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Observational Data from One-Year Follow-up Study Validate Six-Minute Walk Distance as an Outcome Measure in Duchenne and Becker Muscular Dystrophy

SOUTH PLAINFIELD, NJ – December 1, 2010 – Data published in the December issue of the medical journal Muscle and Nerve confirm the utility of six-minute walk distance (6MWD) as a clinically meaningful endpoint in dystrophinopathy, a disease continuum comprising Duchenne and Becker muscular dystrophy (DBMD). The data showed that boys with DBMD experience a significant decline in walking ability compared to healthy boys over one year, suggesting that slowing the loss of walking ability is a major treatment goal. The observational study, which provides the first longitudinal natural history data reflecting changes in 6MWD in patients with DBMD, was conducted at the University of California-Davis, sponsored by PTC Therapeutics and funded in part by a grant from Parent Project Muscular Dystrophy (PPMD).

"The data from the observational study show that the 6-minute walk test is a clinically meaningful outcome measure of walking ability in patients with Duchenne/Becker muscular dystrophy. Progressive loss of walking ability is a major disease manifestation that significantly impacts a patient's quality of life," stated Stuart Peltz, Ph.D., President and CEO of PTC Therapeutics. "The study results support our understanding of recently reported results from a Phase 2b clinical trial of ataluren in nonsense mutation Duchenne/Becker muscular dystrophy. In addition, the critical information provided by this study will help inform the design of future clinical trials in this disorder."

The six-minute walk test (6MWT) was originally developed as an integrated assessment of cardiac, respiratory, circulatory and muscular capacity, and it has been used as a primary outcome measure to support the registration of treatments for other neuromuscular disorders. In a prior short-term study, the 6MWT was established as a feasible, safe and reliable outcome measure in boys with DBMD. Because the short-term study did not provide longitudinal data, it was extended to assess changes in walking ability over a longer period.

RESULTS FROM THE OBSERVATIONAL STUDY
The goal of the one-year study was to determine the ability of the six-minute walk test (6MWT) to detect changes in walking ability in boys with DBMD and compare it to healthy boys of similar age, height and weight. Participants included 18 boys with DBMD and 22 healthy boys who had participated in the prior short-term study. At baseline, all participants were required to be 4 to 12 years of age and capable of walking 10 meters or more without assistive devices.

At 52 weeks, boys with DBMD experienced a loss of 57 meters in mean 6MWD compared with an improvement of 13 meters in healthy boys. The difference in average change in 6MWD between the two study arms was 70 meters ($p=0.037$). The decrease in 6MWD observed in boys with DBMD is consistent with results from a recently-reported registration-directed Phase 2b clinical trial of the investigational new drug ataluren in nonsense mutation DBMD, which showed that patients treated with placebo ($n=57$) experienced an average loss of 43 meters in 6MWD over 48 weeks.

The validity of change in 6MWD as a measure of disease progression was supported by the age-related pattern of changes over time. At baseline, boys with DBMD had significantly reduced walking ability compared to healthy boys at baseline. Though the data showed that young boys with or without the disorder may show improvements in 6MWD, these gains appear to be eclipsed by disease progression in boys with DBMD at about 7 years of age.

The results suggested high variability in disease progression, as measured by the 6MWT, among boys with DBMD. In healthy patients, the variability (standard deviation of the mean change in 6MWD) was 40 meters at one year. For boys with DBMD, variability was 83 meters among those who maintained ambulation and 104 meters among all boys at one year. Two boys with DBMD lost independent ambulation during the study. One boy was 10 years old and walked only 125 meters at baseline and was not receiving corticosteroids. The other boy was 9 years old and began the study with a 6MWD of 350 meters and received corticosteroids throughout the study.

"The 6-minute walk test has rapidly become the primary endpoint of choice for therapeutic trials involving ambulatory boys with Duchenne/Becker muscular dystrophy worldwide and these findings provide the first important longitudinal findings that will assist in the optimal design of these trials," said Craig McDonald, M.D., principal investigator of the observational study of outcome measures in DBMD and director of the NIDRR-funded Rehabilitation Research and Training Center in Neuromuscular Diseases, University of California Davis. "Importantly, the results showed that development-related improvements in walking ability in younger boys with DBMD were overtaken by the progressive muscle disorder as they grew older. Despite age-appropriate increases in height and weight and prevalent corticosteroid use, boys with DBMD experienced a significant loss of walking ability over one year. Treatments that can slow or prevent this progression will be of tremendous benefit in this form of muscular dystrophy."

ABOUT DYSTROPHINOPATHY
Dystrophinopathy is a disease continuum comprising Duchenne and Becker muscular dystrophy (DBMD). Primarily affecting males, DBMD is progressive muscle disorder 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).

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 is the basis for collaborations with leading biopharmaceutical companies. For more information, visit the company's web site at www.ptcbio.com.

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