September 9, 2014

PTC THERAPEUTICS COMPLETES ENROLLMENT OF LANDMARK TRIAL IN DUCHENNE MUSCULAR DYSTROPHY

- Top-line data expected H2 2015- -Data intended to support applications for additional approvals globally–

SOUTH PLAINFIELD, NJ – September 9, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has completed enrollment of ACT DMD, the Phase 3 confirmatory trial of Translarna™ (ataluren) for patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). Top-line data from the trial is expected in the second half of 2015 and will support further approvals globally, following European approval received earlier this year.

ACT DMD is a 48-week clinical trial designed to confirm the effect of Translarna on ambulation in patients with nmDMD. The primary endpoint is the change in walking distance as measured by the six-minute walk test. The trial has reached full enrollment across 54 sites globally. All patients in the trial are eligible to participate in an open-label extension study, which has already begun enrolling patients who have completed the initial 48 weeks of treatment.

"The enrollment of this trial represents a significant achievement in our efforts to develop disease-modifying treatments that advance the standard of care in DMD and improve the quality of life for patients," stated Dr. Haluk Topaloglu, Dept. of Neurology, Hacettepe Children's Hospital in Ankara, Turkey. "The enormous commitment on the part of patients and their families, advocacy groups, study investigators and PTC Therapeutics should be commended."

"The completion of enrollment marks an important milestone in the ACT DMD study," stated Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "We utilized the previous results demonstrating Translarna's clinical activity, as well as the natural history data from ours and other studies to optimize the ACT DMD trial design. This study is one of the largest DMD trials ever performed and we believe it is well powered for a successful outcome. We are pleased to reach this important landmark as it brings us one step closer to broadening Translarna's availability to all patients who may benefit."

ABOUT TRANSLARNA™ (ATALUREN)
Translarna, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of 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 Duchenne muscular dystrophy. PTC has received conditional marketing authorization in the European Economic Area for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Translarna is an investigational new drug in the US. The development of Translarna 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 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:
This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. 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 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 data from such trial; our ability to satisfy the obligations necessary 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 "will," "anticipate," "believe," "estimate," "expect," "look forward to," "intend," "may," "plan" "predict," "project," "target," "potential," "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, conduct and results of clinical trials; the 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.