August 4, 2014

PTC Therapeutics Receives Conditional Approval in the European Union for Translarna™ For the Treatment of Nonsense Mutation Duchenne Muscular Dystrophy

- The first treatment approved for DMD –

SOUTH PLAINFIELD, NJ – August 4, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that the European Commission has granted conditional marketing authorization for Translarna™ (ataluren), in the European Union (EU) for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged five years and older.

"We are delighted that Translarna was approved for the treatment of nonsense mutation Duchenne muscular dystrophy. By targeting the underlying cause of DMD, it has the potential to change the course of the disease. We are moving rapidly to make this product available to patients in the EU as we continue our global efforts to fulfill our vision of making Translarna available to all the boys it may benefit," stated Stuart W. Peltz, Ph.D., CEO of PTC Therapeutics, Inc. "We are grateful to the patients, families, advocacy groups and physicians who have supported PTC Therapeutics through many years of research and development of Translarna. The DMD community has been waiting a long time for treatment options and this conditional approval marks an important day for us all."

The authorization allows PTC to market Translarna in the 28 countries that are Member States of the European Union, as well as European Economic Area members Iceland, Liechtenstein and Norway. As part of the conditional marketing authorization, PTC is obligated to complete its confirmatory Phase 3 trial in nmDMD (ACT DMD) and submit additional efficacy and safety data from the trial.

The approval is based on the safety and efficacy results from a randomized double-blind multicenter study in 174 nmDMD patients for 48 weeks and our additional retrospective analyses of study data. The primary endpoint evaluated the effect of Translarna on ambulation as assessed by the change in distance walked (six-minute walk distance - 6MWD) during a six-minute walk test (6MWT). The post-hoc analysis showed that from baseline to Week 48, patients receiving Translarna (40 mg/kg/day given in 3 doses) had a 12.9 meter mean decline in 6MWD, and patients receiving placebo had a 44.1 meter mean decline in 6MWD. Thus the mean change in observed 6MWD from baseline to Week 48 was 31.3 meters better in the Translarna group than in the placebo group ($p=0.056$). Furthermore, in more severely affected patients whose baseline 6MWD was less than 350 meters, the mean change in observed 6MWD from baseline to Week 48 was 68 meters better in the Translarna group than in the placebo group. Patients on Translarna also showed a slower rate of decline in ambulation based on an analysis of time to 10 percent worsening in 6MWD. The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency found that these results suggest that Translarna slows the loss of walking ability in nmDMD patients.

Additionally, based on a retrospective analysis, patients receiving treatment also trended better in secondary endpoints such as stair climb and stair descend time-function tests, which the CHMP also found to suggest slowing of nmDMD progression relative to placebo. Safety results showed that Translarna was generally well tolerated. Serious adverse events were infrequent, and none was considered to be related to Translarna. The most frequent adverse reactions at the recommended dose were nausea, vomiting, and headache. These adverse reactions generally did not require medical intervention, and no patients discontinued Translarna treatment due to any adverse reaction.

"The world's first approved treatment for the underlying cause of DMD marks a very important moment for patients and their families. It is our highest priority to make Translarna available to patients and we will be working with regulators, payers, physicians and patient organizations to make that a reality." stated Mark Rothera, Chief Commercial Officer, of PTC Therapeutics, Inc.

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 DMD, 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. It is estimated that a nonsense mutation is the cause of DMD in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union. 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 a 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 in 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:
All statements, other than those of historical fact, contained in this press release, including statements regarding the future expectations, plans and prospects for PTC; the timing of regulatory approvals, the pricing and reimbursement process and Translarna availability ; the development of and potential market for Translarna, including our estimates regarding the size of the nmDMD patient population; our Phase 3 ACT DMD trial for Translarna in nmDMD, including the timing of enrollment for such trial; our ability to satisfy the obligations necessary to qualify or continue to qualify for Early Access programs or to obtain full approval for Translarna in nmDMD either in the EU or elsewhere; and the objectives of management, are forward-looking statements. Other forward-looking statements may be identified by the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions. Our actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements we make as a result of a variety of risks and uncertainties, including among others, those related to our expectations for regulatory approvals, the initiation and conduct of clinical trials, availability of data from clinical trials, our scientific approach and general development progress, the availability or commercial potential of our product candidates, market acceptance by physicians, patients, third-party payors and others in the medical community necessary for commercial success, our sales, marketing and distribution capabilities and strategy, and the other factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q, which is on file with the United States Securities and Exchange Commission. You are urged to carefully consider all such factors. In addition, the forward-looking statements included in this press release represent our views only as of the date of this release, and we do not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this release, except as required by applicable law.