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PIVOTAL DATA PRESENTED AT THE WORLD MUSCLE SOCIETY CONGRESS SUGGEST ATALUREN SLOWS THE LOSS OF WALKING ABILITY IN PATIENTS WITH NONSENSE MUTATION DUCHENNE/BECKER MUSCULAR DYSTROPHY

- 29.7 meter average change in 6-minute walk distance compared to placebo at 48 weeks - - Safety results show ataluren was generally well tolerated - - Data to be the basis of interactions with regulatory authorities -

SOUTH PLAINFIELD, NJ – October 15, 2010 – PTC Therapeutics, Inc. today announced that final analyses of Phase 2b efficacy data suggest the investigational new drug ataluren slowed the loss of walking ability in patients with nonsense mutation dystrophinopathy, a disease continuum comprising Duchenne and Becker muscular dystrophy (nmDBMD). These data were presented at the International Congress of the World Muscle Society in Kumamoto, Japan and will be the basis of interactions with the U.S. Food and Drug Administration (FDA) and national regulatory authorities in Europe in the fourth quarter of 2010.

"Since the completion of the Phase 2b trial, we have worked diligently with investigators and expert advisors to thoroughly analyze the full data set. We are pleased that the results of these analyses suggest that ataluren provided clinically meaningful benefits to patients with nonsense mutation dystrophinopathy," said Stuart Peltz, Ph.D., President and Chief Executive Officer of PTC Therapeutics. "We have initiated interactions with regulatory authorities to continue our efforts to bring ataluren to patients."

The primary endpoint of the Phase 2b trial was the change in 6-minute walk distance (6MWD) from baseline to 48 weeks. The data showed a 29.7 meter (approximately 97 feet) difference in the average change in 6MWD when comparing the ataluren (10-, 10-, 20-mg/kg) and placebo arms. This result is consistent with the study hypothesis of a 30-meter difference and the average change in 6MWD observed in registration-directed trials of approved drugs for other diseases. The pre-specified statistical analysis required a post hoc correction, which resulted in a p-value of 0.058. The 6MWD results also showed that there was no difference between a high dose, ataluren (20-, 20-, 40-mg/kg), and placebo. The finding of improvement over placebo at the low dose of ataluren but not at the high dose is consistent with subsequent analysis of nonclinical data which suggest a bell-shaped dose response curve as a class-based effect for drugs that promote nonsense suppression.

An analysis of time to persistent 10% worsening in 6MWD indicated that patients receiving ataluren experienced statistically significant slower disease progression. At 48 weeks, only 26% of patients treated with ataluren (10-, 10-, 20-mg/kg) had progressed compared to 44% of patients treated with placebo (p=0.039). A subgroup analysis showed that mean changes in 6MWD were consistently better among patients receiving ataluren (10-, 10-, 20-mg/kg) than those receiving placebo across subgroups including age, corticosteroid use and baseline 6MWD.

The 6MWD results were further supported by positive trends in muscle function, as measured by timed function tests, in patients receiving ataluren (10-, 10-, 20-mg/kg) compared to those receiving placebo. The study was not powered to demonstrate statistical significance in these tests. In addition, an evaluation of dystrophin protein expression was included in this study as an exploratory endpoint; however, due in part to technical limitations of the assay, no relationship could be established between 6MWD – a measure of clinical benefit – and dystrophin expression.

Safety results showed that ataluren was generally well tolerated and adverse events were similar across treatment arms. No patients discontinued treatment due to an adverse event. Serious adverse events were infrequent and none were considered to be related to ataluren.

"These results suggest for the first time that a therapy which addresses the underlying cause of the disease can slow the loss of walking ability, the primary clinical symptom of dystrophinopathy," said Richard Finkel, M.D., Director of the Neuromuscular Program, Children's Hospital of Philadelphia. "The variability of symptom onset and disease progression in DBMD makes it challenging to assess the clinical benefit of investigational treatments. However, the ataluren data provide encouraging evidence of clinical benefit in patients whose prognosis is poor even with currently available palliative treatments."

ABOUT THE PHASE 2B TRIAL

The first registration-directed study in patients with DBMD, the randomized, double-blind, placebo-controlled Phase 2b trial was designed to evaluate the safety and efficacy of ataluren in patients with nmDBMD. The study enrolled 174 ambulatory males at least five years of age at 37 sites in North America, Europe, Australia and Israel. The primary endpoint was the change in 6MWD over 48 weeks. A clinically

meaningful and validated outcome measure of ambulation, the 6-minute walk test reflects improvements in global endurance and activity. Other outcome measures in the study included evaluations of muscle function, muscle strength, and muscle dystrophin expression. Safety parameters and compliance were also monitored.

Participants were randomized to one of three treatment arms to daily receive:

- ataluren (10 mg/kg morning, 10 mg/kg midday, 20 mg/kg evening)

- ataluren (20 mg/kg morning, 20 mg/kg midday, 40 mg/kg evening)

- 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 dystrophin in nmDBMD.

The FDA and the European Commission have granted ataluren Orphan Drug status for the treatment of nmDBMD and nonsense mutation cystic fibrosis. The FDA has also granted ataluren Subpart E designation for expedited development, evaluation, and marketing 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 DYSTROPHINOPATHY
Dystrophinopathy is a disease continuum comprising Duchenne and Becker muscular dystrophy (DBMD), progressive muscle disorders primarily affecting males caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of skeletal, diaphragm, and heart muscles. Patients with Duchenne muscular dystrophy, the more severe form of the disorder, lose the ability to walk as early as age 10 and experience life-threatening lung and heart complications in their late teens and twenties. A smaller subset is classified as having Becker muscular dystrophy, a milder variation of the disorder that is associated with later manifestation of symptoms. About 10 to 15 percent of all DBMD cases are caused by nonsense mutations in the dystrophin gene. There are an estimated 1,700 and 2,200 patients with nmDBMD in the United States and Europe, respectively. More information about DBMD is available through the Muscular Dystrophy Association (www.mdausa.org) and Parent Project Muscular Dystrophy (www.parentprojectmd.org).

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. For more information, visit the company's web site at www.ptcbio.com.

FOR MORE INFORMATION:

PTC Therapeutics

Media and Investor Contacts:
Jane Baj
PTC Therapeutics, Inc.
(908) 912-9167
jbaj@ptcbio.com

Sheryl Seapy
Pure Communications
(949) 608-0841
sheryl@purecommunicationsinc.com

Patient, Family, and Patient Organization Contact:
Diane Goetz
PTC Therapeutics, Inc.
(908) 912-9256 or (866) 282-5873

patientinfo@ptcbio.com

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