PTC Therapeutics Announces Topline Results of CardinALS Trial of Utreloxastat in ALS Patients

November 26, 2024

- Study failed to meet primary and secondary efficacy endpoints -

WARREN, N.J., Nov. 26, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the global Phase 2 placebo-controlled CardinALS study did not meet its primary endpoint of slowing disease progression on the composite ALSFRS-R and mortality analysis. While there was modest numerical benefit recorded on the primary endpoint and correlation of favorable clinical effect with lowering of plasma neurofilament light chain (NfL), a biomarker of neuronal damage, statistical significance was not achieved (p= 0.52). In addition, significance was not achieved on the secondary efficacy endpoints.

"We wish to thank all of the patients, their families and physicians who participated in the CardinALS trial," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "We are of course disappointed that we were not able to demonstrate treatment efficacy and provide a potential therapy that could address the significant unmet medical need of ALS patients."

Utreloxastat was demonstrated to be safe and well tolerated in the CardinALS trial. However, due to the lack of efficacy and biomarker signal, further development is not planned at this time.

About Amyotrophic Lateral Sclerosis (ALS)
Amyotrophic lateral sclerosis, sometimes referred to as ALS, motor neuron disease, or Lou Gehrig's disease, is a rare, progressive, and fatal neurodegenerative disease that affects motor neurons in the brain and spinal cord.[1,2] ALS becomes life-threatening as people with this disease ultimately lose the ability to move, speak, eat, and breathe.[1]

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to children and adults living 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 Facebook, Instagram, LinkedIn and X.

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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 press release, other than statements of historic fact, are forward-looking statements, including statements with respect to the future expectations, plans and prospects for PTC, including with respect to 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; 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.

References:

[1] Kiernan MC, et al. Amyotrophic lateral sclerosis. The Lancet, 2011; 377(9769):942-955.

[2] Dhasmana S, et al. The panoramic view of amyotrophic lateral sclerosis: A fatal intricate neurological disorder, Life Sciences 2022;288:120156. https://doi.org/10.1016/j.lfs.2021.120156.

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