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

June 20, 2024

- 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 /PRNewswire/ -- 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:

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