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PTC Therapeutics Begins Rolling NDA Submission to the FDA for Translarna to Treat Duchenne Muscular Dystrophy

SOUTH PLAINFIELD, N.J., Dec. 23, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that it has commenced a rolling submission of a New Drug Application (NDA) to the United States Food and Drug Administration (FDA) for Translarna™ for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD). A rolling submission allows completed portions of the application to be submitted and reviewed by the FDA on an ongoing basis. PTC expects to finalize the application in the fourth quarter of 2015 following the completion of the ACT DMD confirmatory Phase 3 clinical trial.

"The initiation of our NDA submission for Translarna marks another significant milestone towards providing Translarna to all nonsense mutation Duchenne muscular dystrophy patients," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We look forward to the completion of the ACT DMD confirmatory Phase 3 clinical trial so that we can finalize the NDA. Gaining US approval, in addition to Translarna's European approval, will help to make Translarna available to patients across the globe. This is our commitment to the patients, families, advocacy groups and physicians who have worked and supported PTC Therapeutics through many years of research and development."

"We want Translarna available for Duchenne patients in the US as fast as possible," stated Pat Furlong, President and Founder of PPMD. "We commend PTC's perseverance and dedicated efforts to speed access to Translarna for people with Duchenne in the United States. Every day counts for people with Duchenne. Our hope is that PTC's efforts pave the way for Translarna as well as other therapies in the US."

About Translarna™ (ataluren)
Translarna, 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 Duchenne muscular dystrophy. Translarna is licensed in the European Economic Area 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 United States . 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 francaise 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 release, including statements regarding the future expectations, plans and prospects for PTC; the timing and scope of our commercial launch; our Phase 3 clinical trial for Translarna™ (ataluren) in nmDMD, including the timing and conduct of the trial and the period during which results will become available; the timing of the finalization our NDA submission; our strategy and future operations; the development of and potential market for Translarna and our other product candidates; the potential advantages of Translarna and objectives of management, are forward-looking statements. Other forward-looking statements may be identified by the words "goal," "plan," "prepare," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "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 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 Translarna and our other product candidates and the factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q as well as any updates to these risk factors filed from time to time in PTC's other filings with the Securities and Exchange Commission. You are urged to carefully consider all such factors. The forward-looking statements contained herein represent PTC's views only as of the date of this press 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 law.