

PTC THERAPEUTICS ANNOUNCES ACHIEVEMENT OF MAJOR MILESTONE IN WELLCOME TRUST BMI1 COLLABORATION

- PTC Receives \$2.3 Million Payment -

SOUTH PLAINFIELD, NJ – July 11, 2012 – PTC Therapeutics, Inc. (PTC) today announced that it has selected an experimental drug candidate to advance in its BMI1 program for aggressive and drug-resistant tumors. The milestone triggers drawdown of the next \$2.3 million installment of its Seeding Drug Discovery Award from the Wellcome Trust.

The candidate, PTC596, is a first-in-class, oral, potent and selective inhibitor of BMI1 protein expression. BMI1 is a protein that plays a central role in the survival and maintenance of tumor stem cells. Tumor stem cells are resistant to current chemotherapy treatments and contribute to tumor recurrence. Elevated levels of BMI1 are associated with more aggressive tumors and a poor prognosis in a wide variety of cancers including glioblastoma. Reducing levels of BMI1 therefore represents a promising new therapeutic strategy to treat drug-resistant cancers.

PTC596 acts by altering the post-translational modification of the BMI1 protein, resulting in reduced levels of BMI1 protein and subsequently reduced action of epigenetic complexes dependent on BMI1. Inhibition of these important complexes leads to the reduced survival and proliferation of tumor stem cells. PTC596 has the potential to be used as a monotherapy or in combination with current standards of care in multiple tumor types.

PTC received a \$5.4 million Seeding Drug Discovery Award from the Wellcome Trust in June 2010. In March 2011, PTC announced that it had achieved its first milestone, the identification of a chemical series that penetrate the blood-brain barrier in animal models and reduce levels of BMI1.

"We are proud to reach this important milestone," stated Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "By targeting one of the underlying causes of tumor recurrence and drug resistance, PTC's oral BMI1 inhibitor has the potential to address aggressive or treatment-resistant tumors in patients. This significant milestone further demonstrates the effectiveness of PTC's technologies in identifying compounds against challenging but important therapeutic targets. Discovering compounds like PTC596 is consistent with our mission to develop novel treatments for serious and life-threatening diseases with high unmet need."

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 that have served as the basis for collaborations with leading biopharmaceutical companies such as AstraZeneca, Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's website at www.ptcbio.com.

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