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NEW EVIDENCE PUBLISHED IN MUSCLE AND NERVE ON MEASURING 6-MINUTE WALK DISTANCE IN DUCHENNE MUSCULAR DYSTROPHY

-Two publications further validate the use of 6MWT as a primary endpoint in Duchenne Muscular Dystrophy (DMD) clinical trials-

SOUTH PLAINFIELD, NJ – August 26, 2013 – Data published in the medical journal Muscle & Nerve demonstrate the clinical meaningfulness of the 6-minute walk test (6MWT) as a primary endpoint to measure disease progression and walking ability in ambulatory Duchenne muscular dystrophy (DMD) trials. The research also showed that a range of 20 to 30 meters in walking ability is a clinically meaningful change, as measured by 6-minute walk distance (6MWD). This analysis is based on natural history data obtained from PTC Therapeutics' Phase 2b trial of ataluren in 174 patients with nonsense mutation Duchenne muscular dystrophy (nmDMD), the first placebo-controlled, multi-national study of a new chemical entity for DMD.

"Given that several novel approaches to the treatment of Duchenne muscular dystrophy have shown promise in preclinical and/or proof-of-concept clinical studies, the research community has faced the need to identify and develop clinically meaningful outcome measures for use in pivotal therapeutic trials. In boys with DMD, walking abnormalities are a major disease manifestation that have great importance to patients and their families," stated Dr. Craig McDonald, one of the world's leading experts in muscular dystrophy clinical endpoints and DMD natural history studies and lead author of the two publications. "We pioneered the development of the 6MWT for DMD with PTC in conjunction with the ataluren trials and I am pleased to have collaborated with the ataluren study group to demonstrate the 6MWT as a reliable, valid, and meaningful endpoint for Duchenne muscular dystrophy." Dr. McDonald added, "The data from the ataluren placebo group has recently been replicated in other natural history studies conducted by investigators in Belgium and Italy. All three data sets show a similar change in 6-minute walk distance over one year as a measure of disease progression."

The 6MWT was originally developed as an integrated assessment of cardiac, respiratory, circulatory and muscular capacity, and it has previously been used as a primary outcome in a number of clinical studies to support the regulatory approval of treatments for a number of other neuromuscular disorders. In a prior short-term study (McDonald et al 2010), the 6MWT was established as a feasible, safe and reliable outcome measure of walking ability in boys with DMD, who have not yet transitioned to full-time wheelchair use. A follow-up longitudinal study concluded that changes in 6MWD depend on stride length and age. Improvements in 6MWD were observed in both healthy subjects and patients with DMD <7 years of age, attributed to maturational development. In contrast, the walking ability of older DMD patients (≥7 years of age) worsened, while the walking ability of older healthy subjects (≥7 years of age) tended to either increase or remain stable. Subsequent studies have confirmed this observation and also demonstrated that 6MWD correlates with other clinical endpoints in DMD, such as timed function tests and the North Star Ambulatory Assessment (NSAA), as well as established patient-reported outcomes of physical function.

"In our Phase 2b Duchenne muscular dystrophy trial, we did not have any natural history to guide our inclusion criteria. The patient population was quite heterogeneous ranging in age from 5 to 20 years old and the baseline 6-minute walk distance ranged from 75 meters to 554 meters," stated Stuart W. Peltz, Ph.D., Chief Executive Officer at PTC Therapeutics, Inc. "Armed with a better understanding of the natural history of the disease, the enrollment criteria for our ongoing Phase 3 trial now enriches the patient population that are in the decline phase of walking ability and would best demonstrate benefit in a 48-week trial. When we looked back at the subgroup of 61 patients from our completed Phase 2b trial that fit the inclusion criteria of the current trial, they showed an average 50 meter improvement in ambulation as measured in the 6-minute walk distance from patients on placebo. We anticipate that the Phase 3 trial will complete enrollment mid-2014."

This report in Muscle & Nerve represents an evaluation of the largest multicenter dataset collected to date to determine the reliability and concurrent validity of the 6MWT and other clinical endpoints in DMD. In addition, the research assesses the distribution-based MCID (minimal clinically important difference) for 6MWD and other commonly-employed functional endpoints (eg. timed function tests and quantitative knee extension strength measures) as well as the 6MWD, which is the most common primary endpoint for ambulatory DMD clinical trials. Moreover, baseline walking ability, as measured by the 6MWD was found to be predictive of time to ≥10% worsening in function, a clinically meaningful milestone, in DMD.

The research findings confirm that the 6MWT is a safe and feasible measure of walking ability in DMD and a valid primary outcome measure for ambulatory DMD clinical trials. In addition, the data supports the clinically meaningful change in walking ability, as measured by 6MWT, to be in the range of 20 to 30 meters, which can serve as a targeted treatment effect in 12-month trials in ambulatory DMD.

ABOUT THE PUBLICATIONS

The natural history data were derived from the placebo arm of a Phase 2b, international, multicenter, randomized, double-blind, placebo-controlled, dose-ranging study to evaluate the efficacy and safety of ataluren in ambulatory male patients with nonsense mutation DMD aged ≥5 years. The other publication analyzes the baseline data from all DMD patients in the same Phase 2b study. The study comprised a 6-week screening period and a 48-week blinded study drug treatment period. Patients were randomized 1:1:1 to receive: ataluren at either high- or low-dose or matching placebo, daily for 48 weeks. Evaluations were performed at screening, baseline, and every 6 weeks for 48 weeks.

There were 174 randomized DMD patients included in the study. All patients screened and randomized were males, ranging in age from 5 to 20 years. Nonsense mutations were distributed across the span of the 79 exons of the dystrophin gene, with no mutational hotspots identified, and represented all three types of premature stop codons. Test-retest reliability was determined using the baseline and screening values for all endpoints. Concurrent validity and MCIDs were determined using pre-treatment data.

ABOUT DUCHENNE MUSCULAR DYSTROPHY (DMD)

Primarily affecting males, Duchenne muscular dystrophy (DMD) is a 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. There are an estimated 34,000 patients with DMD in the United States and Europe and approximately 13 percent of all DMD cases are caused by nonsense mutations in the dystrophin gene. More information about DMD is available through the Muscular Dystrophy Association (www.mdausa.org), Parent Project Muscular Dystrophy (www.parentprojectmd.org), Action Duchenne (www.actionduchenne.org), United Parent Projects Muscular Dystrophy (uppmd.org), Muscular Dystrophy Campaign (www.muscular-dystrophy.org) and AFM (l'Association française contre les myopathies), (www.afm-telethon.fr).

ABOUT PTC THERAPEUTICS, INC. (NASDAQ:PTCT)

PTC is biopharmaceutical company focused on the discovery and development of orally-administered, proprietary small molecule drugs that target post-transcriptional control processes. While PTC's discovery programs are directed at targets in multiple therapeutic areas, PTC is focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. PTC's internally-discovered pipeline addresses multiple therapeutic areas, including neuromuscular disorders, oncology and infectious diseases. For more information on the company, please visit our website www.ptcbio.com.

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FORWARD LOOKING STATEMENTS:

Any statements in this press release about future expectations, plans and prospects for PTC, including statements about enrollment and conduct of PTC's Phase 3 clinical trial of ataluren for nmDMD, the clinical meaningfulness of the 6MWT for regulatory purposes and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" section of the final prospectus for our initial public offering, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent PTC's views only as of the date of this release. PTC anticipates that subsequent events and developments will cause its views to change. However, while PTC may elect to update these forward-looking statements at some point in the future, PTC specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing PTC's views as of any date subsequent to the date of this release.