PTC Therapeutics Announces that PTC518 Has Entered into a Phase 1 Clinical Trial for the Huntington's Disease Program

November 17, 2020

- Initial clinical trial results expected in 1H 2021 -

SOUTH PLAINFIELD, N.J., Nov. 17, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the initiation of a Phase 1 clinical trial to evaluate PTC518 in healthy volunteers. PTC518 was identified from PTC's splicing platform and is being developed for the treatment of Huntington's disease (HD). PTC518 is an orally bioavailable molecule with broad central nervous system and systemic distribution and has been designed to target Huntingtin protein expression with high selectivity and specificity. PTC518 has favorable pharmaceutical properties and has demonstrated uniform lowering of the Huntingtin protein throughout the brain in animal models. Currently there are no approved disease modifying therapies for HD.

"The initiation of the clinical trial to evaluate PTC518 for the Huntington's disease program is an important milestone towards identifying a potential new Huntington's disease treatment that directly targets the underlying cause of the disease," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "PTC518 is the only orally bioavailable small molecule therapeutic that was identified to selectively and specifically modulate Huntington's disease splicing to reduce huntingtin protein. Analogous to the SMA drug from our splicing platform, this trial is anticipated to establish the appropriate PTC518 dose that reduces HTT protein levels. We believe that the convenient oral administration of PTC518 has the potential to change the treatment landscape for these patients."

The Phase 1 study includes both single and multiple ascending dosing regimens that will help establish the safety, pharmacology, and dose selection for the Phase 2 study. In addition, huntingtin mRNA and protein levels will be measured to gain early proof of splicing mechanism in humans as was done in the risdiplam SMA development program. Data from the Phase 1 study are expected to be available in the first half of 2021.

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's 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 Therapeutics, Inc.

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. 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, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; PTC's strategy, future operations, future financial position, future revenues and 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; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and 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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