PTC THERAPEUTICS COMPLETES ENROLLMENT OF PIVOTAL TRIAL IN MUSCULAR DYSTROPHY

- Ataluren designated as generic name for PTC124 -

SOUTH PLAINFIELD, NJ - February 3, 2009 - PTC Therapeutics, Inc. (PTC) today announced that it has successfully completed patient enrollment of its pivotal clinical trial of PTC124 in patients with nonsense mutation Duchenne and Becker muscular dystrophy (nmDMD/BMD). Accrual was completed two months ahead of schedule. The company also announced today that PTC124 has been issued the generic name, ataluren.

The one-year, multi-center, randomized, double-blind, placebo-controlled study is designed to determine whether ataluren can improve walking, muscle function and strength in patients with nmDMD/BMD. It will also evaluate ataluren's long-term safety profile over a longer term than in previous studies. The pivotal trial is ongoing at 37 sites on four continents and in 11 countries. The study has enrolled 174 patients across the United States, Europe, Canada, Australia, and Israel.

"This trial represents a critical milestone for the entire DMD/BMD community - 20 years after sequencing the dystrophin gene we have the first pivotal study for a potential disease-modifying therapy," said Brenda Wong, MD, associate professor of pediatrics and neurology, Cincinnati Children's Hospital Medical Center. "The rapid accrual is a testament to the commitment of boys with DMD/BMD and their families and the strong sense of urgency on the part of so many investigational teams around the world."

"Duchenne/Becker muscular dystrophy is a rare disorder and patients whose disease is due to a nonsense mutation represent only 13 percent of these cases, which created an enrollment challenge. However, the collaborative approach taken by clinical trial sites, advocacy groups, and PTC Therapeutics encouraged rapid identification of eligible candidates for this important study," said Richard Finkel, MD, attending physician, director of the neuromuscular program, Children's Hospital of Philadelphia. "The completion of this trial enrollment is a striking example of the successes we can achieve when patients, families and the medical community come together to address an area of such high unmet medical need."

"Currently available therapies for nmDMD/BMD may alleviate the symptoms of this progressive debilitating disease, but none address the underlying cause of the disease." said Langdon Miller, MD, chief medical officer of PTC. "Ataluren represents a potential breakthrough as the first treatment designed to enable the formation of functioning dystrophin, the missing protein in patients with nmDMD/BMD."

This is the first pivotal trial for ataluren. In addition, PTC is conducting an open-label, long-term safety study in boys with nmDMD who participated in an early Phase 2a trial. PTC also plans to initiate a second pivotal clinical trial of ataluren in patients with nonsense mutation cystic fibrosis (nmCF) in 2009.

The United States Adopted Names Council (USAN) in conjunction with the World Health Organization, International Nonproprietary Names Committee deemed ataluren's mechanism of action to be significantly novel, warranting creation of a new stem, -luren, for pharmaceuticals that are inducers of ribosomal readthrough of nonsense mutation mRNA stop codons.

"We are pleased to have completed enrollment of our first pivotal study of ataluren in boys and young men with nmDMD/BMD. To have achieved this milestone so far ahead of schedule is a tremendous achievement and we are grateful to the investigators, study coordinators, and the patients and their families for their efforts," said Stuart W. Peltz, PhD, president and CEO, PTC Therapeutics. "We look forward to completing this study over the next year as we continue to work to develop additional innovative therapies for the muscular dystrophy community."

ABOUT DMD/BMD

Duchenne and Becker muscular dystrophy (DMD/BMD) are progressive muscle disorders that result in the loss of both muscle function and independence. DMD/BMD is perhaps the most prevalent form 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. Further 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 ATALUREN (PTC124^{TM})

Ataluren is an orally delivered, investigational new drug discovered by PTC Therapeutics. The drug is being developed as a new approach for the treatment of nonsense mutation genetic disorders. Nonsense mutations create a premature stop signal in the mRNA causing the ribosome to terminate translation before a full-length protein is generated. This causes the protein to be truncated and non-functioning. Ataluren is designed to allow the ribosome to continue translation of the mRNA, by overriding the premature stop signal leading to the formation of a functioning protein. Ataluren is a potential therapy for patients with nonsense mutation genetic disorders.

Ataluren has demonstrated proof of concept in Phase 2a clinical trials and is currently in a registration-directed pivotal study with the goal of demonstrating that allowing expression of a critical missing protein will safely provide clinical benefits for patients with nonsense mutation genetic disorders. Ataluren has been generally well tolerated across all clinical studies to date.

The FDA and the European Commission have granted PTC124 (ataluren) orphan drug status for the treatment of DMD and cystic fibrosis due to nonsense mutations. The FDA has also granted PTC124 (ataluren) Subpart E designation for expedited development, evaluation, and marketing.

PTC has an exclusive collaboration with Genzyme Corporation for the development and commercialization of ataluren. PTC Therapeutics will market ataluren in the United States and Canada, while Genzyme will commercialize the product in other regions of the world. The development of ataluren has also been supported by grants from the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, FDA's Office of Orphan Products Development, the National Center for Research Resources and the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), which recently expanded support of ataluren to include funding up to $25 million.

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. PTC has extensive knowledge of post-transcriptional control processes and has developed proprietary technologies that it applies in its drug discovery activities internally and in collaborations with leading biopharmaceutical companies such as Genzyme, Pfizer, Celgene, CV Therapeutics and Schering-Plough. For more information, visit the company's Web site at www.ptcbio.com.

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