PTC Therapeutics Announces Key Regulatory Designations for PTC596 to Advance Treatment of Two Rare Oncology Indications

November 18, 2020

SOUTH PLAINFIELD, N.J., Nov. 18, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that the United States (U.S.) Food and Drug Administration (FDA) has granted PTC596 both Orphan Drug Designation and Fast Track designation for the potential treatment of leiomyosarcoma (LMS), a rare type of cancer that affects smooth muscle tissue. Furthermore, the FDA has also granted PTC596 a Rare Pediatric Disease designation and Orphan Drug Designation for the potential treatment of Diffuse Intrinsic Pontine Glioma (DIPG), an ultra-rare childhood glioma. PTC596 is currently being studied in clinical trials in LMS and DIPG.

"We are very pleased with the FDA's decisions to grant PTC596 these designations," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics. "This brings us one step closer to providing truly novel therapeutic approaches to patients with devastating rare cancers seen in children and adults that do not have good treatment options."

PTC596 is an orally bioavailable small molecule tubulin binding agent that arrests tumor cells in G2/M phase, including cancer stem cells, through the action of inhibiting tubulin polymerization. It is currently in a Phase 1b study for LMS, which accounts for approximately 10 to 28% of all soft tissue sarcomas.[1] Approximately 4000 patients are diagnosed with LMS annually in the US, the risk of developing metastases is approximately 40%, and the 5-year survival rate is estimated to be 13.6% for metastatic LMS.[2] PTC596 is also currently in a clinical study for DIPG, an ultra-rare glioma arising in the brainstem that makes up 10 to 15% of all brain tumors in children.[3] Approximately 300 patients are diagnosed with DIPG annually in the US, and the median overall survival with the current standard of care of radiation therapy, is approximately 9 months with a two-year overall survival rate of less than 10%.[4]

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 globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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Forward-Looking Statements:
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 expectations, plans and prospects for PTC; PTC's strategy, future operations, future financial position, future revenues and 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 Quarterly Report on Form 10-Q and 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.

[1] Bessen, T., Caughey, G. E., Shakib, S., Potter, J. A., Reid, J., Farshid, G., Roder, D., & Neuhaus, S. J. (2019). A population-based study of soft tissue sarcoma incidence and survival in Australia: An analysis of 26,970 cases. *Cancer epidemiology*, *63*, 101590. https://doi.org/10.1016/j.canep.2019.101590

[2] Ries LAG, Young JL, Keel GE, Eisner MP, Lin YD, Horner M-J (editors). SEER Survival Monograph: Cancer Survival Among Adults: U.S. SEER Program, 1988-2001, Patient and Tumor Characteristics. National Cancer Institute, SEER Program, NIH Pub. No. 07-6215, Bethesda, MD, 2007.

[3] Raabe, E. H., & Cohen, K. (n.d.). What is DIPG? Retrieved November 9, 2020, from https://www.dipg.org/dipg-facts/what-is-dipg/

[4] Jansen, M. H., Veldhuijzen van Zanten, S. E., Sanchez Aliaga, E., Heymans, M. W., Warmuth-Metz, M., Hargrave, D., van der Hoeven, E. J., Gidding, C. E., de Bont, E. S., Eshghi, O. S., Reddingius, R., Peeters, C. M., Schouten-van Meeteren, A. Y., Gooskens, R. H., Granzen, B., Paardekooper, G. M., Janssens, G. O., Noske, D. P., Barkhof, F., Kramm, C. M., … van Vuurden, D. G. (2015). Survival prediction model of children with diffuse intrinsic pontine glioma based on clinical and radiological criteria. *Neuro-oncology*, *17*(1), 160–166. https://doi.org/10.1093/neuonc/nou104

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