



## **PTC THERAPEUTICS AND GENZYME CORPORATION ANNOUNCE PRELIMINARY RESULTS FROM THE PHASE 2B CLINICAL TRIAL OF ATALUREN FOR NONSENSE MUTATION DUCHENNE/BECKER MUSCULAR DYSTROPHY**

**SOUTH PLAINFIELD, NJ and CAMBRIDGE, MA – March 03, 2010** – PTC Therapeutics, Inc. and Genzyme Corporation (Nasdaq: GENZ) today announced preliminary results from the Phase 2b clinical trial of ataluren, an investigational new drug, in patients with nonsense mutation Duchenne/Becker Muscular Dystrophy (nmDBMD). The primary endpoint of change in 6-minute walk distance did not reach statistical significance within the 48 week duration of the study. Study results showed that ataluren was well tolerated and no clinical trial patients discontinued treatment due to an adverse event. Additional efficacy analyses are underway in patient subgroups.

"These results further demonstrate the safety profile of ataluren and support continued development," remarked Langdon Miller, M.D., PTC's chief medical officer. "DBMD is a progressive, debilitating, and life-threatening neuromuscular disorder. The variability of symptom onset, disease progression, and 6-minute walk distance creates challenges for clinical development. Importantly, this trial does provide a wealth of valuable data about ataluren and DBMD. Additional analyses will guide the overall clinical and regulatory path forward."

"PTC has a longstanding commitment to discovering and developing new treatments for DBMD and we will continue to collaborate with patients, investigators, and DBMD advocacy groups to advance these efforts," stated Stuart Peltz, Ph.D., president and chief executive officer of PTC Therapeutics. "Data from this Phase 2b study will guide further development of ataluren in nonsense mutation genetic disorders."

"The quality of the data from this well-conducted study and additional analyses will help to inform the clinical development of ataluren in other indications," stated Geoffrey McDonough, M.D., senior vice president and general manager of Genetic Diseases at Genzyme. "We are committed to the development of ataluren and will continue to collaborate with PTC to advance its development for the treatment of genetic disorders."

Ataluren is also currently being investigated for use in patients with nonsense mutation cystic fibrosis in a Phase 3 study and nonsense mutation hemophilia A and B in a Phase 2a study. Ataluren's mechanism of action offers the potential to address multiple genetic disorders with various pathophysiologies and disease manifestations.

### **ABOUT THE PHASE 2B CLINICAL TRIAL**

The randomized, double-blind, placebo-controlled Phase 2b trial was designed to evaluate the safety and efficacy of 48 weeks of ataluren therapy in patients with nmDBMD. The study enrolled 174 participants at 37 sites in North America, Europe, Australia, and Israel. Participants were randomized to receive either a low dose of ataluren (10 mg/kg in the morning, 10 mg/kg at midday, and 20 mg/kg in the evening), a high dose of ataluren (20 mg/kg in the morning, 20 mg/kg at midday, and 40 mg/kg in the evening), or placebo (inactive drug in the morning, at midday, and in the evening). The primary outcome measure was the total distance walked during a 6-minute walk test, a standardized test of ambulation. Other outcome measures in the study evaluated activity at home, muscle and heart function, strength, cognitive ability, muscle integrity, and muscle dystrophin expression. Safety parameters, compliance, and ataluren blood levels were also monitored.

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

Duchenne and Becker muscular dystrophy (DBMD) are progressive muscle disorders 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, may 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 1,900 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)), the Parent Project Muscular Dystrophy ([www.parentprojectmd.org](http://www.parentprojectmd.org)), and the Association Française contre les Myopathies (AFM – [www.afm-france.org](http://www.afm-france.org)).

### **ABOUT ATALUREN**

An investigational new drug discovered by PTC Therapeutics, ataluren 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. Ataluren is currently being investigated for use in patients with genetic disorders due to a nonsense mutation.

PTC Therapeutics and Genzyme Corporation formed an exclusive collaboration in 2008 to develop and commercialize ataluren worldwide. PTC Therapeutics will commercialize the product in the United States and Canada, while Genzyme will commercialize ataluren in other regions of the world. The development of ataluren has also been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); FDA's Office of Orphan Products Development; Muscular Dystrophy Association; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

#### **ABOUT PTC THERAPEUTICS**

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 is the basis for collaborations with leading biopharmaceutical companies such as Celgene, Genzyme, Gilead, Merck, Pfizer and Roche. For more information, visit the company's web site at [www.ptcbio.com](http://www.ptcbio.com).

#### **ABOUT GENZYME**

One of the world's leading biotechnology companies, Genzyme is dedicated to making a major positive impact on the lives of people with serious diseases. Since 1981, the company has grown from a small start-up to a diversified enterprise with more than 12,000 employees in locations spanning the globe and 2009 revenues of \$4.5 billion. With many established products and services helping patients in approximately 100 countries, Genzyme is a leader in the effort to develop and apply the most advanced technologies in the life sciences. The company's products and services are focused on rare inherited disorders, kidney disease, orthopaedics, cancer, transplant and immune disease, and diagnostic testing. Genzyme's commitment to innovation continues today with a substantial development program focused on these fields, as well as cardiovascular disease, neurodegenerative diseases, and other areas of unmet medical need.

#### **GENZYME SAFE HARBOR STATEMENT**

This press release contains forward looking statements regarding Genzyme's future business plans and strategies, including without limitation, statements regarding ataluren's clinical development, ataluren's potential in indications other than nmDBMD and Genzyme's plans to advance ataluren's development for the treatment of various genetic disorders. These forward looking statements are subject to risks and uncertainties that may cause actual results to differ materially from those forecasted. These risks and uncertainties include, among others: that additional analysis of data from the trial is not sufficient to inform the development plan for ataluren; that Genzyme or PTC does not pursue the development of ataluren to treat muscular dystrophies or any other diseases; that ataluren is not found to be a safe or efficacious treatment for muscular dystrophies or any other diseases; and the risks and uncertainties described in reports filed by Genzyme with the Securities and Exchange Commission under the Securities Exchange Act of 1934, as amended, including without limitation the information under the heading "Risk Factors" in Genzyme's Annual Report on Form 10-K for the year ended December 31, 2009. Genzyme cautions investors not to place substantial reliance on the forward-looking statements contained in this press release. These statements speak only as of the date of this press release, and Genzyme undertakes no obligation to update or revise these statements.

#### **FOR MORE INFORMATION:**

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