PTC Therapeutics Enters into a Global License and Collaboration Agreement with Novartis for PTC518 Huntington's Disease Program

December 2, 2024

- PTC to receive $1.0B in cash at closing -
- PTC is eligible to receive up to $1.9B in development, regulatory and sales milestones -
- PTC to share profits in the U.S. and tiered double-digit royalties on ex-U.S. net sales -
- Novartis will assume global development, manufacturing and commercial responsibilities following completion of placebo-controlled portion of ongoing PIVOT-HD study -
- PTC will host a conference call on Dec. 2, 2024, at 8:30 am EST-

WARREN, N.J., Dec. 2, 2024 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) announced today the signing of an exclusive global license and collaboration agreement with Novartis Pharmaceuticals Corporation, a subsidiary of Novartis AG (NYSE: NVS), for its PTC518 Huntington's disease program, which includes related molecules. Under the agreement, PTC will receive an upfront payment of $1.0 billion, up to $1.9 billion in development, regulatory and sales milestones, a profit share in the U.S., and double-digit tiered royalties on ex-U.S. sales.

"PTC518 is the leading oral disease-modifying therapy in development for Huntington's disease and the economics of this agreement are consistent with the promise of this treatment," said Matthew B. Klein, M.D., Chief Executive Officer, PTC Therapeutics. "This collaboration combines PTC's expertise in developing small molecule splicing therapies with Novartis's expertise in global development and commercialization of neuroscience therapies. We are excited to collaborate with Novartis to accelerate the potential of PTC518 for the hundreds of thousands of HD patients worldwide in need of a therapy designed to be well-tolerated and an effective disease-modifying therapy. PTC will use the proceeds of this transaction to expand our splicing platform as well as to support commercial and development portfolio activities."

"Huntington's Disease is a devastating, fatal, familial disease. This agreement with PTC is intended to bolster our neuroscience pipeline and reflects our strategic focus and commitment to explore new and potentially transformative approaches for neurodegenerative diseases with high unmet needs," said Vas Narasimhan, CEO of Novartis. "We look forward to building on our expertise in neurodegenerative diseases and experience in HD with the intention to advance this potential first in class oral therapy for the HD community."

PTC518 was discovered from PTC's validated splicing platform and is currently being studied in the ongoing Phase 2 PIVOT-HD trial. Interim results reported in June 2024 demonstrated that PTC518 treatment resulted in durable, dose-dependent reduction in blood and cerebrospinal fluid (CSF) mutant Huntingtin protein (HTT) levels as well as early signals of dose-dependent benefit on key clinical measurements at 12 months.[1] Importantly, PTC518 continues to demonstrate a favorable safety and tolerability profile.[1]

Novartis will assume responsibility for PTC518's development, manufacturing and commercialization, following the completion of the on-going placebo-controlled portion of PIVOT-HD, which is expected to occur in H1 2025.

The companies will share U.S. profits and losses, on a 40/60 basis (40% PTC and 60% Novartis).

The closing of the transaction is subject to customary closing conditions, including regulatory clearance. The parties anticipate that the agreement will close in the first quarter of 2025.

Conference Call and Webcast Details:
PTC will hold a conference call at 8:30 am EST today to discuss this news. To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About Huntington's Disease
Huntington's disease (HD) is a fatal, hereditary, genetic disorder of the central nervous system.[2] 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.[3] 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.[3,4] There are also cases of infantile HD, when symptoms develop in children who are younger than 10 years old.[3] 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.[5,6] 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's Splicing Platform
PTC has pioneered the use of advanced alternative splicing technology to identify small molecules that affect mRNA splicing for the treatment of disease of high unmet need. PTC's validated splicing platform identified the first-ever approved small molecule splicing modifier - Evrysdi® (risdiplam), and PTC has leveraged the extensive learnings from the SMA program to broaden the platform to support discovery programs across numerous therapeutic areas including neurodegenerative disease, oncology and metabolism. PTC has also developed a powerful high-throughput drug discovery platform (PTSeek ™) that identifies small molecules that modulatepre-mRNA splicing to upregulate or down regulate targeted protein production, accelerating the discovery and early preclinical development process for candidate small molecule splicing agents.

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.

For More Information:

Investors:
Ellen Cavaleri
+1 (615) 618-6228
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, including with respect to PTC's right to receive any upfront payment from Novartis; PTC's right to receive development, regulatory and sales milestones, profit sharing and royalty payments from Novartis; the continued development of PTC518; 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; the expected benefits and opportunities related to the licensing agreement may not be realized or may take longer to realize than expected due to a variety of reasons, including any inability of the parties to perform their commitments and obligations under the agreement, challenges and uncertainties inherent in development; success in early clinical trials, especially if based on a small patient sample, does not ensure that later clinical trials will be successful, and early results from a clinical trial do not necessarily predict final results; data for PTC518 may not be sufficient for obtaining regulatory 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; 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. *PTC Therapeutics, "Interim PIVOT-HD Results Demonstrate Evidence of Favorable CNS Biomarker and Clinical Effects at Month 12 in Huntington's Disease Patients," news release, June 20, 2024, https://ir.ptcbio.com/news-releases/news-release-details/interim-pivot-hd-results-demonstrate-evidence-favorable-cns*
2. World Health Organization, 2020. 8A01.10 Huntington disease. Available at: https://icd.who.int/browse11/l-m/en#/http://id.who.int/icd/entity/2132180242 Accessed October 2021.
3. Gatto EM, González Rojas N, Persi G, et al. Clin Parkinsonism Rel Disord 2020;3:100056.
4. Tabrizi SJ, Flower MD, Ross CA, et al. Nat Rev Neurol 2020;16(10):529–546.
5. Roos RAC. Orphanet J Rare Dis 2010;5:40.
6. Kirkwood SC, Su JL, Conneally P, et al. Arch Neurol 2001;58(2):273–278.