PTC Therapeutics to Report Results from PIVOT-HD Long Term Extension Study

April 28, 2026

WARREN, N.J., April 28, 2026 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) will host a webcast conference call today, April 28 at 4:30 p.m. ET to share results from the 24-month interim analysis of the PIVOT-HD long-term extension study of votoplam. PTC's partner Novartis stated on its quarterly earnings call today that long-term extension data are supportive of the now-initiated Novartis Phase 3 INVEST-HD study and Novartis and PTC will assess potential best next steps for the program including further actions with FDA.

To access the live webcast, please visit the "Events & Presentations" page within the Investors section of the PTC website. A replay of the webcast will be available on the PTC website for 30 days following the event. To participate via phone, please register in advance here to receive dial-in details.

About PIVOT-HD
PIVOT-HD was designed as a 12-month placebo-controlled trial to assess pharmacodynamic effect and safety of votoplam at two dose levels--5mg and 10mg, relative to placebo. Initially, the study included only Stage 2 patients. A Stage 3 cohort of similar size was subsequently added to help identify the best study population for future studies. The primary endpoints of PIVOT-HD were total blood Huntingtin (HTT) lowering at 12 weeks and safety events. Secondary endpoints included 12-month blood HTT levels, and other blood-and central nervous system (CNS) biomarkers as well as changes in Composite Unified Huntington's Disease Rating Scale (cUHDRS).

Following 12 months, patients were eligible to enroll in a long-term extension study in which all subjects would receive votoplam. Those originally randomized to 5mg and 10mg would continue at that dose level; those initially randomized to placebo would be randomized 1:1 to 5mg or 10mg. All subjects and investigators remain blinded to initial treatment assignment.

About Votoplam
Votoplam (formerly 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 Huntingtin (HTT) mRNA degradation and subsequent reduction in HTT protein levels. Votoplam was discovered from PTC's validated splicing platform, following the successful discovery and development of Evrysdi® (risdiplam) for spinal muscular atrophy (SMA). Votoplam was partnered with Novartis in December 2024. Following the completion of the PIVOT-HD clinical trial, Novartis assumed responsibility for votoplam's development, manufacturing and commercialization.

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 (HTT), 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) HTT 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 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 dedicated to the discovery, development and commercialization of clinically differentiated medicines for children and adults living with rare disorders. PTC is advancing a robust and diversified pipeline of transformative medicines as part of its mission to provide access to best-in-class treatments for patients with unmet medical needs. The company's strategy is to leverage its scientific expertise and global commercial infrastructure to optimize value for patients and other stakeholders. To learn more about PTC, please visit www.ptcbio.com and follow us on LinkedIn, X, Instagram and Facebook.

For more information, please contact:

Investors:
Ellen Cavaleri
+1 (615) 618-8228
ecavaleri@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; expectations with respect to PTC's license and collaboration agreement with Novartis Pharmaceuticals Corporation for votoplam for the treatment of Huntington's disease including its right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis, the design and expected timing of clinical trials and studies, the availability of data, and regulatory submissions and responses, including potential accelerated approval; 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:

1. World Health Organization, 2020. 8A01.10 Huntington disease. Available at: https://icd.who.int/browse10/2019/en#/G10. Accessed October 2021.
2. Gatto EM, González Rojas N, Persi G, et al. Clin Parkinsonism Rel Disord 2020;3:100056.
3. Tabrizi SJ, Flower MD, Ross CA, et al. Nat Rev Neurol 2020;16(10):529–546.
4. Roos RAC. Orphanet J Rare Dis 2010; 5:40.
5. Kirkwood SC, Su JL, Conneally P, et al. Arch Neurol 2001;58(2):273–278.

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