



## **PTC THERAPEUTICS ANNOUNCES DRUG DISCOVERY COLLABORATION WITH ROCHE**

### **- Utilizing GEMS technology to address novel or intractable targets -**

**SOUTH PLAINFIELD, N.J., September 2, 2009** – PTC Therapeutics, Inc. (PTC) today announced an exclusive research collaboration and licensing agreement with Roche for the development of orally bioavailable small molecules utilizing PTC's technology called Gene Expression Modulation by Small-molecules (GEMS™). The collaboration focuses initially on four CNS disease targets to be jointly selected.

Under the terms of the agreement, Roche will make an upfront cash payment of \$12 million and fund PTC's research efforts. Subject to achievement of several successive milestones, there is the potential for PTC to earn up to \$239 million in research, development, regulatory and commercial milestone payments per target. PTC would also receive up to double digit royalties for all products resulting from this collaboration. Roche has the option to add four targets to the collaboration across therapeutic areas, for additional cash payments.

"We are pleased to enter into this collaboration with PTC Therapeutics. PTC's expertise in small molecules addressing RNA biology and the demonstrated productivity of their GEMS technology platform make this an attractive collaboration," said Dr. Christer Nordstedt, Head, CNS Discovery of Roche. "We believe that this novel and highly innovative technology will enable Roche to address important disease mechanisms that were intractable with conventional approaches. This may have profound impact on the treatment and management of several CNS disorders and therefore lead to great benefits for the patient."

"We are excited to enter into this partnership with Roche," said Stuart Peltz, Ph.D., president and CEO of PTC Therapeutics. "This alliance is part of our ongoing strategy to leverage the broad value of the GEMS technology beyond our core research areas. Together with Roche, we are well-positioned to pursue high-interest targets to address complex CNS disorders and bring therapeutic options for patients in need."

### **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 PTC THERAPEUTICS, INC.**

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary, 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 genetic disorders, oncology and infectious diseases. PTC has extensive knowledge of post-transcriptional control processes and has developed proprietary technologies that it applies in its drug discovery activities. PTC's expertise has been the basis for collaborations with leading biopharmaceutical companies such as Genzyme, Celgene, Pfizer, Gilead, Roche and Schering-Plough. For more information, visit the company's web site at [www.ptcbio.com](http://www.ptcbio.com).

### **FOR MORE INFORMATION:**

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