wahlig

Von: PTC_Therapeutics [PTC_Therapeutics@ptcbio.com]
Gesendet: Freitag, 30. Mai 2008 12:31
An: wahlig@t-online.de
Betreff: PTC124 Featured at Third Annual Congress of Myology

PTC124 FEATURED AT THIRD ANNUAL CONGRESS OF MYOLOGY

-- PTC Announces Initiation of Additional Phase 2b Clinical Trial Sites in Europe --

SOUTH PLAINFIELD, NJ- May 30, 2008- PTC Therapeutics, Inc. (PTC) today announced that PTC124, the company's orally delivered investigational new drug for the treatment of genetic disorders due to nonsense mutations, was featured in a symposium at Myology 2008, the Third Annual Congress of Myology, on Friday, May 30 in Marseilles. The Congress serves as the annual meeting of The Association Française contre les Myopathies (French Muscular Dystrophy Association) and focuses on research, therapies and clinical trials for various forms of muscular dystrophy.

PTC124 is being studied in Duchenne muscular dystrophy (DMD), a progressive muscle disorder in which patients lack dystrophin, a protein that is critical to the structural stability of muscle fibers. PTC recently launched a global, registration-directed Phase 2b trial in DMD and Becker muscular dystrophy (BMD) to evaluate the efficacy of PTC124 as measured by improvements in the walking ability of patients with this progressive genetic disease.

Data from the previous Phase 2a study were presented and discussed by a panel of leading physicians and researchers, including: Dr. Thomas Voit, M.D., Medical and Scientific Director of the Myology Institute and Dr. Richard Finkel, M.D., Director of the Neuromuscular Program, Children's Hospital of Philadelphia, PA.

"PTC124 represents a promising new therapy for DMD/BMD, as there are currently no available treatments that address the underlying cause of this disease," stated Dr. Voit. "The Phase 2b PTC124 clinical trial sets a gold standard for future clinical trials in muscular dystrophies. I am delighted to be part of these groundbreaking studies."

Presenters at the symposium also announced that the Phase 2b trial of PTC124 was recently initiated in France, Belgium and Sweden and will begin enrolling patients in additional European sites in the coming months, broadening the reach of the clinical development program.

Genetic disorders, such as DMD and cystic fibrosis (CF), are caused by genetic alterations, known as mutations. By targeting a specific type of genetic alteration – nonsense mutations – PTC124 bypasses the defect and leads to the restoration of a functional protein. The company has catalogued over 2,400 distinct genetic disorders for which nonsense mutations are the cause of the disease in a significant percentage of patients. Nonsense mutations inactivate gene function and are known to cause anywhere from five to 70 percent of the individual cases of most of these inherited diseases.

In preclinical models of genetic diseases harboring nonsense mutations, PTC124 was shown to regulate post-transcriptional control processes by restoring full-length, functional proteins. Post-transcriptional control processes are the cellular regulatory events that take place after an RNA molecule is copied from DNA. These processes are critical to proper cellular function and provide an opportunity for therapeutic intervention in a broad range of genetic disorders through the modulation of protein levels.

"We are pleased to have the opportunity to share our exciting clinical progress regarding PTC124 with the myology community," said Langdon Miller, M.D., Chief Medical Officer of PTC. "Through its specificity and novel mechanism of action, PTC124 has shown potential to address a broad range of genetic disorders due to nonsense mutations. We look forward to continuing to advance our clinical programs in DMD and CF and evaluating PTC124 in a number of additional nonsense-mutation-mediated genetic disorders."

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 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 stop 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).

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.clinicaltrials.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.

FOR MORE INFORMATION:

INVESTORS AND MEDIA

Jane Baj
PTC Therapeutics, Inc.
(908) 912-9167
jbaj@ptcbio.com

Sheryl Seapy
Pure Communications
(949) 608-0841
sheryl@purecommunicationsinc.com

PATIENTS, PATIENTS' FAMILIES, INVESTIGATORS AND PATIENT ORGANIZATIONS

Diane Goetz
PTC Therapeutics, Inc.
(908) 912-9256
patientinfo@ptcbio.com

If you would like to be removed from this mailing list, please email info@ptcbio.com and write remove in the subject field.