



## PTC Therapeutics Shares Positive Interim Data from PIVOT-HD Clinical Trial in Huntington's Disease Patients

June 21, 2023

*- Dose-dependent lowering of blood Huntingtin (HTT) protein levels at 12 weeks -  
- Favorable tolerability profile with no treatment-related serious adverse events or NfL spikes -*

*- Conference call and webcast to be held June 21<sup>st</sup> at 8:00 am EDT -*

SOUTH PLAINFIELD, N.J., June 21, 2023 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today shared interim data from the 12-week portion of the PIVOT-HD Phase 2 study of PTC518 in Huntington's disease (HD) patients. The study demonstrated dose-dependent lowering of Huntingtin (HTT) protein levels in peripheral blood cells, reaching a mean 30% reduction in mutant HTT levels at the 10mg dose level. In addition, PTC518 exposure in the cerebrospinal fluid (CSF) was consistent with or higher than plasma unbound drug levels.

PTC518 treatment was also demonstrated to be well tolerated, with no treatment-related serious adverse events, no reports of peripheral neuropathy or dose-limiting toxicities. Furthermore, there were no CSF neurofilament light chain protein (NfL) treatment-related spikes, with an overall trend towards lowering of CSF NfL levels following 12 weeks of PTC518 treatment.

"We are very pleased with the encouraging data from the PIVOT-HD interim analysis demonstrating dose-dependent HTT lowering, desired CSF exposure and a favorable tolerability profile without evidence of treatment-related serious adverse events or CSF NfL spikes," said Dr. Matthew Klein, Chief Executive Officer, PTC Therapeutics, Inc.

PIVOT-HD is a global, placebo-controlled study and consists of two parts: an initial 12-week placebo-controlled phase focused on PTC518 pharmacology and pharmacodynamic effect, followed by a 9-month placebo-controlled portion. The study will initially include two dose levels, 5 milligrams and 10 milligrams, with the ability to include a third dose level of up to 20 milligrams.

### 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.<sup>1</sup> 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.<sup>2</sup> 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.<sup>2,3</sup> There are also cases of infantile HD, when symptoms develop in children who are younger than 10 years old.<sup>2</sup> 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.<sup>4,5</sup> 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](http://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.

### References:

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