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 II directors, each to hold office until the Company's 2027 annual meeting of stockholders or until his or her successor has been duly elected and qualified, as follows:

| Director | For | Withheld | Broker Non-Votes |
|---|---|---|---|
| Emma Reeve | 63,383,975 | 2,829,938 | 4,104,129 |
| Michael Schmertzler | 64,029,610 | 2,184,303 | 4,104,129 |
| Mary Smith | 64,717,219 | 1,496,694 | 4,104,129 |
| Glenn D. Steele, Jr., M.D., Ph.D. | 64,598,479 | 1,615,434 | 4,104,129 |

The appointment of Ernst & Young LLP as the Company's independent registered public accounting firm for the fiscal year ending December 31, 2024 was ratified by the Company's stockholders with 70,118,603 votes "For," 181,310 votes "Against," and 18,129 votes "Abstained."

The non-binding advisory proposal on named executive officer compensation was approved by the Company's stockholders with 64,786,256 votes "For," 1,278,678 votes "Against," 148,979 votes "Abstained," and 4,104,129 broker non-votes.

**Item 7.01. Regulation FD Disclosure.**

The Company will host a conference call on June 20, 2024 at 8:00 a.m. Eastern time. During this conference call, the Company expects to discuss interim data from its Phase 2 study of PTC518 for the treatment of Huntington's disease. Directions on how to access the conference call and a summary of the interim data are included in the press release furnished as Exhibit 99.1 hereto. A copy of the slide deck that will be presented during the conference call is furnished as Exhibit 99.2 hereto.

The information in this Item 7.01 of this Current Report on Form 8-K (this "Report"), including Exhibits 99.1 and 99.2, shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended, or the Exchange Act, except as expressly set forth by specific reference in such a filing. All website addresses given in this Report or incorporated herein by reference are for information only and are not intended to be an active link or to incorporate any website information into this Report.

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

(d) Exhibits

| Exhibit No. | Description |
|---|---|
| 99.1 | Press Release, dated June 20, 2024 issued by PTC Therapeutics, Inc. |
| 99.2 | Corporate Presentation — PIVOT-HD Interim Results |
| 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 20, 2024

By: /s/ Pierre Gravier
Name: Pierre Gravier
Title: Chief Financial Officer

**Exhibit 99.1**

# Interim PIVOT-HD Results Demonstrate Evidence of Favorable CNS Biomarker and Clinical Effects at Month 12 in Huntington's Disease Patients

*- FDA lifts PTC518 partial clinical hold based on PIVOT-HD data -*

*- Conference call and webcast to be held June 20th at 8:00 am EDT -*

**WARREN, N.J., June 20, 2024** -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today shared interim results from the Phase 2 PIVOT-HD study of PTC518 in Huntington's disease (HD) patients. At Month 12, PTC518 treatment resulted in dose-dependent lowering of mutant huntingtin (mHTT) protein in the blood and cerebrospinal fluid (CSF) in the interim cohort of patients. In addition, favorable trends were demonstrated on several relevant HD clinical assessments including Total Motor Score (TMS) and Composite Unified Huntington's Disease Rating Scale (cUHDRS). Furthermore, following 12 months of treatment, PTC518 continues to be safe and well tolerated.

"The evidence of both CNS biomarker and early clinical effects at Month 12 along with the continued favorable tolerability profile supports the promise of PTC518 to address the need for an effective and safe disease-modifying therapy for patients living with Huntington's disease," said Dr. Matthew B. Klein, Chief Executive Officer, PTC Therapeutics, Inc. "With these data in hand, we look forward to the next steps in the PTC518 development program."

At Month 12, durability of dose-dependent mHTT lowering in the blood was demonstrated with lowering of 22% and 43% for 5mg and 10mg dose levels, respectively. In the CSF, dose dependent mHTT lowering was also demonstrated with lowering of 21% and 43%, for 5mg and 10mg dose levels, respectively. In addition, at Month 12, PTC518 treatment resulted in a notable slowing in progression of motor symptoms as assessed by the TMS (2.0 points worsening for 5mg and 1.3 points worsening for 10mg vs. 4.9 points worsening for placebo).

In addition, PTC announced that the FDA has lifted the partial clinical hold on the program based on review of the PIVOT-HD data.

**Today's Conference Call and Webcast**
PTC will hold a conference call at 8:00 am EDT today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

**About PTC518**
PTC is developing a potential treatment for Huntington's disease based on our splicing platform technology. PTC518, a small molecule that can be taken orally, reduces the production of the mutated Huntingtin protein that leads to injury and death of the neuron, which results in disease progression. The orally bioavailable small molecule penetrates the blood brain barrier, is selective, titratable, and not effluxed – which are key differentiation properties.

**About Huntington's Disease**
Huntington's disease (HD) is a rare, hereditary, genetic disorder of the central nervous system.1 It is caused by a defective gene. This gene produces a protein, called Huntingtin, which is involved in the functioning of the nerve cells in the brain (neurons). When the gene is defective, it produces an abnormal (or mutated) Huntingtin protein that is toxic and causes neuron damage and neuron death.2 HD usually presents in people who are in their 30s or 40s. Symptoms can present earlier in life, and this is called the Juvenile HD.2,3 There are also cases of infantile HD, when symptoms develop in children who are younger than 10 years old.2 While symptoms vary from person to person, the disease primarily affects

the brain and results in abnormal movements, difficulties with speech, swallowing and walking, as well as a number of other symptoms including behavioral, cognitive and motor symptoms. 4,5 While there are therapies approved for specific disease symptoms, currently, there is no cure for HD and there are no approved drugs that delay the onset or slow disease progression.

**About PTC Therapeutics, Inc.**
PTC is a 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 Facebook, Instagram, LinkedIn and Twitter at @PTCBio.

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**Forward-Looking Statement**
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this press release, other than statements of historic fact, are forward-looking statements, including statements with respect to the future expectations, plans and prospects for PTC, PTC's strategy, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words, "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; 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; 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.

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.

**Acronyms:**

CNS: Central Nervous System

CSF: Cerebrospinal Fluid

FDA: U.S. Food and Drug Administration

**References:**


1. World Health Organization, 2020. 8A01.10 Huntington disease. Available at: https://icd.who.int/browse11/l-m/en#/http://id.who.int/icd/entity/2132180242 Accessed October 2021.
2. Gatto EM, González Rojas N, Persi G, et al. Clin Parkinsonism Rel Disord 2020;3:100056.
3. Tabrizi SJ, Flower MD, Ross CA, et al. Nat Rev Neurol 2020;16(10):529–546.
4. Roos RAC. Orphanet J Rare Dis 2010;5:40.
5. Kirkwood SC, Su JL, Conneally P, et al. Arch Neurol 2001;58(2):273–278.

# PIVOT-HD Interim Results

Matthew Klein, M.D.
CEO

June 2024

# 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 the future expectations, plans and prospects f PTC, PTC's strategy, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters, 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," similar expressions.

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# Week 12 Interim Readout Met Key Objectives

**PTC518 treatment resulted in dose-dependent lowering** of HTT mRNA and protein levels in blood cells

**PTC518 demonstrated desired CSF exposure** with higher concentrations of free drug in the CSF than plasma

**PTC518 was well tolerated** with no treatment-related serious adverse events and no reports of peripheral neuropathy

**CSF NfL levels remained stable** after 12 weeks of treatment with no treatment-related spikes

# Key Objectives for Month 12 Interim Results

Demonstrate durability of HTT protein lowering with PTC518 in blood cells

Demonstrate evidence of activity on disease biomarkers

Demonstrate evidence of CNS activity on functional clinical scales

Demonstrate continue safety and tolerability PTC518 over 12 mont

# Evidence of Durability of Effect, Safety and Dose-Dependent Benefit on Clinical Measures

**Dose-dependent and durable lowering of HTT protein** in blood at 12 months

**Dose-dependent lowering of CSF mHTT levels**

**Dose-dependent trends of improvement on key clinical measures** including TMS and cUHDRS

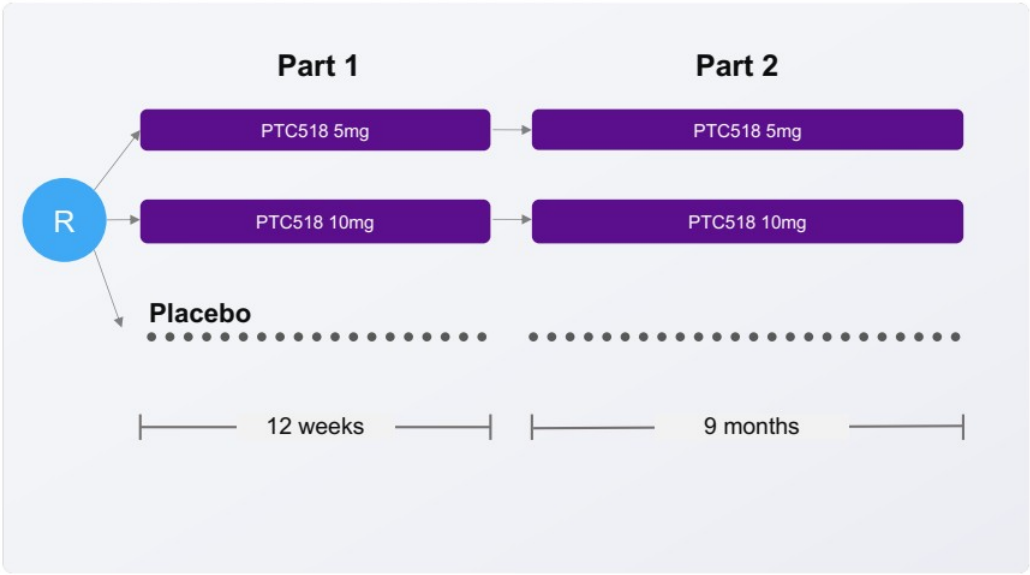
**PTC518 was well tolerated with no evidence of treatment-related NfL spikes** at 12 mont

**FDA Partial Clinical Hold Lifted**



Note: Results based on interim readout

# PIVOT-HD Study Design




## Week 12 Endpoints

- Safety and tolerability
- Blood HTT mRNA and protein lowering
- CNS exposure

## Month 12 Endpoints

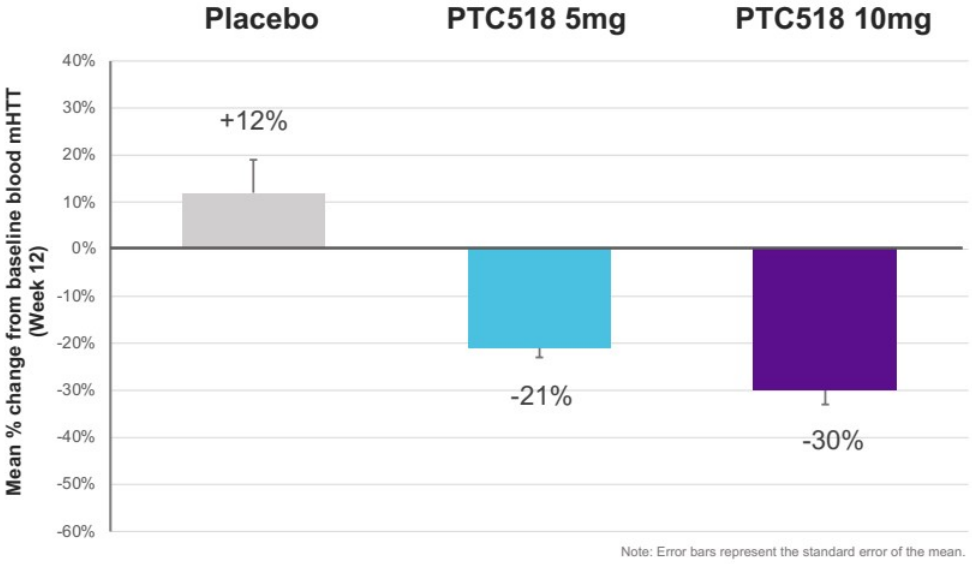
- CSF HTT protein lowering
- CNS biomarkers
- HD clinical scales

# Cohort of Month 12 Patient Characteristics

| Category | Placebo (N=10) | PTC518 5mg (N=10) | PTC518 10mg (N=12) | Overall (N=32) |
|---|---|---|---|---|
| **Age (years) mean** | 47.4 | 45.3 | 46.9 | 46.6 |
| **Gender, n (%)** | | | | |
| **Male** | 7 (70.0%) | 4 (40.0%) | 7 (58.3%) | 18 (56.3%) |
| **Female** | 3 (30.0%) | 6 (60.0%) | 5 (41.7%) | 14 (43.8%) |
| **CAG length** | | | | |
| **Mean (SD)** | 44.3 (2.00) | 44.1 (2.13) | 43.7 (2.31) | 44.0 (2.11) |
| **Min – Max** | 42 – 47 | 42 – 49 | 42 – 50 | 42 – 50 |
| **TFC (Total Functional Capacity) Score** | | | | |
| **Mean** | 13 | 13 | 13 | 13 |

# Blood HTT Protein Lowering Results

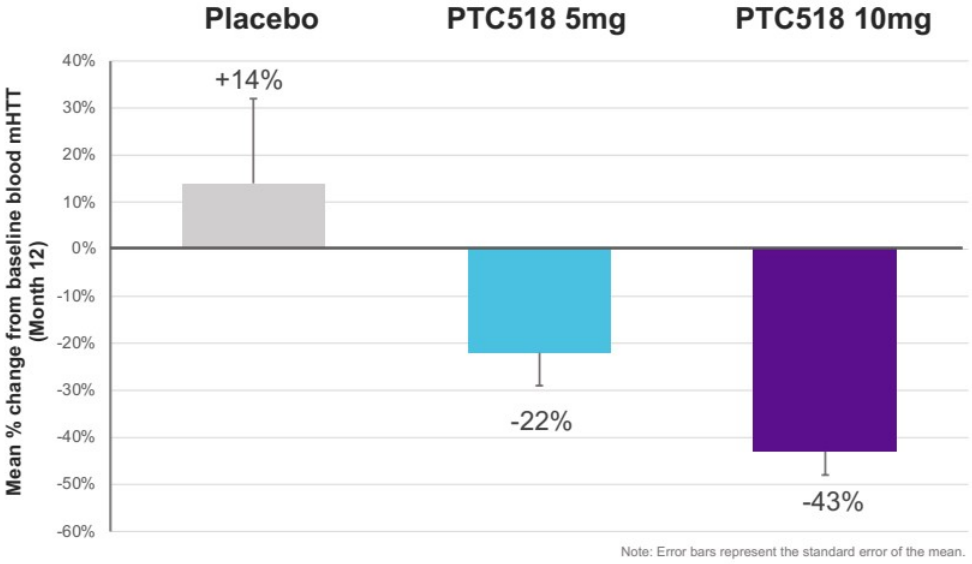
# PTC518 Treatment Resulted in Dose-Dependent Blood mHTT Protein Lowering at Week 12 (June 2023)




Note: Error bars represent the standard error of the mean.

Dose-dependent lowering of HTT protein

# PTC518 Treatment Results in Durable, Dose-Dependent Blood mHTT Protein Lowering at Month 12




Note: Error bars represent the standard error of the mean.

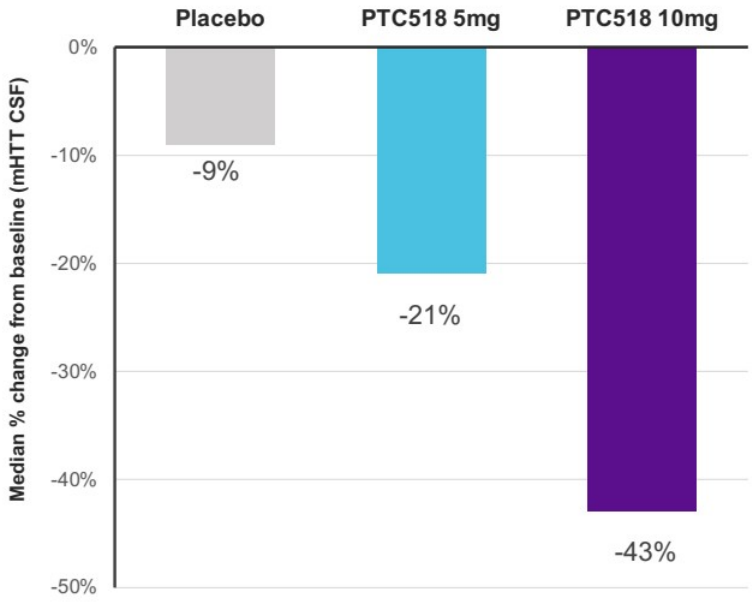
Dose-dependent lowering of HTT protein with steady state reached at 6-9 months



Note: For each subject the mean values of pre- and post-treatments are used

# Biomarker and Clinical Scale Results

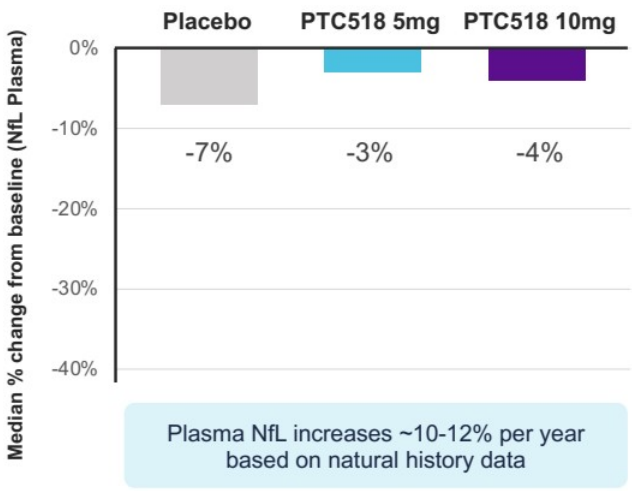
# PTC518 Treatment Results in Dose-Dependent CSF mHTT Protein Lowering at Month 12




CSF protein lowering consistent with protein lowering in blood

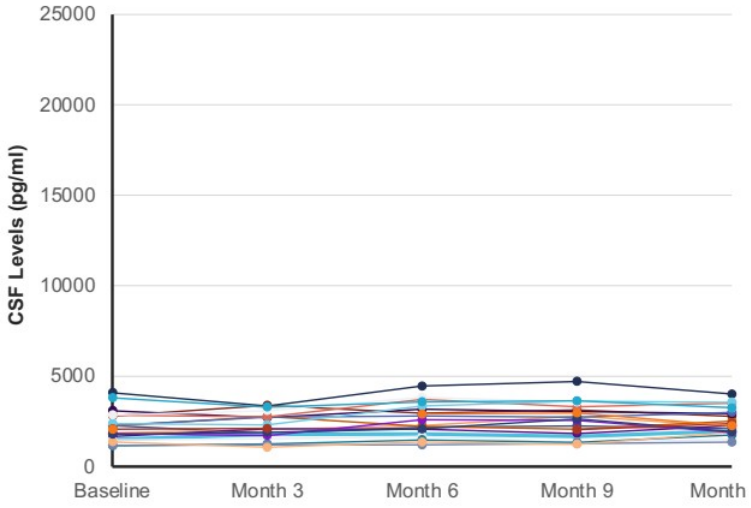
# NfL Levels Consistent Across Treatment Groups With No Evidence of Treatment-Related Spikes

**Change in Plasma NfL Levels from Baseline to Month 12**



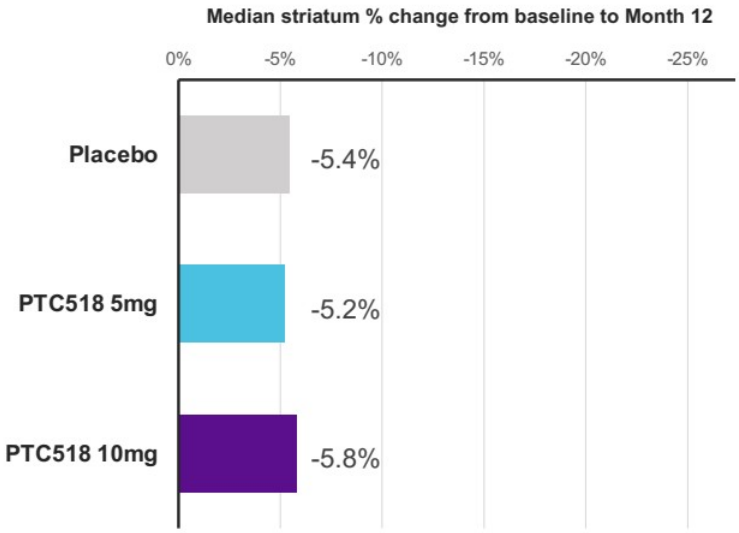

Plasma NfL increases ~10-12% per year based on natural history data

**Individual Subject CSF NfL Trajectories**

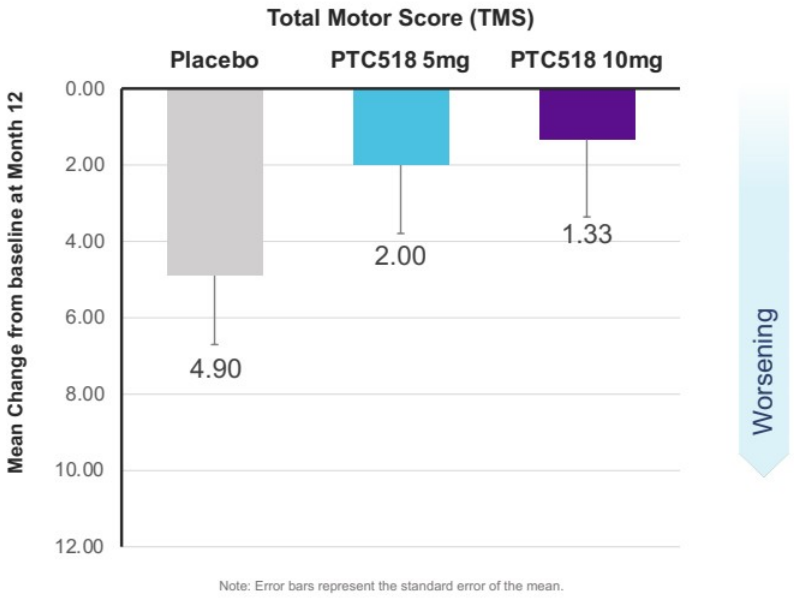



Note: One patient excluded due to non-treatment related viral synd

# Change in Striatum Brain Volume Consistent Across Treatment Groups

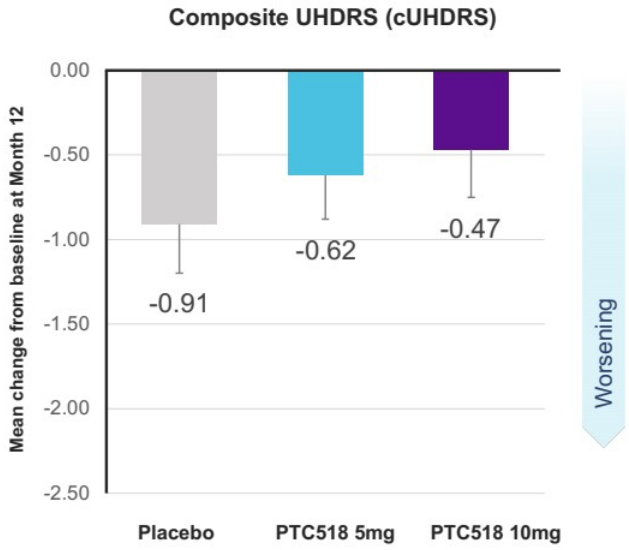


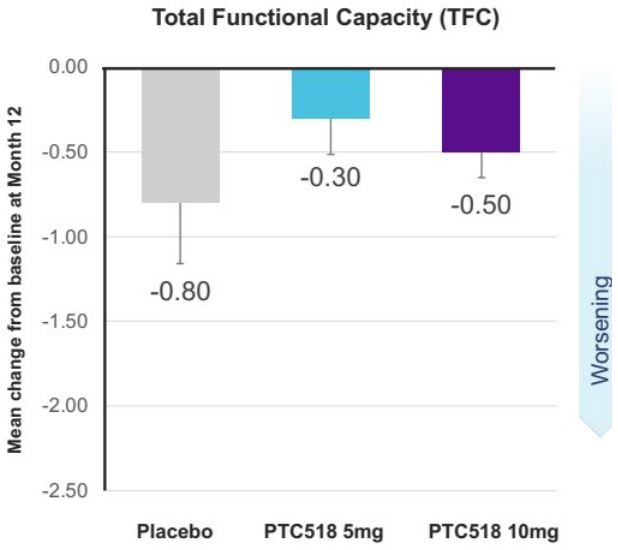
# PTC518 Treatment Demonstrated Trend of Dose-Dependent Improvement of Total Motor Score




Note: Error bars represent the standard error of the mean.

# PTC518 Treatment Demonstrated Trends of Improvement on Other Key Clinical Measures




Note: Error bars represent the standard error of the mean.

# Safety

# PTC518 Treatment Continues to be Well Tolerated At Month 12

PTC518 was well tolerated, with no dose-limiting toxicities

Most common adverse events were nasopharyngitis, influenza, headache, and falls

Similar adverse event profile across all treatment groups, including placebo

# PTC518 Treatment Continues to be Well Tolerated at Month 12

| Category | Placebo (N=10) | PTC518 5mg (N=12) | PTC518 10mg (N=10) |
|---|---|---|---|
| **Subjects with at least one TEAE** | 8 (80.0) | 9 (90.0) | 11 (91.7) |
| **Subjects with at least one serious TEAE** | 2 (20.0) | 0 | 1 (8.3) |
| **Subjects with at least one TEAEs leading to study treatment discontinuation** | 0 | 0 | 0 |
| **Subjects with at least one TEAE leading to death** | 0 | 0 | 0 |
| **Subjects with at least one treatment related AE#** | 1 (10.0) | 5 (50.0) | 6 (50.0) |
| **Subjects with at least one TEAEs by maximum severity N (%)** | 8 (80.0) | 9 (90.0) | 11 (91.7) |
| **Grade 1** | 5 (50.0) | 4 (40.0) | 3 (25.0) |
| **Grade 2** | 1 (10.0) | 4 (40.0) | 7 (58.3) |
| **Grade 3** | 2 (20.0) | 1 (10.0) | 1 (8.3) |
| **Grade 4/5** | 0 | 0 | 0 |



* Unrelated to drug
# Judged by the investigator to be probably or possibly related to treatment

# Evidence of Durability of Effect, Safety and Dose-Dependent Benefit on Clinical Measures

**Dose-dependent and durable lowering of HTT protein** in blood at 12 months

**Dose-dependent lowering of CSF mHTT levels**

**Dose-dependent trends of improvement on key clinical measures** including TMS and cUHDRS

**PTC518 was well tolerated with no evidence of treatment-related NfL spikes** at 12 mont

**FDA Partial Clinical Hold Lifted**



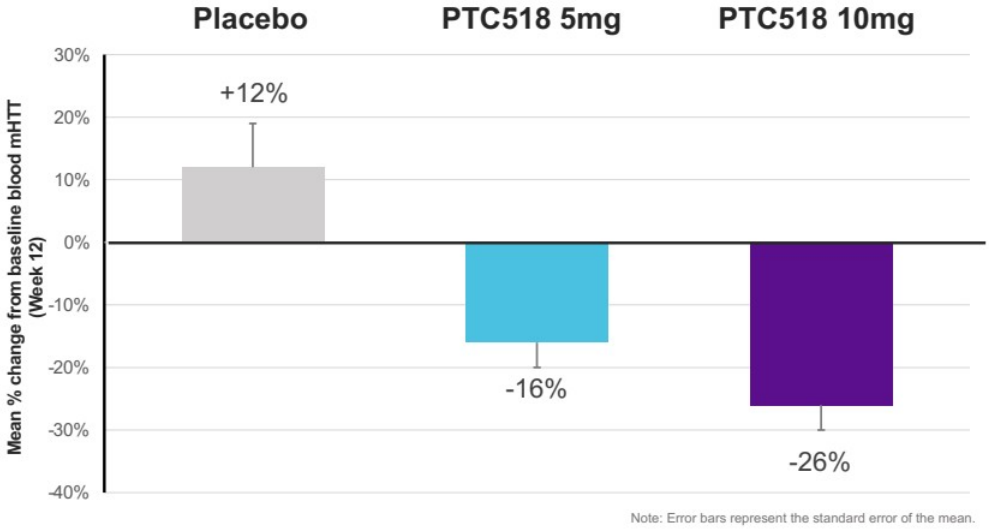
Note: Results based on interim readout

# Additional Week 12 Data

# Patient Characteristics of Additional Week 12 Subjects

| Category | Placebo (Stage 2 N=18) (Stage 3 N=11) | PTC518 5mg (Stage 2 N=13) (Stage 3 N=13) | PTC518 10mg (Stage 2 N=14) (Stage 3 N=12) | Overall (Stage 2 N=45) (Stage 3 N=36) |
|---|---|---|---|---|
| **Age (years) mean** | 48.6 | 48.7 | 47.0 | 48.1 |
| **Gender, n (%)** | | | | |
| **Male** | 20 (69.0%) | 12 (46.2%) | 14 (53.8%) | 46 (56.8%) |
| **Female** | 9 (31.0%) | 14 (53.8%) | 12 (46.2%) | 35 (43.2%) |
| **CAG length** | | | | |
| **Mean (SD)** | 43.7 (2.46) | 43.9 (2.76) | 44.4 (2.80) | 44.0 (2.65) |
| **Min – Max** | 40 - 49 | 40 – 50 | 41 – 50 | 40 – 50 |
| **TFC (Total Functional Capacity) Score** | | | | |
| **Mean** | 12.5 | 12.4 | 12.4 | 12.4 |

# PTC518 Treatment Resulted in Dose-Dependent Blood mHTT Protein Lowering at Week 12




Note: Error bars represent the standard error of the mean.

Dose-dependent lowering of HTT protein



Note: Results include all subjects for whom biomarker data are available

# PTC518 Safety Profile Consistent for Additional Week 12 Subjects

PTC518 was well tolerated, with no dose-limiting toxicities

Most common adverse events were nasopharyngitis, influenza, headache, and falls

Similar adverse event profile across all treatment groups, including placebo

# Summary and Next Steps

Evidence of favorable CNS activity with trends of improvement on key clinical measures and continued safety

Continue PIVOT-HD and PIVOT-HD Open Label Extension studies

Begin preparations for Phase 3 clinical trial