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ATALUREN CLINICAL DATA DEMONSTRATE AN INCREASE IN DYSTROPHIN EXPRESSION IN DUCHENNE MUSCULAR DYSTROPHY PATIENTS

- Data Published in PLOS ONE -

South Plainfield, NJ – December 12, 2013 – PTC Therapeutics, Inc.,(NASDAQ: PTCT) today announced the publication of data in PLOS ONE demonstrating that nonsense mutation Duchenne muscular dystrophy (nmDMD) patients treated with ataluren, an investigational new drug, experienced an increase in dystrophin expression. These data were obtained from PTC's Phase 2a open-label trial of ataluren in which change in full-length dystrophin expression, as assessed by immunofluorescent staining, was the primary endpoint.

Patients with nmDMD typically experience decreased levels of dystrophin protein, which is critical to normal muscle function. The study evaluated whether ataluren restores dystrophin production in the muscle cells of patients with nmDMD. Excisions of the extensor digitorum brevis (EDB) muscles were performed pre- and post-treatment with ataluren on all subjects, and immunofluorescence images were analyzed qualitatively and quantitatively.

"The results of this Phase 2a study support ataluren's activity as a dystrophin restoration therapy in nonsense mutation Duchenne muscular dystrophy patients mediated by ribosomal read-through of nonsense mutations," said Stuart Peltz, Ph.D., CEO of PTC Therapeutics, Inc. "These positive findings were the basis for the progression into a Phase 2b study of ataluren in patients with nmDMD, which further demonstrated the potential of ataluren to slow the progression of nonsense mutation DMD as measured by the 6-Minute Walk Test and informed the design of our ongoing Phase 3 study to confirm the safety and efficacy of ataluren."

"While there are challenges and limitations with currently available methods to assess changes in dystrophin expression, this proof-of-concept study demonstrated that ataluren has the potential to increase dystrophin expression, and thereby modify the course of this severely disabling disease," stated Richard Finkel, M.D., principal investigator at Nemours Children's Hospital, Orlando, FL.

ABOUT THE STUDY:
This Phase 2a open-label, sequential dose-ranging trial recruited 38 boys with nmDMD. The first cohort (n=6) received 16 mg/kg/day of ataluren divided into morning, midday and evening doses (4, 4, and 8 mg/kg); the second cohort (n=20) was dosed at 40 mg/kg/day (10, 10, 20 mg/kg); and the third cohort (n=12) was dosed at 80 mg/kg/day (20, 20, 40 mg/kg). Treatment duration was 28 days. Change in full-length dystrophin expression, as assessed by immunostaining in pre- and post-treatment muscle biopsy specimens, was the primary endpoint.

ABOUT ATALUREN
Ataluren, an investigational new drug discovered and developed by PTC Therapeutics, Inc., 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 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 PTC THERAPEUTICS, INC.
PTC is biopharmaceutical company focused on the discovery and development 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 essential to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and form the basis for collaborations with leading biopharmaceutical companies. For more information on the company, please visit our website www.ptcbio.com.

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FORWARD LOOKING STATEMENTS:
Any statements in this press release about future expectations, plans and prospects for the Company, the development of and potential market for the Company's product candidates, the Company's Phase 3 clinical trials for ataluren in nmDMD, the Company's current and planned filings with regulatory authorities, and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of our product candidates and other factors discussed in the "Risk Factors" in the most recent Quarterly Report, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views only as of the date of this release. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the Company's views as of any date subsequent to the date of this release.