



March 2, 2011

PTC Therapeutics Announces Achievement of Milestone in BMI1 Collaboration with the Wellcome Trust

- PTC Receives \$2.2 Million Payment -

SOUTH PLAINFIELD, NJ – March 2, 2011 – PTC Therapeutics, Inc. (PTC) today announced that it has identified a chemical series of molecules that penetrates the blood-brain barrier in animal models and reduces levels of BMI1, a protein linked to drug-resistant cancers. The research marks a milestone in PTC's collaboration with the Wellcome Trust and triggers drawdown of a \$2.2 million tranche of their \$5.4 million Seeding Drug Discovery award.

The collaboration began in June 2010 when the Wellcome Trust awarded PTC \$5.4 million to support the development of drugs that target BMI1, a protein that has been linked to drug-resistant cancers. Using its proprietary screening technique known as GEMS™, PTC identified a lead chemical series that selectively reduces levels of BMI1. The goal of the program is to identify a drug candidate for the treatment of chemotherapy-resistant cancers.

"We are pleased to reach this first milestone in our collaboration with the Wellcome Trust on schedule," said Stuart W. Peltz, Ph.D., President and CEO of PTC Therapeutics. "We believe the therapeutic potential of a small-molecule inhibitor of BMI1 production is very significant. This milestone will support chemistry optimization efforts toward our next major goal of declaring a development candidate."

BMI1 has been implicated in a wide variety of cancers and has been demonstrated to contribute to therapeutic resistance and treatment failure. It acts by switching off regulatory pathways inside the cell that would normally stop cancer from developing. BMI1 is also 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. Since BMI1 is a factor necessary for tumor stem cell survival, reduction of this protein is likely to increase susceptibility of tumors to current chemotherapy and radiotherapy treatments. Elevated levels of BMI1 in cancers such as glioblastoma are correlated with advanced tumor grade and a poor prognosis.

Rick Davis, Ph.D., Business Development Manager at the Wellcome Trust, said: "This is a significant step forward towards our shared goal of developing a potent BMI1 suppressor for drug-resistant cancer."

ABOUT GEMS™

GEMS is PTC's novel and proprietary technology platform for the identification of small-molecules that modulate post-transcriptional control mechanisms. Compounds identified through the GEMS technology target processes that act through the regulatory regions of messenger RNA molecules. PTC has successfully employed the GEMS technology in drug discovery programs in oncology, infectious diseases, cardiovascular diseases and neuromuscular disorders. The most advanced compound identified through the GEMS technology is PTC299, a small-molecule inhibitor of VEGF expression currently in Phase 2 clinical trials for oncology.

ABOUT WELLCOME TRUST

The Wellcome Trust is a global charitable foundation dedicated to achieving extraordinary improvements in human and animal health. It supports the brightest minds in biomedical research and the medical humanities. The Trust's breadth of support includes public engagement, education and the application of research to improve health. It is independent of both political and commercial interests. www.wellcome.ac.uk

ABOUT PTC THERAPEUTICS, INC.

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare genetic disorders, oncology, and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and which are the basis for collaborations with leading biopharmaceutical companies such as Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's web site at www.ptcbio.com.

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