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PTC THERAPEUTICS ANNOUNCES EUROPEAN MEDICINES AGENCY VALIDATION OF MARKETING AUTHORIZATION APPLICATION FOR ATALUREN IN DUCHENNE MUSCULAR DYSTROPHY

Enrollment for a Global Confirmatory Phase 3 Clinical Trial Planned for 1Q13

SOUTH PLAINFIELD, NJ - **December 6, 2012** - PTC Therapeutics, Inc. (PTC) today announced that the European Medicines Agency (EMA) has validated a Marketing Authorization Application (MAA) seeking conditional approval for ataluren, an investigational new drug for the treatment of patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). Validation of the MAA confirms that the submission is complete and begins the EMA's Committee for Human Medicinal Products' (CHMP) review process. Ataluren is the only treatment currently in clinical development targeting the cause of disease in patients with a nonsense mutation.

"Ataluren is a promising potential therapy for nonsense mutation Duchenne muscular dystrophy," stated Dr. Thomas Voit, Medical and Scientific Director, Institut de Myologie. "PTC has developed a standard for DMD clinical trials and now the DMD community can share in the achievement of the first MAA ever filed for DMD. We appreciate PTC's commitment to the clinical development of ataluren for this severe disorder for which only palliative treatment options currently exist."

The Marketing Authorization Application's submission was accepted by EMA for review on the basis of a 48-week, 174-patient Phase 2b study showing that nmDMD patients treated with ataluren (10, 10, 20 mg/kg given daily) walked on average 31.3 meters farther than patients on placebo, as measured by the change in six-minute walk distance (6MWD) from baseline to Week 48. Patients receiving ataluren demonstrated a slower rate of decline in ambulation, based on an analysis of time to 10 percent worsening in 6MWD. While 44 percent of patients receiving placebo declined 10 percent or more in walking as measured by the 6-minute walk test (6MWT), only 26 percent of patients receiving ataluren declined 10 percent or more. Safety results showed that ataluren was generally well tolerated and adverse events were similar to placebo. Serious adverse events were infrequent and none were considered to be related to ataluren.

"We applaud PTC for its continued commitment to the DMD patient community and its comprehensive clinical development of ataluren," said Dr. Elizabeth Vroom, Chair, United Parent Project Muscular Dystrophy. "There is an urgent need for new therapeutic options for the patients and families and the path to approval of a new therapy can be arduous. We are extremely grateful to PTC not only for the robust work on developing ataluren but for helping set standards in our community which will benefit future drug development."

PTC also announced today the design of a confirmatory Phase 3 clinical trial of ataluren in patients with nmDMD that would support conditional approval and is expected to be initiated in the first quarter of 2013. The primary objective of the multicenter, randomized, double-blind, placebo-controlled Phase 3 study is to confirm the ability of ataluren to slow disease progression as assessed by ambulatory ability based on the 6MWD in patients with nmDMD. Secondary endpoints related to physical function and quality of life will also be assessed. Approximately 220 patients are expected to be enrolled in this global study. Further details concerning the study, including enrollment criteria, will be made available on www.clinicaltrials.gov.

"The EMA's validation of our MAA for ataluren for review is a significant milestone for PTC and for the patients, families and professionals who have contributed to this achievement," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "There is a clear and urgent need for new options for patients living with nmDMD, and we are committed to continuing our efforts to deliver ataluren to all patients who may benefit from this approach."

PTC also is currently conducting ongoing open-label safety and tolerability studies of ataluren for patients who were in previous ataluren clinical trials for nmDMD in both the United States and Europe. The company has worldwide development and commercial rights for ataluren in all indications and territories. Ataluren is also being developed for nonsense mutation cystic fibrosis and PTC is in discussions with regulators regarding next steps.

ABOUT CONDITIONAL APPROVAL
Conditional approval is granted based on a positive benefit/risk ratio in the available data which, while not yet comprehensive, indicate that the public health benefits of immediate availability of a medicine outweigh its risks. The company is given obligations to fulfill, such as the performance of further studies. The approval is renewed on a yearly basis until all obligations have been fulfilled, and is then converted from a conditional approval into a full approval. Conditional approvals can only be

granted for medicines that satisfy an unmet medical need, meaning the medicine is intended to be used for a disease or condition for which no treatment is readily available, and it is therefore important that patients have early access to the medicine concerned.

ABOUT DUCHENNE MUSCULAR DYSTROPHY (DMD)
Primarily affecting males, Duchenne muscular dystrophy (DMD) is a 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. There are an estimated 35,000 patients with DMD in the United States and Europe and approximately 13 percent of all DMD cases are caused by nonsense mutations in the dystrophin gene. More information about DMD is available through the Muscular Dystrophy Association (www.mdausa.org), Parent Project Muscular Dystrophy (www.parentprojectmd.org), Action Duchenne (www.actionduchenne.org), United Parent Projects Muscular Dystrophy (uppmd.org), Muscular Dystrophy Campaign (www.muscular-dystrophy.org) and AFM (l'Association française contre les myopathies), (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 nmDMD. 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. For more information, visit the company's website at www.ptcbio.com.

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