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Top-Line Data from Phase 3 Trial of Ataluren in Patients with Nonsense Mutation Cystic Fibrosis Show Promising Results

- Data Demonstrate Positive Trends in Lung Function and Pulmonary Exacerbations -

SOUTH PLAINFIELD, NJ – June 8, 2012 – PTC Therapeutics, Inc. today announced the results from a Phase 3 study of ataluren, an investigational new drug, in patients with nonsense mutation cystic fibrosis (nmCF). The results demonstrated positive trends in lung function as measured by FEV1 (forced expiratory volume in one second) and in the secondary outcome measure, rate of pulmonary exacerbations. Results also showed a substantial ataluren treatment effect in FEV1 and exacerbation rate in the pre-specified subpopulation of patients not receiving chronic inhaled antibiotics, representing approximately one half of the study population. These data were presented at the European Cystic Fibrosis Society Conference in Dublin, Ireland.

"The data from this trial are promising. Ataluren has the potential to provide benefit to patients with nonsense mutation cystic fibrosis, who currently have few treatment options. Nonsense mutations do not produce any functional CFTR protein and therefore generally result in a more severe form of cystic fibrosis," stated Michael Konstan, M.D., a principal investigator at University Hospitals Rainbow Babies and Children's Hospital in Cleveland, Ohio. "Correcting a nonsense mutation in the cystic fibrosis transmembrane conductance regulator gene is a big challenge. Over 48 weeks, the decrease in FEV1 in ataluren was 50% less than the decrease on placebo, which is a substantial difference. It is also very important to note that the FEV1 and exacerbations tracked closely."

This Phase 3 study, which was conducted across 11 countries, was a double-blind, placebo-controlled study comparing ataluren (n=116) to placebo (n=116) in nmCF patients. The primary endpoint, the relative change from baseline in %-predicted FEV1 at 48 weeks, showed a positive trend favoring ataluren versus placebo, and a larger effect in the patients not receiving chronic inhaled antibiotics. The effect of inhaled antibiotics was largely attributable to the use of inhaled aminoglycosides. In the intent-to-treat population, there was a 3% difference in the relative change from baseline in %-predicted FEV1 between the ataluren and placebo groups at Week 48 (-2.5% change on ataluren vs. -5.5% change on placebo; p=0.124). An analysis of the relative change from baseline in %-predicted FEV1 across all post-baseline study visits demonstrated an average difference between ataluren and placebo of 2.5% (-1.8% average change on ataluren vs. -4.3% average change on placebo; p= 0.0478).

The study was stratified by age, baseline FEV1, and the use of chronic inhaled antibiotics. A statistically significant effect (p=0.0072) was seen between treatment and use of inhaled antibiotics at baseline, indicating that inhaled antibiotics was a significant confounder of the overall results. A substantial treatment effect was seen in the patients not receiving chronic inhaled antibiotics at baseline; the Week 48 difference between the ataluren and placebo arms in FEV1 was 6.7% (-0.2% change on ataluren vs. -6.9% change on placebo). The secondary endpoint, the rate of pulmonary exacerbations (ie, the number of pulmonary exacerbations in 48 weeks) also showed a positive trend in favor of ataluren, with the rate in the ataluren group being 23% lower than the placebo group (p=0.0992). In the patients not receiving chronic inhaled antibiotics, the pulmonary exacerbation rate in the ataluren group was 43% lower than the rate in the placebo group. These results show a consistent treatment effect of ataluren on both pulmonary function and exacerbation rates.

Mean relative change in %-predicted FEV_1 (ITT population)		
Ataluren vs. Placebo		
	Difference	**p-value**
All patients at Week 48 ataluren n=116; placebo n=116	3.0%	0.124
All patients – average over all post-baseline visits ataluren n=116; placebo n=116	2.5%	0.0478
Patients not on chronic inhaled antibiotics ataluren n=52; placebo n=53	6.7%	0.013*

*nominal p-value

Reduction in pulmonary exacerbation rate over 48 weeks (ITT population)		
Ataluren vs. Placebo		
	Decrease in exacerbation rate	**p-value**
All patients ataluren n=116; placebo n=116	23%	0.0992
Patients not on chronic inhaled antibiotics ataluren n=52; placebo n=53	43%	0.014*

*nominal p-value

The tertiary endpoints of sweat test and nasal potential difference did not show an effect between ataluren and placebo. Preliminary data from an ongoing extension study support the sustained effect of ataluren on lung function as measured by FEV1. Further analyses of these data and other study endpoints will be reported at future scientific conferences when completed.

"This is a historic time for patients with cystic fibrosis," said Robert J. Beall, Ph.D., president and CEO, Cystic Fibrosis Foundation. "We are beginning to see the results of our committment to the development of novel treatments for patients with cystic fibrosis. Promising therapeutic approaches that target the underlying cause of cystic fibrosis are fundamental to future therapeutic options that have the potential to change the course of the disease."

Safety results indicate that ataluren was generally well tolerated. The overall incidence of adverse events through Week 48 was similar in the ataluren and placebo groups. The most common adverse events were typical for CF and included pulmonary exacerbation, cough, and upper respiratory tract infection, which occurred at similar frequencies in the ataluren and placebo arms. Most of the serious adverse events, those requiring hospitalization, were pulmonary exacerbations unrelated to study treatment and some patients experienced creatinine elevations that occurred in connection with concomitant treatment with systemic aminoglycosides.

Patients with CF lack adequate levels of the 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 other genotypes, including a shorter life span, a higher probability of end-stage lung disease, and a higher prevalence of pancreatic insufficiency. Ataluren, a protein restoration therapy, is designed to overcome the nonsense mutation and enable the production of a full-length, functional CFTR protein. A simple genetic test can determine if a patient's disease is caused by a nonsense mutation.

"PTC has a long-standing commitment to discovering and developing new treatments for rare, life-threatening disorders such as cystic fibrosis. We are very encouraged by the data from our trial, which was a long-term placebo-controlled study of a CFTR corrector targeting nonsense mutations," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, Inc. "The results in the patients not on inhaled aminoglycosides are particularly promising. Substantial differences between ataluren and placebo in mean relative change in %-predicted FEV1 and pulmonary exacerbation rate were demonstrated and the fact that both endpoints improved is a strong indicator of ataluren's activity."

ABOUT THE PHASE 3 TRIAL

The primary objective of the double-blind, placebo-controlled study was to determine whether ataluren can improve lung function relative to placebo in patients with nonsense mutation cystic fibrosis, as measured by forced expiratory volume in 1 second (FEV1). The secondary objective was to assess pulmonary exacerbation rates for ataluren compared to placebo. Additional endpoints evaluated 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). Patients who completed the study were eligible to receive open-label ataluren in an ongoing extension study.

ABOUT ATALUREN

An investigational new drug, 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 cystic fibrosis transmembrane conductance regulator protein (CFTR) in nmCF.

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.

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

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 that have served as the basis for collaborations with leading biopharmaceutical companies such as AstraZeneca, Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's website at www.ptcbio.com.

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