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Betreff: PTC Therapeutics Announces Initiation of Phase 2b Registration-Directed Clinical Trial of PTC124 in Duchenne/Becker Muscular Dystrophy

PTC THERAPEUTICS ANNOUNCES INITIATION OF PHASE 2B REGISTRATION-DIRECTED CLINICAL TRIAL OF PTC124 IN DUCHENNE/BECKER MUSCULAR DYSTROPHY

First Registration Study of an Investigational Drug for Duchenne/Becker Muscular Dystrophy

SOUTH PLAINFIELD, NJ – April 23, 2008 - PTC Therapeutics, Inc. (PTC), today announced the initiation of an international pivotal trial of PTC124 in patients with Duchenne/Becker muscular dystrophy (DMD/BMD) due to a nonsense mutation. The primary objective of this registration-directed Phase 2b trial is to demonstrate the efficacy of PTC124 as measured by improvements in the walking ability of patients with this progressive genetic disease.

"DMD/BMD is a disorder with a significant need for better treatment options, and we are very encouraged by the promising results we have seen to date with PTC124," said Brenda Wong, M.D., Associate Professor of Pediatrics and Neurology, Cincinnati Children's Hospital Medical Center, Cincinnati, who was involved in the Phase 2a study and is one of the trial's lead investigators. "We believe that the safety profile of PTC124 and activity we have seen in the Phase 2a studies clearly support the initiation of this longer-term, registration-directed efficacy and safety study. We are very pleased to be a part of this groundbreaking trial."

Patients with DMD and BMD are boys and young men who lack dystrophin, a protein that is critical to the structural stability of muscle fibers. Patients develop progressive muscle weakness that leads to loss of ambulation, wheelchair dependency, and eventual decline in respiratory and cardiac function. 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.

PTC124 is a novel, orally delivered drug in development for the treatment of patients with genetic disorders due to a nonsense mutation, a type of mutation found in approximately 13% of patients with DMD. In this double-blind study, patients will be randomized to receive placebo, or one of two dose levels of PTC124, three times per day. Eligible patients will be boys with nonsense-mutation-mediated DMD/BMD who are at least 5 years of age and are able to walk at least 75 meters or approximately 80 yards in six minutes. PTC expects to enroll a total of 165 patients at approximately 35 investigational sites; all study subjects will undergo 48 weeks of blinded treatment. Thereafter, all participants, including those who have been receiving placebo, will be eligible to enroll in an open-label PTC124 extension study.

The primary outcome measure is the total distance walked during a 6-minute walk test, a test of ambulation that has now been standardized for boys with DMD/BMD through a collaboration with noted investigator, Craig McDonald, M.D., at University of California at Davis. Other outcome measures in the Phase 2b study will evaluate activity at home, muscle and heart function, strength, cognitive ability, muscle integrity, and muscle dystrophin expression. Safety parameters, compliance, and PTC124 blood levels also will be monitored.

"We are very pleased to announce the initiation of the Phase 2b trial for PTC124 in boys with DMD/BMD," said Langdon Miller, M.D., Chief Medical Officer of PTC. "We applaud the patients, parents, and clinicians who have committed themselves to this effort. The design of this trial reflects our ongoing collaboration with the advocacy community, investigators at leading neuromuscular centers, and the U.S. and European regulatory agencies. We hope that PTC124 will soon offer a treatment that addresses the underlying cause of the disease for patients with nonsense-mediated DMD/BMD and that the development of PTC124 will set the stage for improving therapeutic options in this disabling and life-threatening disorder."

Stuart W. Peltz, Ph.D., President and Chief Executive Officer of PTC Therapeutics added, "Initiation of the Phase 2b trial is an important milestone for PTC. The trial builds on the results we have achieved to date in DMD and cystic fibrosis (CF) and constitutes a major step forward in establishing the potential for PTC124 as a paradigm shift in the treatment of genetic disorders. Our future plans for PTC124 include the initiation of longer-term studies in CF, as well as additional proof-of-concept studies in other indications."

ABOUT DUCHENNE AND BECKER MUSCULAR DYSTROPHY

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), the 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 in Phase 2 clinical development for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non-functional protein. PTC124 has restored production of full-length, functional proteins in preclinical genetic disease models harboring nonsense mutations. In Phase 1 clinical trials, PTC124 was generally well tolerated, achieved target plasma concentrations that have been associated with activity in preclinical models and did not induce ribosomal read through of normal stop codons. PTC124 has demonstrated pharmacodynamic proof of concept in Phase 2a clinical trials in nonsense-mutation-mediated Duchenne muscular dystrophy (DMD) and cystic fibrosis (CF).

It is estimated that 13% of the cases of DMD and 10% of the cases of CF are due to nonsense mutations. PTC believes that PTC124 is potentially applicable to a broad range of other genetic disorders in which a nonsense mutation is the cause of the disease. The FDA has granted PTC124 Subpart E designation for expedited development, evaluation, and marketing and has granted Orphan Drug designations for the treatment of CF and DMD due to nonsense mutations. PTC124 has also been granted orphan drug status for the treatment of CF and DMD by the European Commission. PTC124's development has been supported by grants from the Muscular Dystrophy Association (MDA), Cystic Fibrosis Foundation Therapeutics, Inc. (CFFT), Parent Project Muscular Dystrophy (PPMD), FDA's Office of Orphan Products Development (OOPD) and by General Clinical Research Center grants from the National Center for Research Resources (NCRR). For additional information on the PTC124 clinical trial, please visit www.clinicialtrials.gov and search using the keyword: PTC124.

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

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