PTC Therapeutics to Host PTC518 Huntington Disease Deep Dive Webinar

April 7, 2021

SOUTH PLAINFIELD, N.J., April 7, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will host a webinar in its deep dive series discussing the PTC518 Huntington disease (HD) program on Thursday, April 15 at 9 a.m. E.T.

The Huntington Disease Deep Dive will provide an overview of PTC's splicing platform and the identification of molecules that have selectivity and specificity. PTC will highlight its Huntington program and the potential advantages of an orally bioavailable compound. PTC will discuss in detail the splicing modifier, PTC518, that is in an ongoing Phase 1 clinical trial. In preclinical studies, PTC518 has demonstrated broad biodistribution that uniformly reaches all regions of the brain and periphery and has shown reduction in Huntingtin protein (HTT) levels with a 1:1 correlation between blood and brain. PTC will also present preliminary results from the Phase 1 study of PTC518.

The webinar can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the webinar and providing the passcode 5190575. A live, listen-only webcast can be accessed on the Events and Presentations page under the investor relations section of PTC Therapeutics' website at www.ptcbio.com. A webcast replay will be available approximately two hours after completion of the webinar and will be archived for 30 days following the webinar.

About Huntington Disease
Huntington disease (HD) is a rare, inherited disease that causes the progressive degeneration of nerve cells in the brain, impacting a person's functional abilities.[1] While HD can present at any age, it is most prevalent in people aged 30 – 50, and it affects approximately 45,000 people in the United States.[2,3] HD is caused by a mutation in the huntingtin gene, which is responsible for creating huntingtin protein (HTT).[3] HTT protein is large and seems to have many functions, especially as the brain develops before birth, but it is not completely understood.[3] As time progresses, the mutated huntingtin protein forms clumps in the brain cells, resulting in damaged cells and eventually cell death.[3]

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 Huntington disease (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 and follow us on Facebook, on Twitter at @PTCBio, and on 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, 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 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.

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.

[1] Huntington's disease. (2020, April 14). Retrieved April 01, 2021, from https://www.mayoclinic.org/diseases-conditions/huntingtons-disease/symptoms-causes/syc-20356117

[2] Fisher ER, Hayden MR. Multisource ascertainment of Huntington disease in Canada: prevalence and population at risk. Mov Disord. 2014;29(1):105-114.

[3] Overview of Huntington's disease. (n.d.). Retrieved April 01, 2021, from https://hdsa.org/what-is-hd/overview-of-huntingtons-disease/#:~:text=Huntington's%20disease%20(HD)%20is%20a,years%20and%20has%20no%20cure

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