



PTC THERAPEUTICS INITIATES PHASE 2 CLINICAL TRIAL OF PTC299 IN PATIENTS WITH NEUROFIBROMATOSIS TYPE 2

- Represents first oral therapy trial for NF2 -

SOUTH PLAINFIELD, NJ - July 27, 2009 - PTC Therapeutics, Inc. (PTC) today announced the initiation of a Phase 2 clinical trial of PTC299 in adult patients with neurofibromatosis type 2 (NF2), a rare genetic disorder that causes the development of non-malignant brain tumors. PTC299 is a novel, orally administered investigational new drug that is designed to selectively block tumor-related vascular endothelial growth factor (VEGF). With its novel mechanism of action, PTC299 has the potential to meet significant unmet medical need in NF2 and other conditions resulting from the overexpression of VEGF.

VEGF plays a critical role in the formation of new blood vessels or angiogenesis and its suppression has been shown to decrease tumor size and improve hearing in some patients with NF2. PTC299 selectively blocks the tumor-related production of VEGF, while sparing the physiological VEGF expression necessary to maintain the growth and function of normal blood vessels. Patients with NF2 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, and become life-threatening. Current standard treatments for NF2-related tumors include surgery and radiation therapy. However, a study conducted at Massachusetts General Hospital and recently published in the *New England Journal of Medicine* validates the potential of anti-angiogenesis therapies for NF2 patients.

"The development of PTC299 for the treatment of NF2 is noteworthy for patients, especially following our recent findings that an angiogenesis inhibitor improved hearing and led to tumor shrinkage in some patients," said Scott R. Plotkin, M.D., Ph.D., director of the Neurofibromatosis Clinic at the Pappas Center for Neuro-Oncology in the Massachusetts General Hospital Cancer Center. "If shown to be efficacious, we believe that its oral administration and generally well-tolerated profile could make PTC299 an especially appealing treatment approach for NF2 patients with growing vestibular schwannomas."

The new PTC299 clinical study is an open-label Phase 2 trial that will evaluate the efficacy, safety, and pharmacodynamics of PTC299 in up to 25 patients with NF2 for up to 48 weeks. The primary objective of the study is to assess how PTC299 affects tumor volume and changes in hearing and word recognition among patients with NF2. The trial will also assess the effects of PTC299 on tumor blood flow and VEGF-related proteins. The study will be conducted at the Massachusetts General Hospital in Boston, Massachusetts.

"We are excited to rapidly advance the clinical development of PTC299 in NF2," said Langdon Miller, M.D., Chief Medical Officer of PTC Therapeutics. "We are hopeful that the novel mechanism of PTC299 will address unmet medical need for patients with NF2, building upon ongoing evaluations of the drug in patients with metastatic breast cancer, Kaposi's sarcoma, and other solid tumors."

In previous Phase 1 clinical studies, PTC299 was generally well tolerated by healthy volunteers who received single or multiple doses, with no serious, dose-limiting or definitively drug-related adverse events reported. All clinical adverse events observed were mild or moderate in severity, did not require intervention, and were not clearly related to PTC299. In addition, adverse events such as bleeding, hypertension and proteinuria were not observed.

The Phase 2 clinical trial of PTC299 is supported by a grant from the U.S. Department of Defense Neurofibromatosis Research Program.

ABOUT PTC299

PTC 299 is a novel, orally administered small-molecule compound that inhibits the production of the protein vascular endothelial growth factor (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 tumors. PTC299 selectively blocks the tumor-related production of VEGF through a mechanism that is distinct from that of other VEGF inhibitors, while sparing the physiological VEGF expression necessary to maintain the growth and function of normal blood vessels. Clinical trials are currently ongoing in metastatic breast cancer, Kaposi sarcoma, and other solid tumors. For more information regarding PTC299 clinical trials, visit www.clinicaltrials.gov and search "PTC299".

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 have been the basis for collaborations with leading biopharmaceutical companies such as Celgene, Genzyme, Gilead, Pfizer, and Schering-Plough. For more information, visit the company's Web site at www.ptcbio.com.

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