



June 7, 2010

PTC THERAPEUTICS AWARDED \$5.4 MILLION GRANT FROM THE WELLCOME TRUST

-Over \$100 Million in Cumulative Grant Funding Raised to Date-

SOUTH PLAINFIELD, NJ – June 7, 2010 – PTC Therapeutics, Inc. (PTC) today announced the receipt of a \$5.4 million Seeding Drug Discovery (SDD) Award from The Wellcome Trust to support the development of drugs that target Bmi-1, a protein that has been linked to drug resistant cancers.

Bmi-1 has been implicated in a wide variety of cancers and has been demonstrated to contribute to drug resistance and treatment failure. It acts by switching off regulatory pathways inside the cell that would normally stop cancer from developing. Bmi-1 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 Bmi-1 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 Bmi-1 in cancers such as glioblastoma correlate with advanced tumor grade and a poor prognosis.

"We are pleased to receive this grant from The Wellcome Trust because it supports our ongoing commitment to identifying novel treatments for serious and life-threatening diseases," stated Stuart W. Peltz, Ph.D., President and Chief Executive Officer at PTC Therapeutics. "Compounds that selectively reduce Bmi-1 levels have broad therapeutic potential in treatment of drug-resistant cancers, such as glioblastoma, a cancer where current therapies extend life by only months."

Using its proprietary novel screening technique known as GEMS™, PTC has identified a lead chemical series that selectively blocks production of the Bmi-1 protein. The program is currently in early lead optimization with the goal of identifying a drug candidate that could be taken orally for the treatment of chemotherapy-resistant cancers.

"PTC's elegant approach to tackling chemotherapy resistance takes us into a truly novel area of research," said Richard Davis, Ph.D., Business Development Manager of The Wellcome Trust. "A successful drug candidate would address a huge unmet medical need."

The Wellcome Trust launched the SDD funding initiative in 2005 to facilitate early-stage small-molecule drug discovery in areas of unmet medical need. After a comprehensive review of the initiative, the Trust has recently announced a five-year extension to the scheme with an injection of £110 million. The awards are highly competitive and applicants must satisfy an independent committee of experts in order to be considered for funding.

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 charity 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 is 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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