



PTC Therapeutics Receives \$2.6 Million in Government Grants

Funding supports continued focus on rare disorders

SOUTH PLAINFIELD, NJ - June 15, 2009 - PTC Therapeutics, Inc. (PTC) today announced the receipt of two government grants to support clinical development programs in rare disorders. The U.S. Department of Defense Neurofibromatosis Research Program has awarded an \$822,345 grant to support a new open-label Phase 2 clinical trial of PTC's product candidate PTC299 in neurofibromatosis type 2 (NF2), a rare genetic tumor. Separately, the Food and Drug Administration (FDA) Office of Orphan Products Development has awarded PTC a four year, \$1.6 million grant towards its ongoing pivotal trial of ataluren (PTC124™) in nonsense mutation Duchenne and Becker muscular dystrophy (nmDMD/BMD).

"We are honored to receive these grant awards from the FDA and from the Department of Defense," said Stuart W. Peltz, Ph.D., president and Chief Executive Officer of PTC Therapeutics. "Obtaining peer-reviewed grant awards are a strong endorsement of PTC's science and support our strong commitment to developing innovative medicines in areas of high unmet medical need."

Patients with NF2, a rare genetic disorder of the central nervous system, typically develop tumors that compress the auditory nerves, often leading to deafness and problems with balance. If unchecked, NF2-related tumors may eventually damage other cranial nerves and the brainstem, becoming life threatening. Current standard treatments for NF2-related tumors include surgery and radiation therapy. VEGF plays a critical role in angiogenesis, or the formation of new blood vessels, and supports tumor growth in patients with NF2. PTC299 is a novel, orally administered small-molecule investigational new drug that selectively blocks the pathological, or disease-related, production of the protein vascular endothelial growth factor (VEGF) in tumors, while sparing physiological VEGF expression.

The grant awarded by the FDA Office of Orphan Products Development provides additional funding towards an ongoing pivotal, placebo-controlled study that is designed to determine whether ataluren can improve walking, muscle function and strength in patients with nmDMD/BMD. The study is also evaluating ataluren's long-term safety profile. The pivotal trial is being conducted at 37 sites across Australia, Canada, Europe, Israel and the United States. PTC announced in February that the study successfully completed enrollment two months ahead of schedule.

ABOUT PTC299

PTC299 is a novel, orally administered small-molecule investigational new drug that inhibits the production of the protein vascular endothelial growth factor, or VEGF, in tumors. PTC discovered PTC299 through PTC's proprietary GEMS (Gene Expression Modulation by Small-Molecules) technology by targeting the post-transcriptional processes that regulate VEGF formation. Overexpression of VEGF plays a key role in the growth of many types of cancers. PTC299 inhibits VEGF production through a mechanism that is distinct from other VEGF inhibitors and has potential application in multiple tumor types.

In Phase 1 clinical studies, PTC299 has been generally well tolerated by healthy volunteers and patients with cancer. Adverse events have been mild or moderate in severity and have not usually been considered drug-related. In addition, adverse events commonly seen with other VEGF blockers, such as bleeding, hypertension and proteinuria, have not been observed. Clinical trials for PTC299 are also being conducted in patients with other solid tumors, metastatic breast cancer, and Kaposi sarcoma.

ABOUT ATALUREN (PTC124™)

Ataluren is an orally delivered, investigational new drug discovered by PTC Therapeutics. The drug is being developed as a new approach for the treatment of nonsense mutation genetic disorders. Nonsense mutations create a premature stop signal in the mRNA causing the ribosome to terminate translation before a full-length protein is generated. This causes the protein to be truncated and non-functioning. Ataluren is designed to allow the ribosome to continue translation of the mRNA, overriding the premature stop signal and leading to the formation of a functioning protein. Ataluren is a potential therapy for patients with nonsense mutation genetic disorders.

Ataluren has demonstrated proof of concept in Phase 2a clinical trials and is currently in a registration-directed pivotal study with the goal of demonstrating that allowing expression of a critical missing protein will safely provide clinical benefits for patients with nonsense mutation genetic disorders. Ataluren has been generally well tolerated across all clinical studies to date.

The FDA and the European Commission have granted PTC124 (ataluren) orphan drug status for the treatment of DMD and cystic fibrosis due to nonsense mutations. The FDA has also granted PTC124 (ataluren) Subpart E designation for expedited development, evaluation, and marketing.

PTC has an exclusive collaboration with Genzyme Corporation for the development and commercialization of ataluren. PTC Therapeutics will market ataluren in the United States and Canada, while Genzyme will commercialize the product in other regions of the world. The development of ataluren has also been supported by grants from the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), FDA's Office of Orphan Products Development, the Muscular Dystrophy Association, the Parent Project Muscular Dystrophy, and the National Center for Research Resources.

ABOUT PTC THERAPEUTICS

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, Pfizer, Celgene, CV Therapeutics and Schering-Plough. For more information, visit the company's Web site at www.ptcbio.com.

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