



PTC THERAPEUTICS ANNOUNCES DATA SHOWING SIX-MINUTE WALK TEST CONSISTENTLY MEASURES AMBULATORY FUNCTION IN PATIENTS WITH DUCHENNE MUSCULAR DYSTROPHY

-Findings Suggest Clinically Meaningful Primary Endpoint for Clinical Trials; Data Presented at the 13th International World Muscle Society Congress -

SOUTH PLAINFIELD, NJ – September 30, 2008 – PTC Therapeutics, Inc. (PTC) today announced promising findings in patients with Duchenne/Becker Muscular Dystrophy (DMD/BMD) at the 13th International World Muscle Society (WMS) Congress. Results from an observational study assessing the utility of the 6-minute walk test (6MWT) as a primary outcome measure in trials of treatment for DMD/BMD showed that the 6MWT clearly differentiates boys with DMD from healthy boys, especially when adjusted for age. The findings also demonstrate that the 6MWT provides a reliable assessment of ambulatory function in boys as young as five years of age, offering a practical and clinically meaningful outcome measure for use in DMD/BMD registration-directed clinical trials. Results from the study were presented today by Craig McDonald, M.D., principal investigator and director of the Rehabilitation Research and Training Center in Neuromuscular Diseases, University of California Davis.

There has been a strong interest in establishing clinically meaningful outcomes measures to advance the development of new treatments for DMD/BMD. The 6MWT is an accepted and standardized measure of ambulatory capacity that was developed to provide an integrated assessment of cardiac, respiratory, circulatory and muscular capacity. Until this study, it had been unclear whether boys with DMD would have the stamina and mental focus to successfully finish the test without injury. This study demonstrated that ambulatory boys with DMD as young as five years old can complete the 6MWT safely and reliably with minimal interruption from falls. The study found that the muscular deficits that are characteristic of boys with DMD impair their stride length and cadence, resulting in shorter 6-minute walk distances than healthy boys. The data correlate well with the known natural history of DMD and suggest that the test should be able to detect treatment effects on DMD-related muscular deficits.

"The 6MWT evaluates the critical human function of ambulation and is highly relevant to patients with DMD, who progressively lose walking ability during childhood," said Dr. McDonald. "Establishing a gold-standard outcome measure for new therapies in DMD has long been a goal among researchers. These findings allow us to confidently use changes in the distance achieved during the 6MWT as a major efficacy endpoint in therapeutic trials."

These findings directly support the design of an ongoing phase 2b, double-blind, randomized trial of PTC124 in boys with DMD/BMD, in which the 6MWT is the primary outcome measure. Demographic and baseline data from this study are also being presented at the WMS Congress today. This multicenter, international study is enrolling 165 ambulatory boys who are at least five years old. Today's presentation reports baseline data on the first 43 subjects, who range in age from 5 to 17 years. Using the 6MWT, subjects were tested and then retested up to six weeks later; the correlation between test and retest was high ($r=0.93$), further demonstrating the reliability of the 6MWT. In addition to the ongoing phase 2b study, PTC124 is being evaluated in an open-label, long-term extension study open to boys who participated in an earlier phase 2a trial.

"The findings from these clinical trials contribute provide strong underpinnings for the design of the PTC124 clinical trials program," said Langdon Miller, M.D., Chief Medical Officer of PTC Therapeutics, Inc. "We are hopeful that these data can benefit the entire field of DMD clinical research and therapeutics development and can establish regulatory precedent of use of the 6MWT in DMD/BMD"

The study was made possible due to funding provided by the Parent Project Muscular Dystrophy (PPMD). In an effort to maximize the utility of the study findings, the PPMD also sponsored the development of a video demonstrating the 6MWT. The video demonstrates how to perform the test, and will ensure consistency across clinical trials and will serve as a resource for clinics using the 6MWT to follow the progress of boys with DMD/BMD.

"Identifying and promoting a clinically meaningful endpoint with proven utility has been an important goal of our organization. We are thrilled to be able to help disseminate information about the 6MWT widely, so it can be quickly and readily integrated into clinical trial protocols," said Pat Furlong, President and CEO of PPMD.

ABOUT DMD/BMD

Duchenne and Becker muscular dystrophy (DMD/BMD) are progressive muscle disorders that cause the loss of both muscle function and independence. DMD/BMD is perhaps the most prevalent 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 10 DMD patients are likely to have a Becker presentation, a milder form of the disease that is associated with later manifestation of symptoms. In essence, DMD and BMD represent a continuum of the same disease. More information regarding DMD and BMD is available through the Muscular Dystrophy Association (www.mdausa.org), Parent Project Muscular Dystrophy (www.parentprojectmd.org) and the Association Française contre les Myopathies (www.afm-france.org).

ABOUT PTC124

PTC124 is an orally delivered, investigational new drug discovered by PTC Therapeutics. The drug is being developed for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely stop the translation process, leading to production of truncated, non-functional proteins. PTC124 induces the cellular translation machinery to read through nonsense mutations, inducing production of full-length, functional proteins. PTC124 has demonstrated proof of concept in phase 2a clinical trials. Across all clinical studies to date, PTC124 has been generally well tolerated. PTC124 is currently in phase 2b development with the goal of demonstrating that increasing functional protein levels in patients with nonsense-mediated genetic disorders will safely provide clinical benefits.

PTC124 has been granted orphan drug status by the FDA and the European Commission for the treatment of cystic fibrosis and DMD due to nonsense mutations. The FDA has also granted PTC124 Subpart E designation for expedited development, evaluation, and marketing.

Genzyme Corporation has an exclusive collaboration with PTC to develop and commercialize PTC124 outside the U.S. and Canada. The development of PTC124 has also been supported by grants from the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, FDA's Office of Orphan Products Development, the National Center for Research Resources and notably, the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), which recently expanded support of PTC124 to include funding up to \$25 million.

ABOUT PTC THERAPEUTICS INC.

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 extensive knowledge of post-transcriptional control processes and has developed proprietary technologies that it applies in its drug discovery activities, including the Gene Expression Modulation by Small-molecules (GEMS) technology, which has been the basis for collaborations with leading biopharmaceutical companies such as Genzyme, Pfizer, Celgene, CV Therapeutics and Schering-Plough. For more information, visit the company's website www.ptcbio.com.

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