



## **PTC Therapeutics to Host Conference Call to Discuss Results of PTC518 Phase 1 Study for Huntington's Disease Program**

September 20, 2021

SOUTH PLAINFIELD, N.J., Sept. 20, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will host a conference call to discuss the positive results from the PTC518 Phase 1 healthy volunteer study. The new results include HTT mRNA and protein lowering, as well as PTC518 exposure levels in the cerebrospinal fluid, consistent with the compound penetrating the blood-brain barrier and not being effluxed out of the brain.

The conference call will be held on Thursday, Sept. 23 at 8:00 a.m. ET. During this call, the company will also provide an update on the PTC518 Phase 2 study, to be conducted in Huntington's disease patients, which PTC expects to initiate by the end of the year.

Huntington's disease is a progressive brain disorder that causes uncontrolled movements and cognitive loss. There are currently no approved disease modifying therapies for this debilitating disease.

The call can be accessed by dialing (877) 303-9216 (within the U.S.) or (973) 935-8152 (outside of the U.S.) five minutes prior to the start of the webinar and providing the passcode 2587770. A live, listen-only audiocast will be available on the Events and Presentations page of the Investor section of PTC Therapeutics' website at [www.ptcbio.com](http://www.ptcbio.com). A replay will be available approximately two hours after completion of the call and will be archived for 30 days.

### **About Huntington's Disease**

Huntington's disease (HD) is a rare, inherited disease that causes the progressive degeneration of nerve cells in the brain, impacting a person's functional abilities. While HD can present at any age, it is most prevalent in people aged 30 to 50, and it affects approximately 45,000 people in the United States. HD is caused by a mutation in the huntingtin gene, which is responsible for creating huntingtin protein (HTT). As time progresses, the mutated huntingtin protein forms clumps in the brain cells, resulting in damaged cells and eventually cell death.

### **About PTC518**

PTC518 is a small molecule splicing modifier that acts via a unique mechanism to promote the inclusion of a novel pseudoexon containing a premature termination codon, thus triggering HTT mRNA degradation and subsequent reduction in HTT protein levels. In preclinical studies using cells isolated from patients with HD, PTC518 reduced both huntingtin mRNA and protein levels with high potency in a dose-dependent manner. In addition, oral administration of PTC518 in the BACHD mouse model of HD achieved dose dependent and equitable lowering of HTT protein throughout the body, including the brain, muscle, and blood.

### **About PTC**

PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need. The company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. To learn more about PTC, please visit us at [www.ptcbio.com](http://www.ptcbio.com) and follow us on Instagram, Facebook, Twitter, and LinkedIn.

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## Forward-Looking Statements

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements contained in this release, other than statements of historic fact, are forward-looking statements, including 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 and other matters; 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: 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; the enrollment, conduct, and results of PTC518 clinical studies for HD; 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; 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.

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