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**PTC Therapeutics Announces Pharmacokinetic and Safety Results from  
Phase 2 Study of PTC124 in Duchenne Muscular Dystrophy**

*- Data Presented at 36<sup>th</sup> Child Neurology Society Annual Meeting -*

**SOUTH PLAINFIELD, NJ – October 11, 2007** - PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced pharmacokinetic and safety data from a Phase 2 clinical trial of PTC124 in patients with Duchenne muscular dystrophy (DMD) due to a nonsense mutation. The results, which include data from the third and final cohort of the study, show that treatment with PTC124 appeared well tolerated at all three dose levels and target plasma concentrations were achieved at the mid- and high-dose levels. These data were presented today at the 36<sup>th</sup> Annual Meeting of the Child Neurology Society (CNS) in Quebec City, Canada.

In the study, patients received 28 days of PTC124 treatment at one of three dose levels. All clinical trial participants are boys with a nonsense mutation in the dystrophin gene, substantially elevated serum creatine kinase levels, and symptoms associated with DMD. The analysis presented today showed that PTC124 appeared well tolerated among the 38 boys included in the study. Adverse events were infrequent, mild to moderate in severity, and did not result in therapy interruptions or discontinuations. There were no concerns based on physical examinations, vital sign measurements, electrocardiograms or laboratory parameters. Compliance with PTC124 treatment was excellent at all three dose levels. Target plasma concentrations associated with activity in a preclinical model of DMD were achieved at the mid- and high-dose levels.

“DMD is a disorder with a significant need for better treatment options and we are encouraged by the 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, OH, who presented the data today at CNS and is one of the trial's lead investigators. “Based on the findings from this study, we believe that the safety profile of PTC124 supports continued testing in longer-term studies.”

“These results add to the growing body of safety data for PTC124, which has now been evaluated in more than 150 subjects, including patients with both DMD and cystic fibrosis. The safety profile has consistently shown that PTC124 appears well tolerated,” said Langdon Miller, M.D., Chief Medical Officer of PTC. “We are looking forward to presenting additional activity data from this study next week at the World Muscle Society meeting in Italy.”

### **About Duchenne Muscular Dystrophy**

Duchenne muscular dystrophy (DMD) is a progressive muscle disorder that causes the loss of both muscle function and independence. DMD 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). More information regarding DMD is available through the Muscular Dystrophy Association ([www.mdausa.org](http://www.mdausa.org)) and the Parent Project Muscular Dystrophy ([www.parentprojectmd.org](http://www.parentprojectmd.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. PTC is currently conducting Phase 2 clinical trials of PTC124 in nonsense-mutation-mediated cystic fibrosis (CF) and Duchenne muscular dystrophy (DMD).

It is estimated that 10% of the cases of CF and 13% of the cases of DMD 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 Fast-Track designations and 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 is 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).

**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:**

Jane Baj

PTC Therapeutics, Inc.

(908) 222-7000, x167

[jbaj@ptcbio.com](mailto:jbaj@ptcbio.com)

Sheryl Seapy

Pure Communications

(949) 608-0841

[sheryl@purecommunicationsinc.com](mailto:sheryl@purecommunicationsinc.com)