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PTC THERAPEUTICS AWARDED $1.6 MILLION FDA ORPHAN DRUG GRANT TO SUPPORT AN ONGOING PHASE 3 STUDY IN CYSTIC FIBROSIS

SOUTH PLAINFIELD, NJ – SEPTEMBER 21, 2010 – PTC Therapeutics, Inc. (PTC) today announced a grant award of approximately $1.6 million from the U.S. Food and Drug Administration (FDA) Office of Orphan Products Development (OPD) to support an ongoing Phase 3 study of ataluren in patients with nonsense mutation cystic fibrosis (nmCF). The goal of the FDA's OPD grant program is to support the clinical development of products for use in rare diseases or conditions where no current therapy exists or where the proposed product will be superior to the existing therapy.

"We are pleased to receive this grant from the FDA to support our ongoing development of ataluren for the treatment of nonsense mutation cystic fibrosis," stated Stuart W. Peltz, Ph.D., President and Chief Executive Officer of PTC Therapeutics. "In addition to recognizing the potential of ataluren, this grant reflects PTC's long-standing commitment to developing new treatments for patients with rare genetic disorders that severely impact survival and quality of life."

The Phase 3 trial will enroll approximately 200 patients at research centers in North America, Europe and Israel. The primary goal of the study is to evaluate whether ataluren can improve lung function as measured by forced expiratory volume (FEV1). Other outcome measures will evaluate whether ataluren can decrease lung infections, reduce the frequency of cough and improve patient-reported quality of life. Patients are randomized to receive either ataluren (40 mg/kg) or placebo daily for 48 weeks. Study candidates include patients who are at least six years of age and have CF due to a nonsense mutation.

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 CFTR. CF patients with Class I mutations typically experience more severe disease symptoms than those with low-risk genotypes, including a greater than two-fold increased risk of death, a higher probability of end-stage lung disease and a higher prevalence of pancreatic insufficiency. Ataluren is designed to restore 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.

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
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. Ataluren is currently being investigated in several clinical trials for use in multiple nonsense mutation genetic disorders.

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.

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 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 has served as the basis for collaborations with leading biopharmaceutical companies such as Celgene, Genzyme, Gilead, Merck, Pfizer and Roche. For more information, visit the company's web site at www.ptcbio.com.

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