FDA Grants Fast Track Designation to PTC518 Huntington's Disease Program

September 26, 2024

WARREN, N.J., Sept. 26, 2024 /PRNewswire/ -- PTC Therapeutics (NASDAQ: PTCT) announced today that the FDA has granted Fast Track designation to the PTC518 program for the treatment of Huntington's disease. Fast Track designation is awarded to promising therapies for diseases of high unmet need.

"The granting of Fast Track designation to the PTC518 program further supports the potential of PTC518 to provide a disease modifying therapy to Huntington's disease patients," said Matthew B. Klein, M.D., Chief Executive Officer of PTC Therapeutics. "We look forward to working collaboratively with FDA to continue to advance the program as efficiently as possible."

PTC518 was discovered using PTC's innovative splicing platform, following the successful discovery and development of Evrysdi® (risdiplam) for the treatment of spinal muscular atrophy.

In June 2024, PTC announced positive results from the 12-month interim data readout of the PIVOT-HD study of PTC518 Huntington's disease patients. The 12-month data demonstrated durable dose-dependent lowering of mutant Huntingtin protein in blood cells, reaching 43% at the 10-milligram dose level along with dose-dependent lowering of mutant Huntingtin protein in the CSF at levels similar to the blood in Phase 2 patients. In addition, at 12 months, there was dose-dependent favorable clinical effect on key disease measurements, including the total motor score and the cUHDRS scale. Importantly, the interim results also demonstrated PTC518 to be safe and well tolerated with no treatment-related NfL spikes.

About Fast Track Designation:
Fast Track is a process designed by the FDA to facilitate the development and expedite the review of investigational treatments that demonstrate a potential to address unmet medical needs in serious or life-threatening conditions. Programs with Fast Track designation can benefit from early and more frequent interactions with the FDA to discuss the product candidate's development plan in addition to a rolling submission of the marketing application. Product candidates with Fast Track designation may also be eligible for priority review and accelerated approval.

About PTC518
PTC518, a small molecule that can be taken orally, reduces the production of the mutated Huntingtin protein that leads to injury and death of the neuron, which results in disease progression. The orally bioavailable small molecule penetrates the blood brain barrier, is selective, titratable, and not effluxed.

About Huntington's Disease
Huntington's disease (HD) is a fatal, hereditary, genetic disorder of the central nervous system.[1] It is caused by a defective gene. This gene produces a protein, called Huntingtin, which is involved in the functioning of the nerve cells in the brain (neurons). When the gene is defective, it produces an abnormal (or mutated) Huntingtin protein that is toxic and causes neuron damage and neuron death.[2] HD usually presents in people who are in their 30s or 40s. Symptoms can present earlier in life, and this is called the Juvenile HD.[2,3] There are also cases of infantile HD, when symptoms develop in children who are younger than 10 years old.[2] While symptoms vary from person to person, the disease primarily affects the brain and results in abnormal movements, difficulties with speech, swallowing and walking, as well as a number of other symptoms including behavioral, cognitive and motor symptoms.[4,5] While there are therapies approved for specific disease symptoms, currently, there is no cure for HD and there are no approved drugs that delay the onset or slow disease progression.

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 X, Facebook, Instagram and LinkedIn.

For More Information:

Investors:
Investor Relations
+1 (908) 912-9848
ir@ptcbio.com

Media:
Jeanine Clemente
+1 (908) 912-9406
jclemente@ptcbio.com

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, PTC's strategy, including with respect to the expected timing of clinical trials and studies, availability of

data, regulatory submissions and responses and other matters, 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; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; 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:

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