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PTC THERAPEUTICS ANNOUNCES ADDITIONAL STUDY OF ATALUREN IN PATIENTS WITH ADVANCED NONSENSE MUTATION DUCHENNE/BECKER MUSCULAR DYSTROPHY

- Study funded in part by a grant from the Muscular Dystrophy Association-

SOUTH PLAINFIELD, NJ – January 19, 2010 – PTC Therapeutics, Inc. (PTC) today announced the initiation of an additional clinical trial of ataluren (PTC124®) in boys and young men with nonsense mutation Duchenne/Becker muscular dystrophy (nmDBMD) who have permanently lost the ability to walk independently. This trial is evaluating the best methods for measuring functional abilities in patients who have lost independent mobility. Patients with nmDBMD develop progressive muscle weakness that leads to deterioration of ambulation, wheelchair dependency, progressive loss of upper limb strength, and eventual respiratory and cardiac failure. The study, which is expected to complete enrollment rapidly, is being funded in part by a $1 million grant from the Muscular Dystrophy Association, and will involve MDA's five-center DMD Clinical Research Network and a site in the United Kingdom.

"Given the relentlessly progressive course of DBMD and its impact on multiple organ systems, we believe patients could benefit from therapeutic interventions at all stages of the disease. This trial is an important step in understanding the best methods for evaluating new clinical treatments for these boys and young men," stated Langdon Miller, M.D., Chief Medical Officer at PTC.

"The Muscular Dystrophy Association is proud to provide $1 million in funding towards this ground-breaking study in DBMD patients who have lost the ability to walk," said Valerie Cwik, M.D., Executive Vice President – Research and Medical Director, Muscular Dystrophy Association. "This trial underscores the importance of MDA's translational research effort. In April 2005, MDA awarded an initial $1.5 million grant to PTC in support of early clinical development of ataluren in nmDBMD. Now we have a trial that will provide valuable information on older DBMD boys and young men that will assist in the design of future clinical trials."

This one-year, Phase 2a study is evaluating the safety, pharmacodynamic activity, and pharmacokinetics of ataluren, an investigational new drug, while assessing the use of several outcome measures of physical, pulmonary, and cardiac function in patients with advanced disease. Approximately 30 patients are being enrolled in the trial at five sites in the United States, which are part of the MDA's DMD Research Network, and at one site in the United Kingdom. Additional information on this study can be found on http://clinicaltrials.gov.

"We are eager to participate in this innovative study of non-ambulatory DMD patients," stated John W. Day, M.D., Ph.D., principal investigator at the University of Minnesota's Paul and Sheila Wellstone Muscular Dystrophy Center. "This precedent-setting trial is an important extension of current studies of younger, ambulatory boys with DMD, and will help define methods for assessing heart, lung and muscle function in patients with more advanced disease as we all continue to work to halt its progressive and debilitating effects on all affected individuals."

PTC announced in February 2009 that it has completed enrollment of a registration-directed clinical trial in patients with nonsense mutation Duchenne and Becker muscular dystrophy. The trial is a multi-center, randomized, double-blind, placebo-controlled study to determine whether ataluren can improve walking, activity, muscle function, and strength and whether the drug can safely be given for a long period of time. Results from this trial are expected to be available in the first half of 2010.

ABOUT DUCHENNE/ BECKER MUSCULAR DYSTROPHY
Duchenne and Becker muscular dystrophy (DBMD) are progressive, debilitating and life-threatening genetic disorders representing a single disease continuum with the same underlying cause. Patients with DBMD do not produce adequate levels of dystrophin, an essential protein that is critical to the structural stability of skeletal and heart muscles. DMD is the most common and most severe form of muscular dystrophy in children, and approximately 20,000 children worldwide are born with DMD annually (one of every 3,500 male children). Patients with BMD typically experience initial symptoms later and have a slower disease progression. Nonsense mutations cause about 10 to 15 percent of all DBMD cases which results in an estimated 1,900 boys and young men with nonsense mutation DBMD in the United States. Further information regarding DBMD is available through the Muscular Dystrophy Association (www.mda.org) or Parent Project Muscular Dystrophy (www.parentprojectmd.org).

ABOUT ATALUREN (PTC124®)
Ataluren is the first investigational new drug designed to restore the production of a functioning protein in patients with genetic disorders due to a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the

synthesis of an essential protein, such as dystrophin in the case of nmDBMD. Ataluren is currently being investigated for use in patients with nonsense mutation Duchenne and Becker muscular dystrophy (nmDBMD), nonsense mutation cystic fibrosis (nmCF) and nonsense mutation hemophilia A and B (nmHA/nmHB). Ataluren has been granted orphan drug status for the treatment of nmDBMD and nmCF by the U.S. Food and Drug Administration (FDA) and the European Commission. The FDA has also granted ataluren Subpart E and Fast Track designation for expedited development, evaluation, and marketing. The development of ataluren has been supported by the FDA Office of Orphan Products Development, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), and the National Center for Research Resources.

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 THE MUSCULAR DYSTROPHY ASSOCIATION
Founded in 1950, the Muscular Dystrophy Association is the nation's largest non-governmental funder of research seeking treatments and cures for more than 40 neuromuscular diseases, including muscular dystrophy, amyotrophic lateral sclerosis (ALS), spinal muscular atrophy (SMA), Charcot-Marie-Tooth disease (CMT), and Friedreich's ataxia (FA).

The first nonprofit organization to be recognized with a Lifetime Achievement Award from the American Medical Association ("for significant and lasting contributions to the health and welfare of humanity"), MDA also provides unparalleled health care services through its network of more than 200 hospital-affiliated clinics; advocates for the families it serves; and invests significant resources educating the medical and scientific communities, as well as the general public about neuromuscular diseases affecting more than 1 million Americans.

Thanks to decades of generous contributions from caring individuals, plus outstanding support received from local, regional and national sponsors, MDA is credited for its role in building the entire field of neuromuscular disease research, while simultaneously nurturing clinical care to significantly improve both quality and length of lives for those affected by neuromuscular diseases.

ABOUT PTC THERAPEUTICS
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 genetic disorders, oncology, and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and are 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.

FOR MORE INFORMATION: INVESTORS AND MEDIA

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