



## **ASTRAZENECA AND PTC THERAPEUTICS ANNOUNCE STRATEGIC ONCOLOGY DRUG DISCOVERY COLLABORATION WITH POTENTIAL EXTENSION TO OTHER THERAPEUTIC AREAS**

**ALDERLEY PARK, UK and SOUTH PLAINFIELD, N.J., June 29, 2011** – AstraZeneca and PTC Therapeutics, Inc. (PTC) today announced that they have entered into an exclusive research collaboration and license agreement for the application of PTC's proprietary GEMS™ technology (Gene Expression Modulation by Small molecules) for the discovery and development of potential new therapies for cancer and other diseases for which there is a great unmet medical need.

Cancer kills more than 7 million people globally every year. The World Health Organization estimates the incidence of cancer will continue to rise to an estimated 11 million deaths by 2030, highlighting the critical need to discover novel and targeted therapies that will offer benefits to patients.

The collaboration between AstraZeneca and PTC is a step forward in drug discovery for novel therapies, considering that classical drug discovery approaches have proven ineffective for more than 90% of all disease targets. GEMS is a novel technology that enables the discovery of drugs that can increase or decrease the expression of a specific protein by targeting regulatory mechanisms. It is an alternative approach to drug discovery and among its advantages is the fact that GEMS delivers small molecules that can be taken orally. Under the terms of the agreement, AstraZeneca will make an undisclosed up-front cash payment for the initiation of the first target in the collaboration plus committed research funding to PTC. Initial efforts will be focused on oncology with an opportunity to pursue up to eight targets across different therapeutic areas.

PTC will also qualify for significant future milestone payments depending on the achievement of research, development, regulatory and commercial milestones. AstraZeneca retains the global commercialization rights and PTC has an option to participate in the development of select product candidates emerging from the collaboration. AstraZeneca will pay PTC tiered royalties on worldwide net sales.

"We are delighted to enter into this collaboration with PTC Therapeutics. PTC's RNA biology expertise and the uniqueness of the GEMS technology are complementary to our internal efforts," said Dr Susan Galbraith, Vice President and Head of the Oncology Innovative Medicines Research Unit at AstraZeneca. "We believe that the GEMS technology will enable AstraZeneca to address important disease mechanisms that were intractable with our existing approaches."

"We are honored to enter into this partnership with AstraZeneca," said Stuart Peltz, Ph.D., President and CEO of PTC Therapeutics. "AstraZeneca is widely recognized for its high scientific standards and track record in developing novel therapies for the world's most serious illnesses."

### Notes to editors:

### **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 multiple oncology clinical trials.

### **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](http://www.ptcbio.com).

### **About AstraZeneca**

AstraZeneca is a global, innovation-driven biopharmaceutical business with a primary focus on the discovery, development and

commercialisation of prescription medicines for gastrointestinal, cardiovascular, neuroscience, respiratory and inflammation, oncology and infectious disease. AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. For more information please visit: [www.astrazeneca.com](http://www.astrazeneca.com).

**Reference:**

1. WHO. Cancer. Fact sheet No. 297. February 2011. <http://www.who.int/mediacentre/factsheets/fs297/en/index.html>.

**For more Information:**

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