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PTC Therapeutics' Cancer Stem Cell Program Targeting BMI1 Enters Phase 1

SOUTH PLAINFIELD, N.J., April 28, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT), today announced that its oncology program targeting BMI1, a protein linked to drug-resistant cancers, has entered a Phase 1 study in patients with advanced solid tumors. The open-label, first-in-human study will investigate the safety and pharmacokinetics of PTC596, an orally available small molecule. PTC's BMI1 program is supported by a collaboration with the Wellcome Trust.

BMI1 has been implicated in a wide variety of cancers and has been demonstrated to contribute to therapeutic resistance and treatment failure. BMI1 is thought to play a role in the survival and maintenance of tumor stem cells in many cancers, including central nervous system cancers such as glioblastoma. Elevated levels of BMI1 have been associated with advanced tumor grade and a poor prognosis.

"We're excited to begin clinical trials for PTC596, an investigational drug that targets an important stem cell regulator, BMI1, which is elevated in a wide array of tumor types," said Robert Spiegel, M.D., Chief Medical Officer, PTC Therapeutics, Inc. "In preclinical models, PTC596 reduced BMI levels leading to depletion of the cancer stem cell population. Importantly, we saw this effect when PTC596 was used alone and in combination with current standards of care."

"Targeting cancer stem cells by BMI1 inhibition is a promising approach to address the challenge of drug-resistant cancers," stated Lillian Siu, M.D., Princess Margaret Cancer Center, Professor of Oncology, University of Toronto. "Cancer is a complex problem and the development of treatments that focus on molecular targets shows promise for the next generation of cancer therapies to make a difference in patients' lives."

PTC's collaboration with the Wellcome Trust began in June 2010 when the Wellcome Trust awarded PTC $5.4 million to support the development of drugs that target BMI1.

About PTC Therapeutics, Inc.
PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website www.ptcbio.com.

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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, other than those of historical fact, contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the timing and conduct of PTC's clinical programs, including statements regarding enrollment and completion of studies and trials, the period during which results will become available, clinical utility, and our ability to advance such programs; PTC's strategy, future operations, future financial position, future revenues or projected costs; and objectives of management. Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "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 initiation, conduct and availability of data from clinical trials; expectations for regulatory approvals; PTC's scientific approach and general development progress; the eligible patient base; PTC's ability to maintain the marketing authorization license of Translarna for the treatment of nonsense mutation DMD in the European Economic Area, which is conditioned upon completion of its Phase 3 confirmatory trial in nonsense mutation DMD and subject to annual review and renewal by the EMA following its reassessment of the risk-benefit balance of the authorization; and the factors discussed in the "Risk Factors" section of PTC's 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. 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 release except as required by law.

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