



PTC Provides Update on Ongoing Global PIVOT-HD Trial For PTC518

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SOUTH PLAINFIELD, N.J., Oct. 18, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) confirmed today that enrollment is active and ongoing for its Phase 2 PIVOT-HD trial of PTC518 for the treatment of Huntington's disease at study sites globally. To date, PTC has received approval for conduct of the planned 12-month study in several European countries as well as Australia.

The Phase 2 PIVOT-HD study consists of two parts; an initial 12-week placebo-controlled part focused on PTC518 pharmacology and pharmacodynamic effect, followed by a 9-month placebo-controlled part, during which blood, CSF, and radiographic biomarker data will be collected.

The study will initially include two dose levels, 5 milligrams and 10 milligrams, with the potential to study a third dose leveraging the titratability of the drug. The approvals received to date for the 12-month study include both the 5mg and 10mg dose levels, as well as a potential third dose pending the results of the first two dosing cohorts.

Enrollment in the 12-week portion of the study began first in the United States and continues globally. To date there have been no treatment-related adverse events reported in the US or outside of the US. However, the FDA has requested additional data to allow the study to proceed in the US. Accordingly, enrollment in the US has been paused while discussions with the FDA continue towards resuming US enrollment.

PTC is working towards sharing data from the 12-week portion of PIVOT-HD within the first half of 2023.

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 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 release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future enrollment, study design, expected timing, availability of data, regulatory submissions and responses and other matters related to PTC's Phase 2 trial of PTC518 for the treatment of Huntington's disease; 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 enrollment, conduct and results of studies of PTC518 for Huntington's disease and events during, or as a result of, the studies that could delay or prevent further development under the program, including with respect to regulatory submissions; 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 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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