Data published in *Muscle and Nerve* validates the Six-Minute Walk Test as an Outcome Measure in Duchenne Muscular Dystrophy

South Plainfield, NJ – December 8, 2009 – Data published online in the medical journal *Muscle and Nerve* support the use of the six-minute walk test (6MWT) as an outcome measure in PTC Therapeutics, Inc.’s ongoing registration-directed clinical trial of ataluren in patients with nonsense mutation Duchenne/Becker muscular dystrophy (nmDMD/BMD). The study results characterize the limitations on walking by patients with DMD relative to healthy boys and indicate that young boys can consistently and reliably perform the test. The data were obtained from an observational study conducted at the University of California Davis which was sponsored by PTC and supported by a grant from Parent Project Muscular Dystrophy (PPMD).

The 6MWT is an accepted and standardized measure of muscle, lung, and heart health in patients with other medical conditions. However, the test had not previously been used to evaluate boys with DMD. The observational study was designed to confirm that boys with DMD would have the stamina and focus required to successfully and consistently complete the test without injury.

“Advancing the validation of clinically meaningful endpoints for therapeutic and natural history studies in DMD has been a focus of the Duchenne clinical and advocacy communities for many years,” said Craig McDonald, M.D., principal investigator of the observational study and director of the Rehabilitation Research and Training Center in Neuromuscular Diseases, University of California Davis. “The publication of these findings demonstrates that the 6MWT can serve as a clinically meaningful outcome measure for documenting the evolution of the disease and the treatment benefits of new therapies for DMD. The new test has rapidly become the favored primary outcome measure worldwide in clinical trials in ambulatory boys with Duchenne muscular dystrophy. This is an exciting moment for all of us involved in the care of boys with this progressively disabling condition.”
The study was designed to evaluate the feasibility, safety and reproducibility of the 6MWT, comparing boys with DMD to healthy boys of the same age. The study evaluated 21 ambulatory boys with DMD and 34 healthy boys, ages four to 12 years old. Boys with DMD were tested twice, one week apart and healthy boys were tested once. This study demonstrated that ambulatory young boys can complete the 6MWT safely and consistently.

“PPMD is proud to have sponsored research that sets the foundation for developing drugs in DMD. The validation of the 6MWT as a clinical endpoint has significant implications for the DMD community” stated Patricia Furlong, Founding President & CEO of Parent Project Muscular Dystrophy. “The ability to study the efficacy of potential DMD treatments will facilitate the advancement of new therapies and ultimately benefit patients. An important part of what we do at PPMD is to help establish universal standards for boys and young men with DMD. It is particularly gratifying to have a validated clinical endpoint to expand the development of therapeutic options.”

The study found that boys with DMD walked profoundly shorter distances in six minutes than healthy boys due to the muscular deficits that are characteristic of DMD, including markedly reduced stride length. The findings also suggest that the 6MWT is highly reproducible, as evidenced by a high degree of correlation ($r=0.91$) between the first and second test in boys with DMD. These findings directly support the use of the 6MWT in PTC’s ongoing Phase 2b, double-blind, randomized trial of ataluren in boys with nmDMD/BMD, for which the 6MWT is the primary outcome measure. The trial is designed to determine whether ataluren taken orally can improve walking, muscle function and strength in patients with nmDMD/BMD and will also evaluate ataluren’s long-term safety profile.

“We are encouraged that pretreatment data from 174 boys participating in our pivotal Phase 2b study of ataluren are consistent with the observational data and show that the 6MWT is safe and reproducible when performed repeatedly at multiple trial sites in many different countries,” said Langdon Miller, M.D., Chief Medical Officer of PTC. “We look forward to sharing the data from the ataluren clinical trial in nmDMD/BMD patients in early 2010.”
ABOUT DMD/BMD
Duchenne and Becker muscular dystrophy (DMD/BMD) are progressive muscle disorders that result in the loss of both muscle function and independence. Patients with DMD/BMD have increasing problems with walking as the disease progresses and eventually must depend upon assistive devices to maintain mobility. DMD/BMD is perhaps the most prevalent form of the muscular dystrophies and is the most common lethal genetic disorder diagnosed during childhood today. Each year, approximately 20,000 children worldwide are born with DMD (one of every 3,500 male children). It is estimated that one in ten DMD patients is likely to have a Becker presentation, a milder form of the disease that is associated with later manifestation of symptoms. Further information regarding DMD and BMD is available through the Muscular Dystrophy Association (www.mdausa.org) and Parent Project Muscular Dystrophy (www.parentprojectmd.org).

ABOUT ATALUREN (PTC124®)
Ataluren is the first investigational new drug designed to restore the formation 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. Ataluren is currently being investigated for use in patients with nmDMD/BMD, nonsense mutation cystic fibrosis (nmCF) and nonsense mutation hemophilia A and B (nmHA/HB). Ataluren has been granted orphan drug status for the treatment of nmCF and nmDMD/BMD by the U.S. Food and Drug Administration (FDA) and the European Commission. The FDA has also granted ataluren Subpart E designation for expedited development, evaluation, and marketing. The development of ataluren has been supported by the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), the FDA Office of Orphan Products Development, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, 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 will commercialize ataluren in the United States and Canada, while Genzyme will commercialize the product in other regions of the world.
ABOUT PTC THERAPEUTICS

PTC is a biopharmaceutical company focused on the discovery, development and commercialization 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 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 that 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 website at www.ptcbio.com.

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