# PTC Therapeutics Announces Initiation of PIVOT-HD Phase 2 Clinical Trial to Evaluate PTC518 in Patients with Huntington's Disease

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SOUTH PLAINFIELD, N.J., March 30, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the initiation of the PIVOT-HD Phase 2 clinical trial evaluating PTC518 in people with Huntington's disease (HD). PIVOT-HD is a global trial starting in the United States. PTC518 is an oral, small molecule splicing modifier that was specifically designed to selectively lower huntingtin mRNA and protein. There are no current treatments for the underlying cause of HD.

"We are excited to advance our Huntington's disease program," said Stuart W. Peltz, CEO, PTC Therapeutics. "In the PIVOT-HD trial, we aim to confirm the dose-dependent lowering of huntingtin protein that was demonstrated in our Phase 1 clinical study and gain insight to biomarker data that could provide meaningful evidence of treatment effect."

The PIVOT-HD Phase 2 clinical trial is designed in two parts: an initial 12-week placebo-controlled phase focused on PTC518 pharmacology and pharmacodynamic effect, followed by a 9-month placebo-controlled phase focused on PTC518 biomarker effect.

**About Huntington's Disease**

Huntington's disease (HD) is a rare, inherited disease that causes the progressive degeneration of nerve cells in the brain that has broad impact on a person's motor and functional capabilities, resulting in both movement disorders and cognitive loss. 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. HD has broad impact on a person's motor and functional capabilities that results in both movement disorders cognitive loss. There are no treatments for the underlying cause of HD.

**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 a Phase 1 healthy volunteer study of PTC518 for Huntington's disease, PTC518 demonstrated a dose-dependent lowering of HTT mRNA and protein to the targeted 30-50% reduction. In addition, PTC518 showed that it passes the blood brain barrier and has minimal efflux which is a key factor in the targeting the underlying cause of Huntington's disease.

**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 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 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, regulatory submissions and responses 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 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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