PTC Therapeutics Completes Enrollment of Phase 3 Trial of Ataluren in Patients with Cystic Fibrosis

- Trial completion is expected in 2011 -

SOUTH PLAINFIELD, NJ – DECEMBER 21, 2010 – PTC Therapeutics, Inc. announced today that it has completed enrollment of a Phase 3 clinical trial of ataluren, an investigational new drug, in patients with nonsense mutation cystic fibrosis (nmCF).

The 48-week study is designed to determine whether ataluren can improve lung function in patients with nmCF. The trial has enrolled 238 patients at 36 sites in North America, Europe and Israel. Patients who complete the treatment phase of the Phase 3 trial are eligible to participate in a 48-week, open-label extension study, which has begun enrolling patients.

"The enrollment of this trial represents an important step forward in our efforts to develop disease-modifying treatments that advance the standard of care in CF and improve quality of life for CF patients," stated Michael Konstan, MD, Chairman, Department of Pediatrics at Rainbow Babies and Children's Hospital in Cleveland, Ohio. Dr. Eitan Kerem, Head, Department of Pediatrics and CF Center, Hadasash University Hospital, Jerusalem, Israel added, "Despite significant advances in the 21 years since the identification of the disease-causing gene, cystic fibrosis remains a debilitating and life-threatening disorder and available therapies focus only on alleviating symptoms. Ataluren couples a patient's genetic diagnosis with a mutation-specific therapeutic approach designed to address the underlying cause of the disease."

Ataluren is a protein restoration therapy designed to enable the formation of full-length, functional cystic fibrosis transmembrane regulator (CFTR) protein in patients with cystic fibrosis due to a nonsense mutation. CFTR is a critical protein lacking in CF patients. 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 lower-risk genotypes, including a greater than twofold increased risk of death, a higher probability of end-stage lung disease and a higher prevalence of pancreatic insufficiency. A simple genetic test can determine if a patient's disease is caused by a nonsense mutation.

"We are pleased to have completed enrollment of our second pivotal clinical trial of ataluren in patients with a nonsense mutation genetic disorder. This is a tremendous achievement and a testament to the commitment of clinical trial patients and their families, as well as study investigators and trial site staff," stated Stuart Peltz, Ph.D., president and CEO of PTC Therapeutics. "PTC is committed to improving the quality of life of patients with serious and life-threatening diseases through our innovative scientific approach to the discovery of novel treatments."

PHASE 3 STUDY DESIGN
The primary objective of the registration-directed, double-blind, placebo-controlled study is to determine whether ataluren can improve lung function in patients with nmCF, as measured by forced expiratory volume in 1 second (FEV1). Additional secondary endpoints are evaluating other aspects of patient function, drug activity, and safety. The 48-week trial enrolled 238 patients, ages six years and older, at multiple sites in North America, Europe, and Israel. Patients were randomly assigned to one of two treatment arms: ataluren (10 mg/kg morning, 10 mg/kg midday, 20 mg/kg evening) or placebo (morning, midday, evening).

ABOUT ATALUREN
An investigational new drug discovered by PTC Therapeutics, ataluren is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as the CFTR protein in nonsense mutation cystic fibrosis.

The development of ataluren has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); FDA's Office of Orphan Products Development; Muscular Dystrophy Association; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

The FDA and the European Commission have granted ataluren Orphan Drug status for the treatment of nonsense mutation cystic fibrosis and nonsense mutation Duchenne and Becker muscular dystrophy. The FDA has also granted ataluren Subpart E designation for expedited development, evaluation, and marketing for CF and dystrophinopathy and Fast Track designation for the development of treatment for nonsense mutation dystrophinopathy.

COLLABORATION WITH GENZYME
PTC Therapeutics has an exclusive collaboration with Genzyme Corporation for the development and commercialization of ataluren. PTC Therapeutics will commercialize ataluren in the United States and Canada, while Genzyme will commercialize the product in other regions of the world.

ABOUT CYSTIC FIBROSIS (CF)
CF is a life-threatening genetic disorder that causes serious lung infections and digestive complications. The predicted median age of survival for a person with CF is about 37 years. 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 about 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. Based on the current standard of care, the treatment burden for CF patients is high and, on average, adults with CF take 7 daily therapies. More information regarding CF is available through the Cystic Fibrosis Foundation (www.cff.org).

ABOUT PTC THERAPEUTICS, INC.
PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered 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 rare genetic disorders, oncology, and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and is the basis for collaborations with leading biopharmaceutical companies. For more information, visit the company's web site at www.ptcbio.com.

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