May 16, 2014

Ataluren Phase 3 Trial Results in Nonsense Mutation Cystic Fibrosis Published in The Lancet Respiratory Medicine

Data Demonstrate Positive Trends in Lung Function and Pulmonary Exacerbations

SOUTH PLAINFIELD, NJ – May 16, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the results of a Phase 3 study of ataluren in patients with nonsense mutation cystic fibrosis (nmCF) were published in Lancet Respiratory Medicine. The results demonstrated positive trends in both the primary endpoint, lung function as measured by relative change in % predicted FEV1 (forced expiratory volume in one second) and in the secondary outcome measure, rate of pulmonary exacerbations. The collective data from this trial, including retrospective and subgroup analyses support the conclusion that ataluren was active and showed clinically meaningful improvements over placebo in these trials.

"The overall data from this trial are promising. Patients on ataluren experienced fewer pulmonary exacerbations and showed a stabilization in their FEV1 results, particularly in the subgroup of patients that did not use chronic inhaled aminoglycosides. Such stabilization of disease is an important clinical endpoint, particularly for this patient population that has one of the most severe forms of CF. CF patients with nonsense mutations do not produce any functional CFTR protein and therefore generally have a more severe form of cystic fibrosis. Current treatments for nonsense mutation cystic fibrosis focus on alleviating symptoms and reducing infections, whereas ataluren targets the underlying cause of disease," stated Michael Konstan, M.D., lead study investigator, and Chairman of Pediatrics, at University Hospitals Rainbow Babies & Children's Hospital in Cleveland, Ohio.

The Phase 3 double-blind, placebo-controlled study, which was conducted across 11 countries, compared 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 patients not receiving chronic inhaled tobramycin. 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=0.12) which was not statistically significant. 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= 0.048). There were 23% fewer pulmonary exacerbations in the ataluren group compared to placebo (p=0.0992). Further results from a post hoc analysis of the subgroup of patients not receiving chronic inhaled tobramycin showed a 5.7% difference in relative change from baseline in % predicted FEV1 favoring ataluren, with a mean change from baseline of -0.7% in the ataluren arm, and – 6.4% in the placebo arm (nominal p=0.0082). In addition, there were 40% fewer exacerbations in ataluren-treated patients in this subgroup. The outcomes observed in multiple endpoints between the subgroup of patients who were not prescribed chronic inhaled tobramycin and the subgroup of patients who were prescribed chronic inhaled tobramycin as well as post-hoc in vitro testing showing the interference of aminoglycoside antibiotics with ataluren activity support the hypothesis that inhaled tobramycin may interfere with ataluren's mechanism of action.

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, except for the occurrence of creatinine elevations that occurred more frequently in the ataluren group in connection with concomitant treatment with systemic aminoglycosides. Most treatment emergent adverse events were of mild (Grade 1) or moderate (Grade 2) severity, and no life-threatening adverse events were reported. Most serious adverse events reported in this study were CF pulmonary exacerbations and were considered unrelated to ataluren treatment. Eight patients in the ataluren arm and three patients in the placebo arm discontinued treatment due to an adverse event.

"We are very encouraged by the data from this trial. Given spirometry and pulmonary exacerbation results in the subgroup of patients not receiving chronic inhaled tobramycin, and a favorable safety profile, this study supports further clinical testing of ataluren as a potential first-in-class treatment for nmCF patients not receiving chronic inhaled tobramycin," stated Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics, Inc. "We look forward to initiating a confirmatory ataluren trial in nmCF patients later this year."

ABOUT THE PHASE 3 TRIAL

The primary endpoint of the randomized, double-blind, placebo-controlled study was the relative change in % predicted FEV1 from baseline to Week 48 as assessed by spirometry. Spirometry was performed at screening, at randomization, and every eight weeks during the 48 week study duration. The secondary objective was rate of pulmonary exacerbations. 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 36 sites in 11 countries in North America and Europe. 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 CYSTIC FIBROSIS
Cystic fibrosis (CF) is a disabling and life-threatening autosomal recessive disorder resulting from mutations that cause dysfunction in the cystic fibrosis transmembrane conductance regulator (CFTR). 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. Approximately 10% of patients have CF due to a Class I nonsense mutation in at least one allele of the CFTR gene. Available therapies for treatment of lung manifestations of CF, such as inhaled antibiotics do not address the underlying defect. There are no marketed treatments that target the defect associated with CF caused by nonsense mutations.

ABOUT ATALUREN
Ataluren, an investigational new drug discovered and developed by PTC Therapeutics, 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 dystrophin in nmDMD. 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 and development 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 essential to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and which form the basis for collaborations with leading biopharmaceutical companies. For more information on the company, please visit our website www.ptcbio.com.

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FORWARD LOOKING STATEMENTS:
Any statements in this press release about future expectations, plans and prospects for the Company, the development of and potential market for the Company's product candidates, the timing and conduct of our clinical trial of ataluren for the treatment of cystic fibrosis caused by nonsense mutations, including statements regarding the timing of initiation and completion of the trial and the period during which the results of the trial will become available, the potential advantages of ataluren, and our estimates regarding the potential market opportunity for ataluren, and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" in our most recent Quarterly Report, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views only as of the date of this release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.