PTC THERAPEUTICS INITIATES REGISTRATION-DIRECTED PHASE 3 TRIAL OF ATALUREN IN CYSTIC FIBROSIS

- First investigational drug designed to address the underlying cause of nonsense mutation cystic fibrosis -

SOUTH PLAINFIELD, NJ – September 10, 2009 – PTC Therapeutics, Inc. (PTC) today announced the initiation of a Phase 3 trial of ataluren (formerly PTC124®), an investigational protein restoration therapy in patients with nonsense mutation cystic fibrosis (nmCF). Patients with CF lack adequate levels of the cystic fibrosis transmembrane conductance regulator (CFTR) protein, a chloride channel necessary for normal function of the lung, pancreas, liver, and other organs. In nmCF, an interruption in the genetic code—known as a nonsense mutation—prematurely halts the synthesis of CFTR, causing the protein to be short and non-functioning. Nonsense mutations are categorized as Class I mutations that result in little or no production of the CFTR protein. CF patients with Class I mutations typically experience more severe disease symptoms than those with low-risk genotypes, including a greater than twofold increased risk of death1, a higher probability of end-stage lung disease, and a higher prevalence of pancreatic insufficiency.2 Ataluren is designed to promote restoration of the missing CFTR. Through advances in genetic analysis, a simple test can now determine if a patient's disease is caused by a nonsense mutation.

The primary objective of the registration-directed double-blind, placebo-controlled study is to evaluate whether ataluren can improve lung function, as measured by forced expiratory volume in one second (FEV1), in patients with nmCF. Other outcome measures will evaluate whether ataluren can reduce symptoms associated with nmCF, decrease lung infections, reduce the frequency of cough, and improve patient-reported quality of life. The 48-week trial is now enrolling patients at multiple research centers in North America, Europe, and Israel. Study candidates include patients who are at least six years of age and have CF due to a nonsense mutation.

"Promising Phase 2 clinical trial data show that patients treated with ataluren can produce functional CFTR protein, resulting in improvements in chloride channel activity," said Frank Accurso, M.D., Professor of Pediatrics and Section Head of Pulmonary Medicine of the University of Colorado, Denver and a leading ataluren investigator. "With the newly initiated Phase 3 study we hope to determine if the effects of ataluren on the underlying cause of the disease can result in clinical benefit for patients with nmCF."

"As an oral therapy that may address the underlying cause of the disease, ataluren has the potential to improve the management of nmCF for patients and their physicians," said Christiane DeBoeck, M.D., Ph.D., Principal Investigator for University Hospital Leuven. "It is our hope that this long-term clinical trial of ataluren will advance our knowledge of the disease and the standard of care for nmCF patients."

"The initiation of this Phase 3 trial represents an important step forward in our efforts to develop treatments for the underlying cause of cystic fibrosis," said Robert J. Beall, Ph.D., President and CEO of the Cystic Fibrosis Foundation. "Phase 2 studies of ataluren showed encouraging potential to restore CFTR protein production and we are pleased to be supporting this study." In July 2008, Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), the nonprofit affiliate of the Cystic Fibrosis Foundation, expanded its existing collaboration with PTC Therapeutics. PTC will receive up to $25 million from CFFT in support of the development of ataluren.

An orally administered therapy, ataluren is the first investigational new drug designed to enable the formation of a functioning protein in patients with genetic disorders due to a nonsense mutation. Restoring functional CFTR protein may enable ataluren to address the underlying cause of nmCF and change the course of the disease. Currently available therapies for CF focus only on the alleviation of symptoms.

"We are excited to expand the ataluren registration program to include nmCF, an area of significant unmet medical need," said Langdon Miller, M.D., Chief Medical Officer of PTC Therapeutics. "We are hopeful that this Phase 3 trial together with our ongoing studies in nmDMD/BMD, will add to a growing body of data demonstrating ataluren's potential for treatment of patients with nonsense mutation genetic disorders."

ABOUT CYSTIC FIBROSIS (CF)
CF is a life-threatening genetic disease that causes serious lung infections and digestive complications. According to the Cystic

Fibrosis Foundation, CF affects approximately 30,000 adults and children in the United States and nearly 70,000 people worldwide. Genetic testing is required to confirm a complete diagnosis and to determine if a patient's disease is caused by a nonsense mutation. It is estimated that nonsense mutations are the cause of CF in 10 percent of patients in the United States and Europe and over 50 percent of patients in Israel. Available treatments for CF are designed to alleviate symptoms rather than correct the underlying cause of the disease. These treatments include chest physical therapy to clear thick mucus from the lungs, antibiotics to treat lung infections, and a mucus-thinning drug designed to reduce the number of lung infections and improve lung function. In addition, the majority of cystic fibrosis patients take pancreatic enzyme supplements to assist with food absorption in digestion. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

ABOUT ATALUREN (PTC124®)
Ataluren is the first investigational new drug designed to restore the formation of a functioning protein in patients with genetic disorders due to a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. Ataluren is currently being investigated for use in patients with nmCF and nmDMD/BMD.

Ataluren has been granted orphan drug status for the treatment of nmCF and nmDMD/BMD by the U.S. Food and Drug Administration (FDA) and the European Commission. The FDA has also granted ataluren Subpart E designation for expedited development, evaluation, and marketing. The development of ataluren has been supported by the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), the FDA Office of Orphan Products Development, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, and the National Center for Research Resources.

COMPLETED ATALUREN CLINICAL TRIALS
Data from Phase 2a clinical trials of ataluren in pediatric and adult patients with nmCF show that administration of ataluren results in production of functional CFTR and statistically significant improvements in CFTR chloride channel function in the airways. Ataluren treatment is also associated with reductions in cough frequency and improvements in pulmonary function tests.

Across all ataluren clinical trials to date, including Phase 1 healthy-volunteer trials, ataluren has been generally well tolerated. In Phase 2a trials in nmCF and nmDMD/BMD, adverse events have been largely consistent with background symptoms and have usually been mild. No concerning adverse findings have been identified based on physical examinations, vital sign measurements, electrocardiograms, or laboratory studies. The mean compliance with ataluren therapy has been greater than 90 percent in all trials.

COLLABORATION WITH GENZYME
PTC Therapeutics 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.

ABOUT THE CYSTIC FIBROSIS FOUNDATION
The Cystic Fibrosis Foundation, the leading organization focused on curing and controlling cystic fibrosis (CF), has invested nearly $300 million in drug research with biotech companies since 1998 to develop therapies to fight CF. As a result, the Foundation has built a drug pipeline with more than 30 promising therapies in development. Virtually all the approved CF therapies available today were made possible because of the support of the Foundation. Based in Bethesda, MD, the Foundation has 70 chapters and branch offices, and supports and accredits a nationwide network of more than 110 CF care centers that provide treatment and vital resources to patients and families. For more information on the Cystic Fibrosis Foundation, visit www.cff.org.

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, Gilead, Roche and Schering-Plough. For more information, visit the company's Web site at www.ptcbio.com.

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