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## **PTC THERAPEUTICS INITIATES OPEN-LABEL STUDY FOR ATALUREN IN PATIENTS WITH NONSENSE MUTATION DUCHENNE/BECKER MUSCULAR DYSTROPHY**

**SOUTH PLAINFIELD, NJ – June 27, 2012** – PTC Therapeutics, Inc. (PTC) today announced the initiation of an open-label study in the European Union, Israel, Australia and Canada for patients with nonsense mutation Duchenne/Becker muscular dystrophy (nmDBMD) who received ataluren in a prior, PTC-sponsored clinical study. The primary objective of this study is to gain further information on the long-term safety and tolerability of ataluren, an investigational new drug. PTC launched a similar study in the United States in November 2010.

"The initiation of this open-label study is based on the potential ataluren has shown in previous studies in nonsense mutation Duchenne/Becker muscular dystrophy," stated Stuart W. Peltz, PhD, CEO of PTC Therapeutics. "We are encouraged by the results we have obtained in our Phase 2b study and are committed to the continued advancement of the development of ataluren."

Data from PTC's 48-week Phase 2b study in nmDBMD show that patients treated with ataluren (10-, 10-, 20-mg/kg) walked on average 30 meters further than patients on placebo, as measured by the change in 6-minute walk distance (6MWD) from baseline to Week 48. This result is consistent with the study hypothesis of a 30-meter difference and in the average change in 6MWD observed in registration-directed trials of approved drugs for other diseases. Patients receiving ataluren also had a slower rate of decline in ambulation, based on an analysis of time to 10% worsening in 6MWD. Positive trends in muscle function, as measured by timed function tests, were observed in patients treated with ataluren when compared to patients treated with placebo. Ataluren was generally well tolerated. Serious adverse events were infrequent and none were considered to be related to ataluren.

"As a representative of the patient community, I applaud PTC for taking this initiative to ensure that patients from prior ataluren trials continue to have access to this promising therapy," stated Elizabeth Vroom, president of the Dutch Duchenne Parent Project and the chair of the worldwide United Parent Projects Muscular Dystrophy (UPPMD). "Patients and families who previously participated in the Phase 2b trial are very eager to have access to ataluren again based on their experience on the drug. Now patients previously treated with ataluren in other countries will have the same opportunity as those in the U.S. to again receive ataluren. All other families with sons with a premature stop hope this open-label trial will help make ataluren available for the larger community."

PTC Therapeutics has worldwide development and commercial rights for ataluren in all indications and territories.

### **ABOUT THIS TRIAL**

The primary objective of this open-label study is to assess the long-term safety and tolerability of ataluren in patients with nonsense mutation Duchenne/Becker muscular dystrophy who had prior exposure to ataluren in a PTC sponsored clinical trial. Secondary objectives include specific efficacy measures. The study will include up to 96 patients, 21 sites and 10 countries. Patients will receive ataluren 3 times per day (10, 10, 20 mg/kg), for approximately 48 weeks.

### **ABOUT DUCHENNE/BECKER MUSCULAR DYSTROPHY**

Primarily affecting males, Duchenne/Becker muscular dystrophy (DBMD) is 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. A smaller subset is classified as having Becker muscular dystrophy, a milder variation of the disorder that is associated with later manifestation of symptoms. About 10 to 15 percent of all DBMD cases are caused by nonsense mutations in the dystrophin gene. There are an estimated 1,700 and 2,200 patients with nmDBMD in the United States and Europe, respectively. More information about DBMD is available through the Muscular Dystrophy Association ([www.mdausa.org](http://www.mdausa.org)), Parent Project Muscular Dystrophy ([www.parentprojectmd.org](http://www.parentprojectmd.org)), Action Duchenne ([www.actionduchenne.org](http://www.actionduchenne.org)), United Parent Projects Muscular Dystrophy ([uppm.org](http://uppm.org)) and AFM (l'Association française contre les myopathies), ([www.afm-telethon.fr](http://www.afm-telethon.fr)).

### **ABOUT ATALUREN**

Ataluren, an investigational new drug discovered and developed by PTC Therapeutics, is a protein restoration therapy

designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in nmDBMD. The development of ataluren has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); Muscular Dystrophy Association; FDA's Office of Orphan Products Development; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

**ABOUT PTC THERAPEUTICS, INC.**

PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered 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 rare genetic disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and that have served as the basis for collaborations with leading biopharmaceutical companies such as AstraZeneca, Celgene, Genzyme, Merck, Pfizer and Roche. For more information, visit the company's website at [www.ptcbio.com](http://www.ptcbio.com).

**FOR MORE INFORMATION:**

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

Sheryl Seapy  
Pure Communications  
(949) 608-0841  
[sheryl@purecommunicationsinc.com](mailto:sheryl@purecommunicationsinc.com)