

FOR IMMEDIATE RELEASE

**PTC THERAPEUTICS INITIATES PHASE 2 STUDY
OF PTC124 IN CYSTIC FIBROSIS**

SOUTH PLAINFIELD, NJ – December 6, 2005 - PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced the initiation of a Phase 2 study of PTC124 in patients with cystic fibrosis (CF) due to a nonsense mutation. PTC124 is a novel, orally administered drug that targets nonsense mutations and is being investigated initially as a treatment for CF and Duchenne muscular dystrophy (DMD), with the potential to treat a number of other genetic disorders.

Langdon L. Miller, M.D., PTC's Chief Medical Officer, commented, "Through conduct of the Phase 2 trial, we intend to establish proof of principle for PTC124 by demonstrating production of full-length, functional cystic fibrosis transmembrane conductance regulator (CFTR) in patients with CF due to a nonsense mutation. We hope that the pharmacodynamic effects of PTC124 can eventually be translated into clinical benefit for CF patients with this life-threatening disease."

The Phase 2 clinical study is enrolling patients who have CF due to the presence of a nonsense mutation in the CFTR gene. The primary endpoint of this Phase 2 clinical study is assessment of nasal transepithelial potential difference (NPD or TEPD) as a measure of CFTR function in response to treatment with PTC124. Secondary assessments of the induction of CFTR cellular protein, pulmonary function, safety, pharmacokinetics, and compliance will also be performed.

"The initiation of Phase 2 is an important milestone in the development of PTC124 and a wonderful achievement for PTC and our multiple collaborators," stated Stuart W. Peltz, Ph.D., President and CEO of PTC. "PTC124 is a new type of treatment, aimed at the root cause of the disease, and the progress we have been able to achieve is due to the dedication and support of multiple researchers, investigators, clinicians, and patient advocacy groups."

"We are excited that this innovative therapy, that addresses the basic CF defect, has moved into the next stage of clinical trials," said Preston W. Campbell, III, M.D., Executive Vice President for Medical Affairs at the Cystic Fibrosis Foundation. "PTC has been a great partner every step of the way."

PTC has commenced recruitment for the Phase 2 study in CF at the University of Alabama, Birmingham (UAB), AL and the Denver Children's Hospital in Denver CO. Additional sites in the United States include the Johns Hopkins Hospital in Baltimore, MD; and the Rainbow Babies' and Children's Hospital in Cleveland, OH; More details regarding the design and conduct of this study is available at www.clinicaltrials.gov.

"We are thrilled to have begun the Phase 2 study of PTC124 in CF patients. It is particularly rewarding for us because Dr. David Bedwell and his team here at UAB were the first scientists to investigate the concept of readthrough of nonsense mutations as a potential treatment strategy for CF, and developed the preclinical data that support the PTC124 clinical research program in CF," commented JP Clancy, M.D., Director of Pediatric Pulmonary Medicine at UAB. "The protocol will be performed at four sites across the US, and there has already been quite a bit of interest expressed by patients from other CF centers about how they can participate. All of the centers are nearly ready to go, and we hope to have preliminary results to report in 2006."

PTC also has plans to initiate a separate CF study of PTC124 in Israel, where nonsense-mutation-mediated CF is particularly prevalent, and hopes to initiate a Phase 2 study of PTC124 in DMD within the fourth quarter of 2005.

ABOUT PTC THERAPEUTICS, INC.

PTC is a biopharmaceutical company focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms. Post-transcriptional control processes are the sequence of events in the cell that ultimately regulate the rate and timing of all protein production. PTC's compounds alter these processes by selectively modulating how RNA is used to produce proteins. By applying this approach, PTC has advanced its drug discovery programs rapidly from targets to preclinical and clinical drug candidates, building a robust pipeline across genetic disorders, oncology, and infectious diseases.

ABOUT PTC124

PTC124 represents a first-in-class, orally delivered investigational new drug for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non-functional protein. In pre-

clinical trials, PTC124 allowed the cellular machinery to bypass the nonsense mutation, continue the translation process, and thereby restore the production of a full-length, functional protein. PTC124 has demonstrated the ability to restore full-length functional protein in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical studies, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models, and did not induce ribosomal readthrough of normal stop codons. Pharmacokinetic modeling of the Phase 1 results has allowed development of a dosing regimen for the Phase 2 studies in cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD). It is estimated that 10% of the cases of CF and 15% of the cases of DMD are due to nonsense mutations. In addition to CF and DMD, other potential indications include hemophilia, neurofibromatosis, retinitis pigmentosa, epidermolysis bullosa, and lysosomal storage disorders. PTC124 may represent a unique opportunity to use a single small-molecule drug to address chronic and life-threatening diseases of high unmet medical need. The FDA has granted PTC124 fast-track designation for the treatment of CF and orphan drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of DMD and CF by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA). PTC124's development is supported by grants from the Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Muscular Dystrophy Association (MDA), FDA's Office of Orphan Products Development (OOPD), and by General Clinical Research Center grants from the National Center for Research Resources (NCRR).

ABOUT CYSTIC FIBROSIS (CF)

CF is a life-threatening, genetic disease affecting approximately 70,000 people worldwide. A defective gene coding the cystic fibrosis transmembrane conductance regulator (CFTR) protein causes the body to produce abnormally thick, sticky mucus that leads to chronic lung-infections and impairs digestion. It is estimated that approximately ten percent of CF patients have the disease due to a nonsense mutation. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

FOR MORE INFORMATION:

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