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PTC Therapeutics Announces Launch of Translarna™ (ataluren) in Germany

-First drug for the underlying cause of nonsense mutation Duchenne muscular dystrophy -

SOUTH PLAINFIELD, N.J., Dec. 3, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Translarna™ (ataluren) is now commercially available to patients in Germany, the first country to launch in the European Union (EU), with first shipments expected to begin this week.

"The launch of Translarna in Germany marks another exciting milestone for the DMD community," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Through our reimbursed early access programs and commercial efforts, we are working hard to bring Translarna to those who may benefit from this drug. We remain committed to bringing Translarna to patients in Europe, and around the world, as quickly as possible. We know every day counts for the patients and their families."

The launch follows the European Commission marketing authorization, which was received in August 2014 for the use of Translarna to treat ambulatory patients with nonsense mutation Duchenne muscular dystrophy who are 5 years and older. A confirmatory Phase 3 clinical trial of Translarna, ACT DMD, in nonsense mutation Duchenne muscular dystrophy patients completed enrollment in September 2014. Top-line data is expected in the fourth quarter of 2015. Currently, Translarna is not approved for use in the United States.

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 non-profit 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 (jppmd.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 release, including statements regarding the future expectations, plans and prospects for PTC; the timing and scope of our commercial launch; our Phase 3 clinical trials for Translarna™ (ataluren) in nmDMD and nmCF; 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 "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.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/ptc-therapeutics-announces-launch-of-translarna-ataluren-in-germany-300003974.html>

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